

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION**  
Washington, D.C. 20549

**AMENDMENT NO. 2  
TO  
FORM S-1  
REGISTRATION STATEMENT  
UNDER  
THE SECURITIES ACT OF 1933**

**BRIDGEBIO PHARMA, INC.**

(Exact name of Registrant as specified in its charter)

Delaware  
(State or Other Jurisdiction of  
Incorporation or Organization)

2834  
(Primary Standard Industrial  
Classification Code Number)

84-1850815  
(I.R.S. Employer  
Identification Number)

421 Kipling Street  
Palo Alto, CA 94301  
(650) 391-9740  
(Address, including zip code, and telephone number, including area code, of Registrant's principal executive offices)

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Chief Executive Officer  
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**Approximate date of commencement of the proposed sale to the public:  
As soon as practicable after the effective date of this Registration Statement.**

If any of the securities being registered on this Form are to be offered on a delayed or continuous basis pursuant to Rule 415 under the Securities Act, check the following box.

If this Form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering.

If this Form is a post-effective amendment filed pursuant to Rule 462(c) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering.

If this Form is a post-effective amendment filed pursuant to Rule 462(d) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering.

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company" and "emerging growth company" in Rule 12b-2 of the Exchange Act. (Check one):

Large accelerated filer  Accelerated filer

Non-accelerated filer  Smaller reporting company

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 7(a)(2)(B) of the Securities Act.

**CALCULATION OF REGISTRATION FEE**

Title of each Class of Securities to be Registered	Amount to be Registered(1)	Proposed Maximum Offering Price per Share(2)	Proposed Maximum Aggregate Offering Price(2)	Amount of Registration Fee(3)
Common Stock, par value \$0.001 per share	17,250,000	\$16.00	\$276,000,000	\$33,451.20

- (1) Includes 2,250,000 shares that the underwriters have an option to purchase.  
 (2) Estimated solely for the purpose of calculating the registration fee pursuant to Rule 457(a) under the Securities Act of 1933, as amended.  
 (3) The registrant previously paid \$27,500 of this amount in connection with the initial filing of this registration statement.

**The Registrant hereby amends this Registration Statement on such date or dates as may be necessary to delay its effective date until the Registrant shall file a further amendment which specifically states that this Registration Statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act of 1933, as amended, or until the Registration Statement shall become effective on such date as the Securities and Exchange Commission, acting pursuant to said Section 8(a), may determine.**

## EXPLANATORY NOTE

We currently operate as BridgeBio Pharma LLC, a Delaware limited liability company, or the LLC entity. On May 17, 2019, we formed BridgeBio Pharma, Inc., a Delaware corporation, which is the registrant whose name appears on the cover of this registration statement, or the Corporation, as a stand-alone entity. Prior to the completion of this offering, we intend to complete a series of transactions pursuant to which we will form BridgeBio Pharma Merger Sub LLC, or Merger Sub LLC, an entity that will be a wholly-owned subsidiary of the Corporation. Merger Sub LLC will be merged with and into the LLC entity prior to the completion of this offering with the LLC entity being the surviving entity. As part of this merger, the unitholders of the LLC entity will exchange their units in the LLC entity for shares of common stock of the Corporation.

We refer to these transactions throughout the prospectus included in this registration statement collectively as the “Reorganization.” See “Reorganization” for further detail regarding these transactions. On the effective date of the Reorganization, the members of the board of managers of the LLC entity will become the members of the board of directors of the Corporation and the officers of the LLC entity will become the officers of the Corporation.

Except as disclosed in the accompanying prospectus, the audited combined and consolidated financial statements for 2017 and 2018 and the notes thereto and the condensed consolidated financial statements as of and for the three months ended March 31, 2018 and 2019 and the notes thereto and selected historical consolidated financial data and other financial information included in this registration statement are those of the LLC entity and do not give effect to the Reorganization.

Shares of the common stock of the Corporation are being offered by the prospectus included in this registration statement.

The information in this preliminary prospectus is not complete and may be changed. We may not sell these securities until the registration statement filed with the Securities and Exchange Commission is effective. This preliminary prospectus is not an offer to sell these securities and is not soliciting an offer to buy these securities in any jurisdiction where such offer or sale is not permitted.

Subject to Completion, dated June 17, 2019.

Preliminary prospectus

15,000,000 shares



Common stock

This is an initial public offering of our shares of common stock. We are offering shares of our common stock to be sold in the offering. The initial public offering price is expected to be between \$14.00 and \$16.00 per share.

Prior to this offering, there has been no public market for our common stock. We have applied to list our common stock on the Nasdaq Global Market under the symbol "BBIO."

We are an "emerging growth company" as defined under the federal securities laws and, as such, have elected to comply with certain reduced public company reporting requirements.

	Per share	Total
Initial public offering price	\$	\$
Underwriting discounts and commissions <sup>(1)</sup>	\$	\$
Proceeds to us before expenses	\$	\$

(1) See "Underwriting (Conflicts of Interest)" for a description of the compensation payable to the underwriters.

We have granted the underwriters an option for a period of 30 days to purchase up to 2,250,000 additional shares of common stock.

**Investing in our common stock involves a high degree of risk. See "[Risk Factors](#)" beginning on page 15.**

**Neither the Securities and Exchange Commission nor any other regulatory body has approved or disapproved of these securities or passed upon the accuracy or adequacy of this prospectus. Any representation to the contrary is a criminal offense.**

The underwriters expect to deliver the shares to purchasers on or about \_\_\_\_\_, 2019.

**J.P. Morgan**

**Goldman Sachs & Co. LLC**

**Jefferies**

**SVB Leerink**

**KKR**

**Piper Jaffray**

**Mizuho Securities**

**BMO Capital Markets**

**Raymond James**

, 2019

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We and the underwriters have not authorized anyone to provide any information other than that contained in this prospectus or in any free writing prospectus prepared by or on behalf of us or to which we have referred you. We take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give you. We and the underwriters are not making an offer to sell these securities in any jurisdiction where the offer or sale is not permitted. You should assume that the information appearing in this prospectus is accurate only as of the date on the front cover of this prospectus. Our business, financial condition, results of operations and prospects may have changed since that date.

**Through and including \_\_\_\_\_, 2019 (the 25th day after the date of this prospectus), all dealers that effect transactions in these securities, whether or not participating in this offering, may be required to deliver a prospectus. This is in addition to the dealers' obligation to deliver a prospectus when acting as underwriters and with respect to their unsold allotments or subscriptions.**

For investors outside of the United States: We have not, and the underwriters have not, done anything that would permit this offering or possession or distribution of this prospectus in any jurisdiction where action for that purpose is required, other than the United States. Persons outside of the United States who come into possession of this prospectus must inform themselves about, and observe any restrictions relating to, the offering of the shares of common stock and the distribution of this prospectus outside of the United States.

## **PRESENTATION OF NUMERICAL FIGURES**

Numerical figures included in this prospectus have been subject to rounding adjustments. Accordingly, numerical figures shown as totals in various tables may not be arithmetic aggregations of the figures that precede them.

## **FINANCIAL STATEMENT PRESENTATION**

The combined and consolidated financial statements for the years ended December 31, 2017 and 2018 and the condensed consolidated financial statements for the three months ended March 31, 2018 and 2019 represent the operations of BridgeBio Pharma LLC and its consolidated subsidiaries, including its controlled variable interest entities. Prior to the completion of this offering, we will complete a series of transactions pursuant to which BridgeBio Pharma, Inc. will succeed to the business of BridgeBio Pharma LLC and its consolidated subsidiaries, and the unitholders of BridgeBio Pharma LLC will become stockholders of BridgeBio Pharma, Inc., as described under the heading “Reorganization.” In this prospectus, we refer to this transaction as the “Reorganization.” We expect that these transactions will not have a material effect on our consolidated financial statements at the time of the Reorganization.

## PROSPECTUS SUMMARY

*This summary highlights information contained elsewhere in this prospectus and does not contain all of the information that you should consider in making your investment decision. Before investing in our common stock, you should read the entire prospectus carefully, including the sections entitled “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and our combined and consolidated financial statements and the related notes.*

*Prior to the completion of this offering, we will complete a series of transactions pursuant to which BridgeBio Pharma LLC will become a wholly owned subsidiary of BridgeBio Pharma, Inc., a newly formed Delaware corporation. See “Reorganization.” Except where the context otherwise requires or where otherwise indicated, the terms “BridgeBio,” “we,” “us,” “our,” “our company,” “the company,” and “our business” refer, prior to the Reorganization discussed below, to BridgeBio Pharma LLC and its consolidated subsidiaries, including its controlled variable interest entities, or VIEs, and, after the Reorganization, to BridgeBio Pharma, Inc. and its consolidated subsidiaries, including its controlled VIEs.*

### **Overview**

We are a team of experienced drug discoverers, developers and innovators working to create life-altering medicines that target well-characterized genetic diseases at their source. We founded BridgeBio in 2015 to identify and advance transformative medicines to treat patients who suffer from Mendelian diseases, which are diseases that arise from defects in a single gene, and cancers with clear genetic drivers. Our pipeline of over 15 development programs includes product candidates ranging from early discovery to late-stage development. Several of our programs target indications that we believe present the potential for our product candidates, if approved, to target portions of market opportunities of at least \$1.0 billion in annual sales. We have four product candidates in clinical trials that, if positive, we believe could support the filing of an application for marketing authorization. Two of these product candidates are in Phase 3 clinical trials, one is in a Phase 2/3 clinical trial, and one is in a Phase 2 clinical trial.

We focus on genetic diseases because they exist at the intersection of high unmet patient need and tractable biology. Our approach is to translate research pioneered at academic laboratories and leading medical institutions into products that we hope will ultimately reach patients. We are able to realize this opportunity through a confluence of scientific advances, including: (i) identification of the genetic underpinnings of disease as more cost-efficient genome and exome sequencing becomes available; (ii) progress in molecular biology; and (iii) the development and maturation of longitudinal data and retrospective studies that enable the linkage of genes to diseases. We believe that this early-stage innovation represents one of the greatest practical sources for new drug creation.

We believe we have developed a sustainable and scalable product platform that supports the continued growth of our company and the advancement of our pipeline.



### Product Platform

Systematic disease mapping  
World class R&D minds and capabilities  
Partnered with leading institutions  
Focus on capital efficiency  
Best owner mentality

Continued growth  
and terminal value



### Current Pipeline

15 programs targeting diseases at their source  
Four Phase 2 or Phase 3 clinical programs  
Focus on Mendelian diseases and targeted oncology  
Small molecules, biologics, gene therapies  
5+ therapeutic areas

Present value and  
near-term catalysts

### *Our Platform*

Our platform is distinguished by several key elements:

- **World class discovery and development talent:** Our team has previously submitted over 30 investigational new drug applications, or INDs, and 15 new drug applications, or NDAs, in aggregate. Our operations are overseen by a Management Committee that is comprised of renowned leaders in cancer and rare disease drug development.
- **Disciplined approach to target identification and prioritization:** We pair a systematic mapping of the genetic disease landscape with a proprietary set of over 10 criteria to narrow our focus on diseases with attractive attributes for drug development. We look for diseases with high unmet need and well-characterized mechanisms that present opportunities to address the root cause of disease.
- **Opportunistic approach to drug candidate selection:** We seek the best science and drug mechanisms of action, wherever they can be found. We accept programs that meet our standards at any stage of development, and we are agnostic to therapeutic area. However, we only pursue programs with treatment modalities that we believe are biologically suited to address the target disease.
- **Focus at the level of each program:** We maintain a decentralized structure wherein each program is housed in its own subsidiary. This allows us to build a team of experts and specialists tailored to the needs of each program, and who are economically incentivized at the program level. We enable our subsidiary leaders to make certain operational decisions outside of a centralized management hierarchy, as we fundamentally believe that those operators who have the most intimate program knowledge are best positioned to make key operational decisions.

- **Operational efficiency:** We aim to rapidly and decisively advance our product candidates to objective critical decision points. At each stage of research, discovery or development, we direct resources toward the opportunities that we believe are the most promising, and we discontinue programs that do not meet performance thresholds. We field a minimum viable team for each asset, with the goal of ensuring that each program has sufficient personnel to fit its purpose while reducing excess overhead costs. We accomplish this by hiring the best talent, centralizing and sharing certain support functions across various programs, and leveraging external providers where appropriate. This enables us to minimize traditionally fixed costs at the program level.
- **Portfolio breadth and diversification:** We have built a broad and diversified portfolio, with programs that vary across stage of development, therapeutic category and modality. We believe that our programs are biologically uncorrelated, covering different diseases, different targets and different modalities, such that the results of one program will not impact the development of others. Further, the breadth of our portfolio mitigates the impact of failure of any single program. As a result, we can be objective about each of our programs and allocate capital efficiently, delivering staged funding across our portfolio based on each program's scientific merits.
- **Optimized ownership for each program:** When we believe that we are best suited to continue a program's development, we will continue to fund it internally. If we believe a strategic partner is better suited to progress a program, we will consider externalizing development at economically attractive terms.

### ***Our Pipeline***

Our product platform supports the advancement of our current pipeline, which can be divided into three key categories:

- **Mendelian:** Ten small molecule and protein replacement product candidates, of which two are currently in Phase 3 clinical trials, one is in a Phase 2/3 clinical trial, one is in a Phase 1/2 clinical trial, three are currently in IND-enabling development and three are in lead optimization. Over the next 24 months, we expect to have at least six of these product candidates in clinical development. Several of our product candidates in this category target some of the most prevalent Mendelian diseases, including TTR amyloidosis, or ATTR, and achondroplasia. Two of our programs in this category have received breakthrough therapy designation from the FDA.
- **Oncology:** Four targeted oncology programs, of which one is in Phase 2 clinical development, one is in IND-enabling development, and two are in lead optimization, that address key oncogenic pathways including FGFR, KRAS and SHP2. These programs have potentially broad applicability across a number of solid tumor types with high unmet patient need.
- **Gene therapy:** Focused on developing treatments for Mendelian diseases that are particularly suited to gene therapy. Our two programs are both in IND-enabling development and we expect both of these programs to be in the clinic by 2020. Our gene therapy programs are led by executives who have substantial domain expertise and are recognized leaders in this field, and we are actively building our gene therapy capabilities.

Of our development programs, we believe the following, which we refer to as our key value drivers, have the greatest potential to drive significant value for our company due to a combination of factors, including their stage of development, potential availability of expedited development pathways, degree of unmet medical need and potential market size in the applicable target indication:

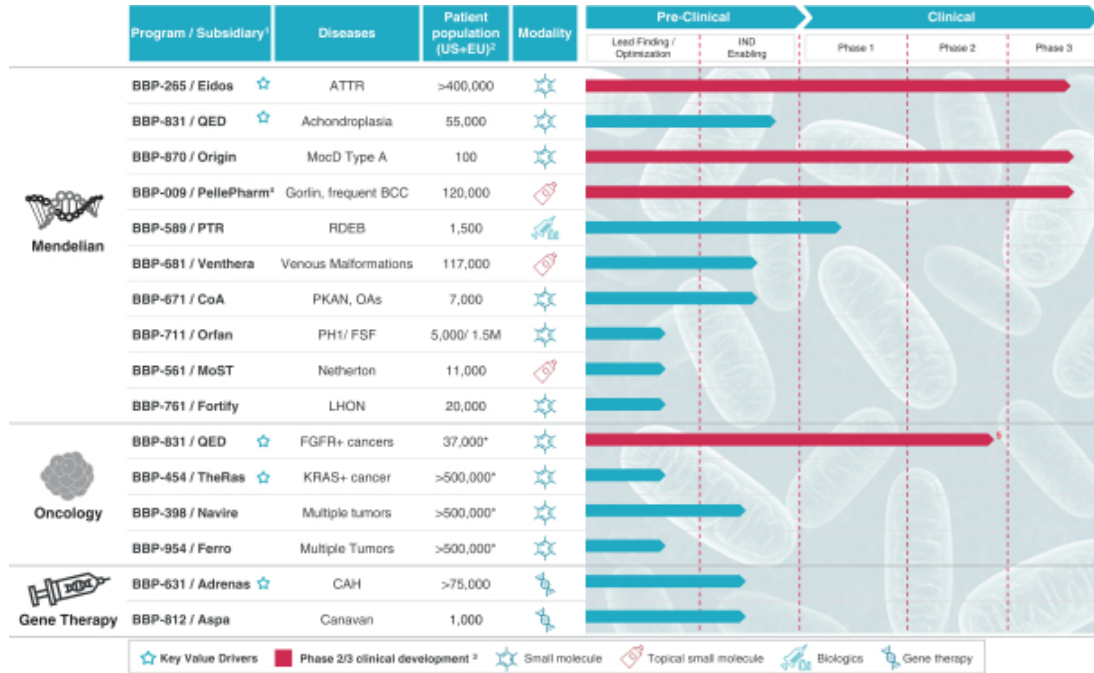
- BBP-265 (also known as AG10, under development at our subsidiary, Eidos Therapeutics, Inc.), a small molecule stabilizer of TTR that is in an ongoing Phase 3 clinical trial for the treatment of ATTR-CM.



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- BBP-831 (under development at our subsidiary, QED Therapeutics, Inc.), a small molecule selective FGFR1-3 inhibitor being developed for the treatment of FGFR-driven cancers and achondroplasia, for which we intend to submit an NDA in 2020 for the treatment of cholangiocarcinoma as a second-line or later therapy.
- BBP-631 (under development at our subsidiary, Adrenas Therapeutics, Inc.), an AAV5 gene transfer product candidate in preclinical development for the treatment of congenital adrenal hyperplasia, or CAH, driven by 21-hydroxylase deficiency, or 21OHD.
- BBP-454 (under development at our subsidiary TheRas, Inc.), a preclinical development program for small molecule inhibitors of KRAS for the treatment of pan-mutant KRAS-driven cancers, which act via two novel binding pockets.

The following table summarizes our material development programs, their estimated patient populations, their therapeutic modalities and their development status:



<sup>1</sup> Each of our programs is housed in a separate subsidiary. <sup>2</sup> Patient population: Prevalence except for asterisked figures which represent incidence; <sup>3</sup> Ongoing clinical trial we believe could support filing an application for marketing authorization, although the FDA and other regulatory authorities have not indicated their agreement or that additional trials will not be required. <sup>4</sup> We are party to an option agreement pursuant to which LEO Pharma A/S has been granted an exclusive, irrevocable option to acquire PellePharm, including the BBP-009 program. If the option is exercised by LEO Pharma A/S, we will no longer have rights to develop and commercialize BBP-009. See "Business —Our Material Agreements—BBP-009 (Patidegib): Option Agreement with LEO Pharma A/S." <sup>5</sup> Planned NDA submission for the treatment of cholangiocarcinoma as a second-line or later therapy

**Overview of Pipeline Key Value Drivers**

Of our development programs, we believe the following are our key value drivers. Accordingly, we currently expect that we will prioritize these programs in our near-term use of cash and cash equivalents. See “Use of Proceeds.”

**BBP-265/AG10 (Eidos): TTR Amyloidosis**

<b>Summary</b>	<ul style="list-style-type: none"> <li>We are developing BBP-265, an oral small molecule transthyretin, or TTR, stabilizer, for the treatment of ATTR, including both cardiomyopathy and polyneuropathy manifestations, or ATTR-CM and ATTR-PN, respectively</li> </ul>
<b>Development Status and Catalysts</b>	<ul style="list-style-type: none"> <li>Ongoing – Phase 3 clinical trial in ATTR-CM</li> <li>2019 – Planned initiation of Phase 3 clinical trial in ATTR-PN</li> <li>2019 – Anticipated reporting of data from ATTR-CM Phase 2 open-label extension on safety and key cardiac biomarkers</li> <li>2021 – Anticipated reporting of 12-month six-minute walk distance data from Phase 3 ATTRibute study in patients with ATTR-CM</li> <li>2021 – Potential NDA submission in ATTR-CM</li> </ul>
<b>Disease Overview</b>	<ul style="list-style-type: none"> <li>The dissociation of TTR tetramers into monomers and subsequent aggregation as amyloid proteins, or amyloid deposition, can cause ATTR-CM (wild-type and mutant) and ATTR-PN. Both manifestations of disease are progressive, have a significant negative impact on quality of life and are eventually fatal</li> <li>Prevalence greater than 400,000 worldwide for ATTR-CM, and greater than 10,000 worldwide for ATTR-PN</li> </ul>
<b>Our Product Concept</b>	<ul style="list-style-type: none"> <li>TTR stabilizer, designed to bind TTR and mimic the conformation of the naturally occurring T119M rescue mutation, which “super-stabilizes” TTR tetramers</li> <li>Phase 2 clinical trial completed in ATTR-CM patients demonstrated tolerability, near-complete TTR stabilization and normalization of serum TTR levels. We believe that higher levels of TTR stabilization will result in improved clinical outcomes</li> </ul>

**BBP-831/Infigratinib (QED): FGFR-Driven Cancers**

<b>Summary</b>	<ul style="list-style-type: none"> <li>We are developing infigratinib, an oral FGFR1-3 selective tyrosine kinase inhibitor, or TKI, for the treatment of FGFR-driven cancers</li> </ul>
<b>Development Status and Catalysts</b>	<ul style="list-style-type: none"> <li>Ongoing – Preparation for NDA submission in advanced cholangiocarcinoma, or CCA, as a second-line or later therapy</li> <li>Ongoing – Investigator-initiated trial in certain cancers involving FGFR translocations</li> <li>2019 – Anticipated first patient enrollment in Phase 3 clinical trial in advanced CCA as a first-line therapy</li> <li>2020 – Planned initiation of Phase 3 clinical trial in adjuvant urothelial carcinoma, or UC</li> </ul>

- 2020 – Anticipated NDA filing for treatment of advanced CCA as a second-line or later therapy

**Disease Overview**

- CCA is a rare, aggressive cancer of the bile ducts of the liver where the majority of newly diagnosed cases are non-resectable
  - Incidence is approximately 37,000 in the United States and European Union. Approximately 15% to 20% of patients have FGFR2 fusions or translocations
- UC is a cancer of the lining of the urinary tract. Patients undergoing first-line tumor excision for muscle invasive bladder cancer, or MIBC, and invasive upper tract urothelial cancer, or invasive UTUC, subtypes are most likely to be candidates for adjuvant therapy
  - Incidence is approximately 41,000 for MIBC and approximately 19,500 for invasive UTUC patients in the United States and European Union, which comprise our initial targeted indications. Approximately 15% to 20% of all patients with MIBC and approximately 50% to 60% of patients with invasive UTUC have FGFR3 genomic alterations
- Approximately 0.6% of all solid tumor cancers have fusions or translocations in the FGFR gene

**Our Product Concept**

- Designed to abrogate signaling via the FGFR1-3 pathways and inhibit cancer growth in FGFR-driven cancers, including CCA and UC
- In Phase 1 and Phase 2 clinical trials, infigratinib has shown activity that we believe to be meaningful in clinical measures such as overall response rate, in advanced CCA with FGFR2 fusions or translocations and in UC with FGFR3 genomic alterations

***BBP-831/Infigratinib (QED): Achondroplasia***

**Summary**

- We are developing infigratinib, an oral FGFR1-3 selective TKI in preclinical development for the treatment of achondroplasia

**Development Status and Catalysts**

- 2020 – Planned IND submission and initiation of Phase 1/2 clinical trial
- 2021 – Anticipated reporting of clinical proof-of-concept data from Phase 1/2 clinical trial

**Disease Overview**

- Achondroplasia is the most common form of disproportionate short stature. All cases are driven by autosomal dominant FGFR3 gain of function mutations
- Prevalence of greater than 55,000 in the United States and European Union, incidence of one in 10,000 to 30,000 live births worldwide

**Our Product Concept**

- Designed to inhibit overactive FGFR3 signaling, the underlying source of the disease
- Anticipated dosing levels significantly below those studied in our oncology clinical trials

***BBP-631 (Adrenas): Congenital Adrenal Hyperplasia***

**Summary**

- We are developing BBP-631, a preclinical adeno-associated virus, or AAV, gene transfer product candidate, for the treatment of CAH caused by 21OHD

<b>Development Status and Catalysts</b>	<ul style="list-style-type: none"><li>• Ongoing – Nonhuman primate studies, preparation for good laboratory practice, or GLP, toxicity studies</li><li>• 2020 – IND submission anticipated</li><li>• 2021 – Anticipated reporting of clinical proof-of-concept data</li></ul>
<b>Disease Overview</b>	<ul style="list-style-type: none"><li>• CAH is a debilitating, life-threatening disease defined by an inability to produce the steroids cortisol and aldosterone, and an excess production of testosterone. Complications include adrenal crises, dehydration and virilization in females<ul style="list-style-type: none"><li>• Over 90% of cases are caused by inactivating mutations in 21-hydroxylase, or 21OH</li></ul></li><li>• Prevalence estimated to be more than 75,000 in the United States and European Union. Newborn screening for 21OHD is conducted in every U.S. state and most European countries</li></ul>
<b>Our Product Concept</b>	<ul style="list-style-type: none"><li>• Intravenously-administered AAV5 gene transfer therapy intended to replace the 21OH enzyme in the adrenal cortex, potentially normalizing steroid levels (e.g., cortisol, aldosterone and androgens)</li><li>• A study in nonhuman primates demonstrated significant transfection in the adrenals, where 21OH is synthesized, with sustained vector genome counts and mRNA expression through three months</li></ul>
<b>BBP-454 (TheRas): KRAS-Driven Cancers</b>	
<b>Summary</b>	<ul style="list-style-type: none"><li>• We are advancing BBP-454, a preclinical development program for small molecule inhibitors of KRAS for the treatment of pan-mutant KRAS-driven cancers</li></ul>
<b>Development Status and Catalysts</b>	<ul style="list-style-type: none"><li>• Ongoing – Lead optimization</li><li>• 2020 – Development candidate nomination anticipated</li></ul>
<b>Pathway Overview</b>	<ul style="list-style-type: none"><li>• KRAS is a key driver of a number of large cancer indications with high unmet need including non-small cell lung cancers, pancreatic adenocarcinomas and colorectal adenocarcinomas. Historically, KRAS has been thought to be an undruggable target, due to its lack of clear binding pockets</li><li>• Incidence of over 500,000 patients diagnosed with a KRAS-driven cancer in the United States and European Union</li></ul>
<b>Our Product Concept</b>	<ul style="list-style-type: none"><li>• We are developing small molecule, pan-mutant KRAS inhibitors, which act through binding to two novel sites on KRAS</li><li>• Our first approach involves compounds that bind KRAS at a novel pocket on the hypervariable region, characterized by Frank McCormick, one of our co-founders and leader of the NCI RAS initiative, which prevents KRAS from binding to the cell membrane, thereby preventing signaling via the KRAS pathway</li><li>• The second approach involves targeting a unique residue on KRAS which promotes its degradation and thus down-regulates signaling</li></ul>

### **Risks Associated with Our Business**

Our ability to execute on our business strategy is subject to a number of risks, which are discussed more fully in the section of this prospectus entitled “Risk Factors.” You should carefully consider these risks before making an investment in our common stock. These risks include, among others, the following:

- We have incurred significant operating losses since our inception and have not generated any revenue from product sales. We expect to incur continued losses for the foreseeable future and may never achieve or maintain profitability.
- We will require substantial additional funding. If we are unable to raise capital when needed, or on attractive terms, we could be forced to delay, reduce or eliminate our research or product development programs or any future commercialization efforts.
- We have a limited operating history, have not successfully completed late-stage clinical trials for any product candidate, have not generated revenue from product sales or profits and do not expect to generate revenue or profits for the foreseeable future. We may never obtain approval for any of our product candidates or achieve or sustain profitability.
- Clinical drug development involves a lengthy and expensive process. We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of BBP-265, BBP-831, BBP-631, BBP-454 and our other product candidates.
- Certain of our product candidates are based on novel gene therapy technology with which there is limited clinical or regulatory experience to date, and therefore face heightened challenges for obtaining and maintaining regulatory approval, negative perception among the public and the medical community and additional manufacturing challenges.
- If we experience delays or difficulties in the enrollment of patients in clinical trials, including in our Phase 3 clinical trials of BBP-265 and BBP-831, our receipt of necessary marketing approvals could be delayed or prevented.
- The incidence and prevalence for target patient populations of our product candidates have not been established with precision. If the market opportunities for our product candidates are smaller than we estimate or if any approval that we obtain is based on a narrower definition of the patient population, our revenue potential and ability to achieve profitability will be adversely affected.
- We face substantial competition, including competition from large pharmaceutical and biotechnology companies that are currently pursuing the development and commercialization of products for the treatment of the indications that our four key value drivers are pursuing, which may result in others discovering, developing or commercializing products before or more successfully than we do.
- We may enter into collaborations with third parties for the development and commercialization of our product candidates. If those collaborations are not successful, we may not be able to capitalize on the market potential of these drug candidates.
- Manufacturing pharmaceutical products is complex and subject to production delays and product loss for a variety of reasons. We contract with third parties for the manufacture of our product candidates for preclinical testing and clinical trials and expect to continue to do so for commercialization. This reliance on third parties increases the risk that we will not have sufficient quantities of our drug candidates or products or such quantities at an acceptable cost or quality, which could delay, prevent or impair our development or commercialization efforts.
- If we are unable to obtain and maintain sufficient patent protection for our product candidates, or if the scope of the patent protection is not sufficiently broad, third parties, including our competitors, could develop and commercialize products similar or identical to ours, and our ability to commercialize our

product candidates successfully may be adversely affected. Additionally, if we are subject to or fail to prevail against claims that we have infringed the intellectual property rights of third parties, including in an ongoing patent litigation suit filed against our investee PellePharm, Inc., our ability to commercialize our product candidates will be harmed.

- Because our principal stockholders and certain members of our management own a significant percentage of our stock, they will be able to exert significant control over matters subject to stockholder approval, and may therefore be able to influence us through their ownership positions and determine matters that are subject to approval by our stockholders in a manner that may not be in your best interests as a stockholder.
- We have limited resources, and we may expend our resources to pursue a particular product candidate and fail to capitalize on development opportunities or product candidates that may be more profitable or for which there is a greater likelihood of success.
- We have identified material weaknesses in our internal control over financial reporting. If our remediation of these material weaknesses is not effective, or if we experience additional material weaknesses in the future, we may not be able to accurately or in a timely manner report our financial condition or results of operations, which may adversely affect the value of our common stock.

### **Corporate Information**

BridgeBio LLC was formed in April 2015. BridgeBio Pharma LLC, or the LLC entity, was formed in March 2016. In June 2017, BridgeBio Pharma LLC merged with BridgeBio LLC, with BridgeBio Pharma LLC being the surviving entity. Our principal executive offices are located at 421 Kipling Street, Palo Alto, CA 94301, and our telephone number is (650) 391-0740. Our corporate website address is <https://bridgebio.com>. Information contained on or accessible through our website is not a part of this prospectus, and the inclusion of our website address in this prospectus is an inactive textual reference only.

On May 17, 2019, we formed BridgeBio Pharma, Inc., a Delaware corporation, or the Corporation, as a stand-alone entity. We intend to complete a series of transactions pursuant to which we will form BridgeBio Pharma Merger Sub LLC, or Merger Sub LLC, an entity that will be a wholly-owned subsidiary of the Corporation. Merger Sub LLC will be merged with and into the LLC entity prior to the completion of this offering, with the LLC entity being the surviving entity and a wholly-owned subsidiary of the Corporation. As part of this merger, the unitholders of the LLC entity will exchange their units in the LLC entity for shares of common stock of the Corporation. See “Reorganization” and “Description of Capital Stock” for additional information, including a description of the terms of our capital stock following the Reorganization and the terms of our amended and restated certificate of incorporation and amended and restated bylaws that will be in effect immediately prior to completion of this offering.

The number of shares of the Corporation’s common stock that holders of units in the LLC entity will be entitled to receive in the Reorganization will be determined in accordance with our Fourth Amended and Restated Limited Liability Company Agreement, dated November 20, 2018, as amended, or the LLC Agreement, and varies depending on the series of units owned by the holder in the LLC entity. The number of shares of common stock that holders of units in the LLC entity will receive in connection with the Reorganization will also vary depending on the initial public offering price set forth on the cover page of this prospectus.

The information in this prospectus is based on our estimate that, in the Reorganization, an aggregate of 99,999,967 shares of our common stock will be issued to holders of units in the LLC entity based on an initial public offering price per share of common stock of \$15.00, the midpoint of the price range set forth on the cover page of this prospectus. To the extent that the actual initial public offering price per share for this offering is greater or less than \$15.00, the actual number of shares of common stock to be issued in connection with the Reorganization will be adjusted accordingly. See “Pricing Sensitivity Analysis” for how the number of shares to be issued in the

Reorganization would be affected by an initial public offering price per share of common stock at the low-, mid- and high-points of the estimated price range set forth on the cover of this prospectus or if the underwriters' option to purchase additional shares of common stock is exercised in full.

We use various trademarks and trade names in our business, including, without limitation, our corporate name and logo. All other trademarks or trade names referred to in this prospectus are the property of their respective owners. Solely for convenience, the trademarks and trade names in this prospectus may be referred to without the ® and ™ symbols, but such references should not be construed as any indicator that their respective owners will not assert, to the fullest extent under applicable law, their rights thereto.

#### **Implications of Being an Emerging Growth Company**

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act, or the JOBS Act, enacted in April 2012, as amended. For so long as we remain an emerging growth company, we are permitted and intend to rely on certain exemptions from various public company reporting requirements, including not being required to have our internal control over financial reporting audited by our independent registered public accounting firm pursuant to Section 404(b) of the Sarbanes-Oxley Act of 2002; as amended, or the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and any golden parachute payments not previously approved. In particular, in this prospectus, we provide only two years of audited financial statements and have not included all of the executive compensation related information that would be required if we were not an emerging growth company. Accordingly, the information contained herein may be different than the information you receive from other public companies in which you hold stock.

We will remain an emerging growth company until the earlier to occur of (i) December 31, 2024, (ii) the last day of the fiscal year in which we have total annual gross revenues of at least \$1.07 billion, (iii) the last day of the fiscal year in which we are deemed to be a "large accelerated filer," under the rules of the U.S. Securities and Exchange Commission, which means the market value of our equity securities that is held by non-affiliates exceeds \$700 million as of the prior June 30th, and (iv) the date on which we have issued more than \$1.0 billion in non-convertible debt during the prior three-year period.

In addition, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This provision allows an emerging growth company to delay the adoption of some accounting standards until those standards would otherwise apply to private companies. We have elected to use this extended transition period for complying with new or revised accounting standards that have different effective dates for public and private companies until the earlier of the date we (i) are no longer an emerging growth company or (ii) affirmatively and irrevocably opt out of the extended transition period provided in the JOBS Act. As a result, our combined and consolidated financial statements may not be comparable to companies that comply with new or revised accounting pronouncements as of public company effective dates.

## THE OFFERING

<b>Common stock offered by us</b>	15,000,000 shares
<b>Common stock to be outstanding immediately after this offering</b>	114,999,967 shares (or 117,249,967 shares if the underwriters exercise their option to purchase additional shares in full).
<b>Option to purchase additional shares</b>	We have granted the underwriters an option to purchase up to 2,250,000 additional shares of common stock from us. The underwriters can exercise this option at any time within 30 days from the date of this prospectus.
<b>Use of proceeds</b>	We estimate that we will receive net proceeds from the sale of our common stock in this offering of approximately \$203.7 million, or \$235.1 million if the underwriters fully exercise their option to purchase additional shares, assuming an initial public offering price of \$15.00 per share, the midpoint of the price range set forth on the cover page of this prospectus, and after deducting estimated underwriting discounts and commissions and offering expenses payable by us. We intend to use the net proceeds from this offering for the uses as set forth in the “Use of Proceeds” section of this prospectus.
<b>Risk factors</b>	You should read carefully “Risk Factors” beginning on page 15 and other information included in this prospectus for a discussion of factors that you should consider before deciding to invest in our common stock.
<b>Conflicts of interest</b>	Affiliates of KKR Capital Markets LLC own more than 10% of our common stock. Because KKR Capital Markets LLC is an underwriter for this offering, it is deemed to have a “conflict of interest” within the meaning of FINRA Rule 5121(f)(5)(B). Accordingly, this offering is being made in compliance with the requirements of FINRA Rule 5121. Since KKR Capital Markets LLC is not primarily responsible for managing this offering, pursuant to FINRA Rule 5121, the appointment of a qualified independent underwriter is not necessary. KKR Capital Markets LLC will not confirm sales to discretionary accounts without the prior written approval of the account holder. See “Underwriting (Conflicts of Interest)—Conflicts of Interest.”
<b>Proposed Nasdaq Global Market symbol</b>	“BBIO”
<b>Directed share program</b>	At our request, the underwriters have reserved up to 5.0% of the shares of our common stock offered by this prospectus for sale, at the initial public offering price, to our directors and officers and certain other parties related to us. Shares purchased by our and our subsidiaries’ directors, officers and certain employees will be subject to the 180-day lock-up restriction described in the “Underwriting” section of this prospectus. The number of shares of common stock



available for sale to the general public will be reduced to the extent these individuals purchase such reserved shares. Any reserved shares that are not so purchased will be offered by the underwriters to the general public on the same basis as the other shares offered by this prospectus.

The number of shares of our common stock to be outstanding after this offering assumes the Reorganization takes place prior to the completion of this offering and is based on (i) an assumed initial public offering price of \$15.00 per share, the midpoint of the price range set forth on the cover page of this prospectus, and (ii) 99,999,967 shares of our common stock (which includes 7,019,309 shares of restricted common stock) outstanding as of March 31, 2019, assuming the exchange of all outstanding units of BridgeBio Pharma LLC as of March 31, 2019 into shares of common stock of BridgeBio Pharma, Inc. See the section of the prospectus titled “Reorganization.”

The number of shares of our common stock to be outstanding immediately following the completion of this offering excludes:

- 11,500,000 shares of our common stock reserved for issuance under our 2019 Stock Option and Incentive Plan, which will become effective in connection with this offering, as well as any future increases in the number of shares of our common stock reserved for issuance under our 2019 Stock Option and Incentive Plan; and
- 2,000,000 shares of our common stock reserved for issuance under our 2019 Employee Stock Purchase Plan, which will become effective in connection with this offering, as well as any future increases in the number of shares of our common stock reserved for issuance under our 2019 Employee Stock Purchase Plan.

In this prospectus, unless otherwise indicated or the context otherwise requires, the number of shares of common stock outstanding and the other information based thereon reflects and assumes:

- no exercise by the underwriters of their option to purchase up to an additional 2,250,000 shares of our common stock from us;
- the completion of the Reorganization, including the exchange of all outstanding units of BridgeBio Pharma LLC as of March 31, 2019 for an aggregate of 99,999,967 shares of common stock of BridgeBio Pharma, Inc. (which includes 7,019,309 shares of restricted common stock), prior to the completion of this offering, assuming an initial public offering price of \$15.00 per share, the midpoint of the price range set forth on the cover page of this prospectus (see “Reorganization”); and
- the filing of our amended and restated certificate of incorporation and the adoption of our amended and restated bylaws, which will occur immediately prior to the completion of this offering.

The number of shares of common stock of BridgeBio Pharma, Inc. that holders of units of BridgeBio Pharma LLC will receive in the Reorganization will vary depending on the initial public offering price. See “Pricing Sensitivity Analysis” for additional information.

## SUMMARY CONSOLIDATED FINANCIAL DATA

The following information is presented for BridgeBio Pharma LLC, which will become a wholly owned subsidiary of BridgeBio Pharma, Inc., the entity whose shares are being offered hereby. You should read the following summary consolidated financial data together with our combined and consolidated financial statements and the related notes appearing at the end of this prospectus and the “Selected Consolidated Financial Data” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” sections of this prospectus. We have derived the combined and consolidated statements of operations and comprehensive loss data for the years ended December 31, 2017 and 2018 from our audited combined and consolidated financial statements appearing at the end of this prospectus. We derived the consolidated statements of operations and comprehensive loss data for the three months ended March 31, 2018 and 2019 and the consolidated balance sheet data as of March 31, 2019 from our interim condensed consolidated financial statements appearing at the end of this prospectus. Our interim condensed consolidated financial statements were prepared on the same basis as our audited combined and consolidated financial statements and, in our opinion, reflect all adjustments, consisting only of normal recurring adjustments, that are necessary for the fair statement of our interim condensed consolidated financial statements. Our historical results are not necessarily indicative of the results that may be expected in the future.

	Year Ended December 31,		Three Months Ended March 31,	
	2017	2018	2018	2019
	(in thousands, except share, unit, per share and per unit data)			
<b>Statements of Operations and Comprehensive Loss Data:</b>				
Operating expenses:				
Research and development	\$ 30,556	\$ 140,073	\$ 33,831	\$ 44,853
General and administrative	13,302	43,587	8,007	18,899
Total operating expenses	<u>43,858</u>	<u>183,660</u>	<u>41,838</u>	<u>63,752</u>
Loss from operations	(43,858)	(183,660)	(41,838)	(63,752)
Other income (expense), net:				
Interest income	39	2,004	1	2,107
Interest expense	(13)	(2,547)	(7)	(1,671)
Gain on deconsolidation of PellePharm	—	19,327	—	—
Loss from PellePharm	—	(275)	—	(4,599)
LEO call option expense	—	(3,009)	—	(1,514)
Other expense	—	(1,291)	(586)	(7)
Total other income (expense), net	<u>26</u>	<u>14,209</u>	<u>(592)</u>	<u>(5,684)</u>
Net loss and comprehensive loss	(43,832)	(169,451)	(42,430)	(69,436)
Net loss attributable to redeemable convertible noncontrolling interests and noncontrolling interests	13,267	38,702	8,274	8,251
Net loss and comprehensive loss attributable to BridgeBio	\$ (30,565)	\$ (130,749)	\$ (34,156)	\$ (61,185)
Cumulative returns on redeemable convertible preferred units (Series A, Series B and Series C)				
	(5,672)	(13,287)	(3,121)	—
Net loss attributable to redeemable founder units and redeemable common units	<u>\$ (36,237)</u>	<u>\$ (144,036)</u>	<u>\$ (37,277)</u>	<u>\$ (61,185)</u>
Net loss per unit attributable to redeemable founder unitholders and redeemable common unitholders, basic and diluted				
	<u>\$ (2.18)</u>	<u>\$ (8.01)</u>	<u>\$ (2.13)</u>	<u>\$ (3.25)</u>
Total weighted-average redeemable founder units and redeemable common units used in computing net loss per unit, basic and diluted				
	<u>16,650,073</u>	<u>17,991,781</u>	<u>17,485,526</u>	<u>18,827,234</u>
Pro forma net loss per share, basic and diluted <sup>(1)</sup>				
		<u>\$ (2.32)</u>		<u>\$ (0.66)</u>
Pro forma weighted average common shares outstanding, basic and diluted <sup>(1)</sup>				
		<u>62,087,997</u>		<u>92,741,876</u>

- (1) On May 17, 2019, we formed the Corporation as a stand-alone entity. We then intend to complete a series of transactions pursuant to which we will form Merger Sub LLC, an entity that will be a wholly owned subsidiary of the Corporation. Merger Sub LLC will be merged with and into the LLC entity prior to the completion of this offering, with the LLC entity being the surviving entity. As part of this merger, the unitholders of the LLC entity will exchange their units in the LLC entity for shares of the Corporation. These transactions are collectively referred to as the Reorganization. See the section of this prospectus titled “Reorganization.” We have presented pro forma basic and diluted net loss per share for the periods ended December 31, 2018 and March 31, 2019, which consists of our historical net loss attributable to BridgeBio Pharma LLC and its consolidated subsidiaries, divided by the pro forma basic and diluted weighted average number of shares of common stock outstanding after giving effect to the exchange of all of the outstanding units in BridgeBio Pharma LLC into 99,999,967 shares of our common stock prior to the closing of this offering in connection with the Reorganization. Net loss used in calculating net loss per share does not reflect (i) the estimated expenses of this offering or (ii) compensation and expenses for our board of directors and other costs related to operating as a public company. For more information on how we calculate basic and diluted pro forma weighted average number of shares outstanding, see Note 16 to our audited combined and consolidated financial statements and Note 16 to our unaudited condensed consolidated financial statements included elsewhere in this prospectus.

	As of March 31, 2019		
	Actual	Pro Forma(1)	Pro Forma As Adjusted(2)
(in thousands)			
<b>Consolidated Balance Sheet Data:</b>			
Cash and cash equivalents	\$ 373,027	\$ 373,027	\$ 577,661
Working capital(3)	347,001	347,001	552,479
Total assets	401,374	401,374	604,227
Total long-term debt obligations	54,861	54,861	54,861
Redeemable convertible preferred units	479,044	—	—
Redeemable founder units	1,754	—	—
Redeemable common units	1,645	—	—
Undesignated preferred stock	—	—	—
Common stock	—	100	115
Accumulated deficit	(234,733)	(234,733)	(234,733)
Noncontrolling interests	58,318	58,318	58,318
Total members’ deficit, actual; total stockholders’ equity, pro forma and pro forma as adjusted	(176,415)	310,459	514,156

- (1) The consolidated pro forma balance sheet data gives effect to the Reorganization, pursuant to which all outstanding units of BridgeBio Pharma LLC will be exchanged for 99,999,967 shares of common stock of BridgeBio Pharma, Inc. based on an assumed initial public offering price of \$15.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus. The number of shares of common stock of BridgeBio Pharma, Inc. that holders of units of BridgeBio Pharma LLC will receive in the Reorganization will vary depending on the initial public offering price. See “Pricing Sensitivity Analysis” for more information.
- (2) The pro forma as adjusted consolidated balance sheet data gives further effect to our issuance and sale of 15,000,000 shares of our common stock offered in this offering at an assumed initial public offering price of \$15.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. See “Pricing Sensitivity Analysis” for how some of the pro forma as adjusted information provided above would be affected by the initial public offering price per share of common stock at the low-, mid- and high-points of the estimated price range indicated on the cover of this prospectus or if the underwriters’ option to purchase additional shares of common stock is exercised in full.
- (3) We define working capital as current assets less current liabilities.

## RISK FACTORS

*Investing in our common stock involves a high degree of risk. You should consider carefully the following risks, together with all the other information in this prospectus, including our combined and consolidated financial statements and notes thereto, as well as the risks, uncertainties and other information set forth in the reports and other materials filed or furnished by our majority-controlled subsidiary Eidos Therapeutics, Inc., or Eidos, with the U.S. Securities and Exchange Commission, or the SEC, before you invest in our common stock. If any of the following risks or the risks included in the public filings of Eidos actually materializes, our operating results, financial condition and liquidity could be materially adversely affected. As a result, the trading price of our common stock could decline and you could lose part or all of your investment.*

### **Risks Related to our Financial Position, Need for Additional Capital and Growth Strategy**

***Drug development is a highly uncertain undertaking and involves a substantial degree of risk. We have incurred significant losses since our inception and anticipate that we will continue to incur significant losses for the foreseeable future. We have not generated any revenue since inception, which, together with our limited operating history, may make it difficult for you to assess our future viability.***

Pharmaceutical and biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of risk. We are a clinical-stage biopharmaceutical company with a limited operating history upon which you can evaluate our business and prospects. Our subsidiaries, on whose success we largely rely, are also early-stage biopharmaceutical companies. To date, we have focused principally on identifying, acquiring or in-licensing and developing our product candidates at the subsidiary level, all of which are in discovery, lead optimization, preclinical or clinical development. Our product candidates will require substantial additional development time, including extensive clinical research, and resources before we would be able to apply for or receive regulatory approvals and begin generating revenue from product sales.

We are not profitable and have incurred losses in each year since our inception in April 2015. Our net losses for the years ended December 31, 2017 and 2018 and the three months ended March 31, 2019 were \$43.8 million, \$169.5 million and \$69.4 million respectively. As of March 31, 2019, we had an accumulated deficit of \$234.7 million. We have no products approved for commercial sale and have not generated any revenues from product sales, and have financed operations solely through the sale of equity securities and debt financings. We continue to incur significant research and development, or R&D, and other expenses related to ongoing operations and expect to incur losses for the foreseeable future. We anticipate these losses will increase significantly following the completion of this offering and we will not generate any revenue from product sales until after we have successfully completed clinical development and received regulatory approval for the commercial sale of one or more product candidates.

Because of the numerous risks and uncertainties associated with drug development, we are unable to predict the timing or amount of our expenses, or when we will be able to generate any meaningful revenue or achieve or maintain profitability, if ever. In addition, our expenses could increase beyond our current expectations if we are required by the U.S. Food and Drug Administration, or FDA, or comparable foreign regulatory authorities, to perform nonclinical or preclinical studies or clinical trials in addition to those that we currently anticipate, or if there are any delays in any of our or our future collaborators' clinical trials or the development of our product candidates that we may identify. Even if our future product candidates that we may identify are approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate and ongoing compliance efforts.

We may never be able to develop or commercialize a marketable drug or achieve profitability. Revenue from the sale of any product candidate for which regulatory approval is obtained will be dependent, in part, upon the size of the markets in the territories for which we gain regulatory approval, the accepted price for the product, the ability to obtain reimbursement at any price and whether we own the commercial rights for that territory. Our growth strategy depends on our ability to generate revenue. In addition, if the number of addressable patients is

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not as anticipated, the indication approved by regulatory authorities is narrower than expected, or the reasonably accepted population for treatment is narrowed by competition, physician choice or treatment guidelines, we may not generate significant revenue from sales of such products, even if approved. Even if we are able to generate revenue from the sale of any approved products, we may not become profitable and may need to obtain additional funding to continue operations. Even if we achieve profitability in the future, we may not be able to sustain profitability in subsequent periods. Our failure to achieve sustained profitability would depress the value of our company and could impair our ability to raise capital, expand our business, diversify our research and development pipeline, market our product candidates, if approved, that we may identify and pursue or continue our operations. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' equity and working capital.

***We will require substantial additional funding to achieve our business goals. If we are unable to obtain this funding when needed and on acceptable terms, we could be forced to delay, limit or terminate our product development efforts.***

Developing biopharmaceutical products is expensive and time-consuming, and we expect to require substantial additional capital to conduct research, preclinical testing and human studies, may establish pilot scale and commercial scale manufacturing processes and facilities, and establish and develop quality control, regulatory, marketing, sales and administrative capabilities to support our existing programs and pursue potential additional programs. We are also responsible for the payments to third parties of expenses that may include milestone payments, license maintenance fees and royalties, including in the case of certain of our agreements with academic institutions or other companies from whom intellectual property rights underlying their respective programs have been in-licensed or acquired. Because the outcome of any preclinical or clinical development and regulatory approval process is highly uncertain, we cannot reasonably estimate the actual amounts necessary to successfully complete the development, regulatory approval process and commercialization of any future product candidates we may identify.

As of March 31, 2019, we had working capital of \$347.0 million and cash and cash equivalents of \$373.0 million. We estimate that the net proceeds from this offering will be approximately \$203.7 million (or approximately \$235.1 million if the underwriters exercise in full their option to purchase additional shares), after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. We expect that the net proceeds from this offering, together with our existing cash will be sufficient to fund our operations through at least the next 24 months. However, our operating plan may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned, through public or private equity or debt financings or other sources, such as strategic collaborations or license and development agreements. Any additional fundraising efforts for us may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize product candidates that we may identify and pursue. Moreover, such financing may result in dilution to stockholders, imposition of debt covenants and repayment obligations, or other restrictions that may affect our business. In addition, we may seek additional capital due to favorable market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans.

Our future funding requirements will depend on many factors, including, but not limited to:

- the time and cost necessary to complete ongoing and planned clinical trials, including Eidos' ongoing and planned Phase 3 clinical trials of BBP-265; our Phase 2 clinical trial of infigratinib in CCA as a second-line therapy, Phase 3 clinical trial of infigratinib in CCA as a first-line therapy and Phase 3 clinical trial of infigratinib in adjuvant UC; our Phase 3 clinical trial of BBP-009 in Gorlin syndrome and Phase 2b clinical trial in high frequency basal cell carcinoma; and our Phase 1/2 clinical trial of BBP-589 in dystrophic epidermolysis bullosa;
- the time and cost necessary to pursue regulatory approvals for our product candidates, and the costs of post-marketing studies that could be required by regulatory authorities;

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- the progress, timing, scope and costs of our nonclinical studies, preclinical studies, clinical trials and other related activities, including the ability to enroll patients in a timely manner, for the ongoing and planned clinical trials set forth above, and potential future clinical trials;
- the costs of obtaining clinical and commercial supplies of raw materials and drug products for our product candidates, including protein or gene therapies such as BBP-589, BBP-631, and BBP-812 and any other product candidates we may identify and develop;
- our ability to successfully identify and negotiate acceptable terms for third-party supply and contract manufacturing agreements with contract manufacturing organizations, or CMOs;
- our ability to successfully commercialize product candidates;
- the manufacturing, selling and marketing costs associated with our product candidates, including the cost and timing of expanding our internal sales and marketing capabilities or entering into strategic collaborations with third parties to leverage or access these capabilities;
- the amount and timing of sales and other revenues from our product candidates, if any are approved, including the sales price and the availability of adequate third-party reimbursement;
- the cash requirements of any future acquisitions or discovery of product candidates;
- the time and cost necessary to respond to technological and market developments;
- the costs of acquiring, licensing or investing in intellectual property rights, products, product candidates and businesses;
- our ability to attract, hire and retain qualified personnel; and
- the costs of maintaining, expanding and protecting our intellectual property portfolio.

Additional funds may not be available when we need them, on terms that are acceptable, or at all. If adequate funds are not available to us on a timely basis, we may be required to delay, limit or terminate one or more research or development programs or the commercialization of any product candidates or be unable to expand operations or otherwise capitalize on business opportunities, as desired, which could materially affect our business, prospects, financial condition and results of operations.

***Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights to current product candidates or to any future product candidates on unfavorable terms.***

We may seek additional capital through a combination of public and private equity offerings, debt financings, strategic partnerships and alliances and licensing arrangements. We, and indirectly, our stockholders, will bear the cost of issuing and servicing any such securities and of entering into and maintaining any such strategic partnerships or other arrangements. Because any decision by us to issue debt or equity securities in the future will depend on market conditions and other factors beyond our control, we cannot predict or estimate the amount, timing or nature of any future financing transactions. To the extent that we raise additional capital through the sale of equity or debt securities, your ownership interest will be diluted, and the terms may include liquidation or other preferences that adversely affect your rights as a stockholder. The incurrence of additional indebtedness would result in increased fixed payment obligations and could involve additional restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. Additionally, any future collaborations we enter into with third parties may provide capital in the near term, but limit our potential cash flow and revenue in the future. If we raise additional funds through strategic partnerships and alliances and licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies or product candidates, or grant licenses or other rights on unfavorable terms.

In addition, if one of our subsidiaries raises funds through the issuance of equity securities, and our stockholders' equity interest in such subsidiary could be substantially diminished. If one of our subsidiaries raises additional

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funds through collaboration and licensing arrangements, it may be necessary to relinquish some rights to our technologies or our product candidates or grant licenses on terms that are not favorable to us.

***If we engage in acquisitions or strategic partnerships, this may increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities, and subject us to other risks.***

We may engage in various acquisitions and strategic partnerships in the future, including licensing or acquiring complementary products, intellectual property rights, technologies, or businesses. Any acquisition or strategic partnership may entail numerous risks, including:

- increased operating expenses and cash requirements;
- the assumption of indebtedness or contingent liabilities;
- the issuance of our equity securities which would result in dilution to our stockholders;
- assimilation of operations, intellectual property, products and product candidates of an acquired company, including difficulties associated with integrating new personnel;
- the diversion of our management's attention from our existing product programs and initiatives in pursuing such an acquisition or strategic partnership;
- retention of key employees, the loss of key personnel, and uncertainties in our ability to maintain key business relationships;
- risks and uncertainties associated with the other party to such a transaction, including the prospects of that party and their existing products or product candidates and regulatory approvals; and
- our inability to generate revenue from acquired intellectual property, technology and/or products sufficient to meet our objectives or even to offset the associated transaction and maintenance costs.

In addition, if we undertake such a transaction, we may issue dilutive securities, assume or incur debt obligations, incur large one-time expenses and acquire intangible assets that could result in significant future amortization expense.

***If we obtain a controlling interest in additional companies in the future, it could adversely affect our operating results and the value of our common stock, thereby disrupting our business.***

As part of our strategy, we expect to form and invest in additional wholly-owned subsidiaries and variable interest entities, or VIEs. Investments in our existing and any future subsidiaries involve numerous risks, including, but not necessarily limited to:

- risk of conducting research and development activities in new therapeutic areas or treatment modalities in which we have little to no experience;
- diversion of financial and managerial resources from existing operations;
- successfully negotiating a proposed acquisition, in-license or investment in a timely manner and at a price or on terms and conditions favorable to us;
- successfully combining and integrating a potential acquisition into our existing business to fully realize the benefits of such acquisition;
- the impact of regulatory reviews on a proposed acquisition, in-license or investment; and
- the outcome of any legal proceedings that may be instituted with respect to the proposed acquisition, in-license or investment.

If we fail to properly evaluate potential acquisitions, in-licenses, investments or other transactions associated with the creation of new research and development programs or the maintenance of existing ones, we might not achieve the anticipated benefits of any such transaction, we might incur costs in excess of what we anticipate, and management resources and attention might be diverted from other necessary or valuable activities.

#### **Risks Related to our Business and the Clinical Development, Regulatory Review and Approval of our Product Candidates**

***Our product candidates are in preclinical or clinical development, which is a lengthy and expensive process with uncertain outcomes and the potential for substantial delays. We cannot give any assurance that any of our product candidates will receive regulatory approval, which is necessary before they can be commercialized.***

Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidates in humans. To date, we have focused substantially all of our efforts and financial resources on identifying, acquiring, and developing our product candidates, including conducting lead optimization, nonclinical studies, preclinical studies and clinical trials, and providing general and administrative support for these operations. We cannot be certain that any clinical trials will be conducted as planned or completed on schedule, if at all. Our inability to successfully complete preclinical and clinical development could result in additional costs to us and negatively impact our ability to generate revenue. Our future success is dependent on our ability to successfully develop, obtain regulatory approval for, and then successfully commercialize product candidates. We currently have no products approved for sale and have not generated any revenue from sales of drugs, and we may never be able to develop or successfully commercialize a marketable drug.

All of our product candidates require additional development; management of preclinical, clinical, and manufacturing activities; and regulatory approval. In addition, we will need to obtain adequate manufacturing supply; build a commercial organization; commence marketing efforts; and obtain reimbursement before we generate any significant revenue from commercial product sales, if ever. Many of our product candidates are in early-stage research or translational phases of development, and the risk of failure for these programs is high. We cannot be certain that any of our product candidates will be successful in clinical trials or receive regulatory approval. Further, our product candidates may not receive regulatory approval even if they are successful in clinical trials. If we do not receive regulatory approvals for our product candidates, we and our subsidiaries may not be able to continue operations, which may result in us dissolving the subsidiary, out-licensing the technology or pursuing an alternative strategy.

***If we are unable to obtain regulatory approval in one or more jurisdictions for any product candidates that we may identify and develop, our business will be substantially harmed.***

We cannot commercialize a product until the appropriate regulatory authorities have reviewed and approved the product candidate. Approval by the FDA and comparable foreign regulatory authorities is lengthy and unpredictable, and depends upon numerous factors, including substantial discretion of the regulatory authorities. Approval policies, regulations, or the type and amount of nonclinical or clinical data necessary to gain approval may change during the course of a product candidate's development and may vary among jurisdictions, which may cause delays in the approval or the decision not to approve an application. We have not obtained regulatory approval for any product candidates, and it is possible that our current product candidates and any other product candidates which we may seek to develop in the future will not ever obtain regulatory approval. We cannot be certain that any of our product candidates will receive regulatory approval or be successfully commercialized even if we receive regulatory approval.



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Obtaining marketing approval is an extensive, lengthy, expensive and inherently uncertain process, and regulatory authorities may delay, limit or deny approval of our product candidates for many reasons, including but not limited to:

- the inability to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that the applicable product candidate is safe and effective as a treatment for our targeted indications;
- the FDA or comparable foreign regulatory authorities may disagree with the design, endpoints or implementation of our clinical trials;
- the population studied in the clinical program may not be sufficiently broad or representative to assure safety or efficacy in the full population for which we seek approval;
- the FDA or comparable foreign regulatory authorities may require additional preclinical studies or clinical trials beyond those that we currently anticipate;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from nonclinical studies or clinical trials;
- the data collected from clinical trials of product candidates that we may identify and pursue may not be sufficient to support the submission of an NDA, biologics license application, or BLA, or other submission for regulatory approval in the United States or elsewhere;
- we may be unable to demonstrate to the FDA or comparable foreign regulatory authorities that a product candidate's risk-benefit ratio for its proposed indication is acceptable;
- the FDA or comparable foreign regulatory authorities may identify deficiencies in the manufacturing processes, test procedures and specifications, or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may change in a manner that renders the clinical trial design or data insufficient for approval.

The lengthy approval process, as well as the unpredictability of the results of clinical trials and evolving regulatory requirements, may result in our failure to obtain regulatory approval to market product candidates that we may pursue in the United States or elsewhere, which would significantly harm our business, prospects, financial condition and results of operations.

### ***We may encounter substantial delays in clinical trials, or may not be able to conduct or complete clinical trials on the expected timelines, if at all.***

Clinical testing is expensive, time consuming, and subject to uncertainty. We cannot guarantee that any of our ongoing and planned clinical trials will be conducted as planned or completed on schedule, if at all. Moreover, even if these trials are initiated or conducted on a timely basis, issues may arise that could result in the suspension or termination of such clinical trials. A failure of one or more clinical trials can occur at any stage of testing, and our ongoing and future clinical trials may not be successful. Events that may prevent successful or timely initiation or completion of clinical trials include:

- inability to generate sufficient preclinical, toxicology, or other *in vivo* or *in vitro* data to support the initiation or continuation of clinical trials;
- delays in confirming target engagement, patient selection or other relevant biomarkers to be utilized in preclinical and clinical product candidate development;
- delays in reaching a consensus with regulatory agencies as to the design or implementation of our clinical studies;
- delays in reaching agreement on acceptable terms with prospective contract research organizations, or CROs, and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and clinical trial sites;

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- delays in identifying, recruiting and training suitable clinical investigators;
- delays in obtaining required Institutional Review Board, or IRB, approval at each clinical trial site;
- imposition of a temporary or permanent clinical hold by regulatory agencies for a number of reasons, including after review of an IND or amendment, clinical trial application, or CTA, or amendment, or equivalent application or amendment; as a result of a new safety finding that presents unreasonable risk to clinical trial participants; a negative finding from an inspection of our clinical trial operations or study sites;
- developments in trials for other product candidates with the same targets or related modalities as our product candidates conducted by competitors that raise regulatory or safety concerns about risk to patients of the treatment; or if the FDA finds that the investigational protocol or plan is clearly deficient to meet its stated objectives;
- difficulties in securing access to materials for the comparator arm of certain of our clinical trials;
- delays in identifying, recruiting and enrolling suitable patients to participate in clinical trials, and delays caused by patients withdrawing from clinical trials or failing to return for post-treatment follow-up;
- difficulty collaborating with patient groups and investigators;
- failure by CROs, other third parties, or us to adhere to clinical trial requirements;
- failure to perform in accordance with the FDA's or any other regulatory authority's current good clinical practices, or GCP, requirements, or regulatory guidelines in other countries;
- occurrence of adverse events, or AEs, associated with the product candidate that are viewed to outweigh its potential benefits;
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols;
- changes in the standard of care on which a clinical development plan was based, which may require new or additional trials;
- the cost of clinical trials of any product candidates that we may identify and pursue being greater than we anticipate;
- clinical trials of any product candidates that we may identify and pursue producing negative or inconclusive results, which may result in our deciding, or regulators requiring us, to conduct additional clinical trials or abandon product development programs;
- transfer of manufacturing processes to larger-scale facilities operated by a CMO, or by us, and delays or failure by our CMOs or us to make any necessary changes to such manufacturing process; and
- delays in manufacturing, testing, releasing, validating, or importing/exporting sufficient stable quantities of product candidates that we may identify for use in clinical trials or the inability to do any of the foregoing.

Any inability to successfully initiate or complete clinical trials could result in additional costs to us or impair our ability to generate revenue. For example, on October 30, 2018, the FDA notified our subsidiary Phoenix Tissue Repair Inc. of a partial clinical hold, but allowed it to proceed with the planned Phase 1/2 study using only the existing drug substance of BBP-589 that was identified by the FDA. The FDA requested additional development of the analytical test method to quantitate relative potency of any new batch of product we intend to use for future clinical studies. Although we believe the existing product lot for BBP-589 identified in the IND, which is not subject to the partial clinical hold, is sufficient to complete our proposed Phase 1/2 clinical trial, we will need to reconcile the identified deficiency in the potency assay and provide the FDA with the requested information before we can release additional lots of BBP-589 for clinical use. We cannot assure you that the FDA will deem our response satisfactory to address its request and we may never be able to secure a release of the partial clinical hold. In addition, if we make manufacturing or formulation changes to our product candidates, we may be

required to or we may elect to conduct additional nonclinical studies or clinical trials to bridge data obtained from our modified product candidates to data obtained from nonclinical and clinical research conducted using earlier versions. Clinical trial delays could also shorten any periods during which our products have patent protection and may allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize product candidates and may harm our business and results of operations.

We could also encounter delays if a clinical trial is suspended or terminated by us, by the data safety monitoring board, or DSMB, including for our ongoing and planned Phase 3 clinical trials of BBP-265, our ongoing Phase 2 and planned Phase 3 clinical trials of BBP-831 and our ongoing Phase 3 clinical trial of BBP-009, or by the FDA or other regulatory authority, or if the IRBs of the institutions in which such trials are being conducted suspend or terminate the participation of their clinical investigators and sites subject to their review. Such authorities may suspend or terminate a clinical trial due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a product candidate, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

Moreover, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA or comparable foreign regulatory authorities. The FDA or comparable foreign regulatory authority may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the study. The FDA or comparable foreign regulatory authority may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA or comparable foreign regulatory authority, as the case may be, and may ultimately lead to the denial of marketing approval of one or more of our product candidates.

Delays in the initiation, conduct or completion of any clinical trial of our product candidates will increase our costs, slow down the product candidate development and approval process and delay or potentially jeopardize our ability to commence product sales and generate revenue. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates. In the event we identify any additional product candidates to pursue, we cannot be sure that submission of an IND or a CTA will result in the FDA or comparable foreign regulatory authority allowing clinical trials to begin in a timely manner, if at all. Any of these events could have a material adverse effect on our business, prospects, financial condition and results of operations.

***Our clinical trials may fail to demonstrate substantial evidence of the safety and effectiveness of product candidates that we may identify and pursue for their intended uses, which would prevent, delay or limit the scope of regulatory approval and commercialization.***

Before obtaining regulatory approvals for the commercial sale of any of our product candidates, we must demonstrate through lengthy, complex and expensive nonclinical studies, preclinical studies and clinical trials that the applicable product candidate is both safe and effective for use in each target indication, and in the case of our product candidates regulated as biological products, that the product candidate is safe, pure, and potent for use in its targeted indication. Each product candidate must demonstrate an adequate risk versus benefit profile in its intended patient population and for its intended use.

Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical development process. Most product candidates that begin clinical trials are never approved by regulatory authorities for commercialization. We have limited experience in designing clinical trials and may be unable to design and execute a clinical trial to support marketing approval.

We cannot be certain that our current clinical trials or any other future clinical trials will be successful. Additionally, any safety concerns observed in any one of our clinical trials in our targeted indications could limit the prospects for regulatory approval of our product candidates in those and other indications, which could have a material adverse effect on our business, financial condition and results of operations. In addition, even if such clinical trials are successfully completed, we cannot guarantee that the FDA or comparable foreign regulatory authorities will interpret the results as we do, and more trials could be required before we submit our product candidates for approval. This is particularly true for clinical trials in very rare diseases, such as with BBP-870 for MoCD Type A, where the very small patient population makes it difficult or impossible to conduct two traditional, adequate and well-controlled studies, and therefore the FDA or comparable foreign regulatory authorities are often required to exercise flexibility in approving therapies for such diseases. Moreover, results acceptable to support approval in one jurisdiction may be deemed inadequate by another regulatory authority to support regulatory approval in that other jurisdiction. To the extent that the results of the trials are not satisfactory to the FDA or comparable foreign regulatory authorities for support of a marketing application, we may be required to expend significant resources, which may not be available to us, to conduct additional trials in support of potential approval of our product candidates. For example, we intend to file an NDA for BBP-831 in second line and later advanced CCA with FGFR2 fusions or translocations in 2020. However, the FDA could disagree that data from our Phase 2 trial are sufficient to file an NDA or to approve BBP-831 for such an indication. Even if regulatory approval is secured for a product candidate, the terms of such approval may limit the scope and use of the specific product candidate, which may also limit its commercial potential.

***Results of earlier studies or clinical trials may not be predictive of future clinical trial results, and initial studies or clinical trials may not establish an adequate safety or efficacy profile for our product candidates to justify proceeding to advanced clinical trials or an application for regulatory approval.***

The results of nonclinical and preclinical studies and clinical trials may not be predictive of the results of later-stage clinical trials, and interim results of a clinical trial do not necessarily predict final results. In addition, for certain of our product candidates that we acquired, we did not undertake the preclinical studies and clinical trials. The results of preclinical studies and clinical trials in one set of patients or disease indications, or from preclinical studies or clinical trials that we did not lead, may not be predictive of those obtained in another. In some instances, there can be significant variability in safety or efficacy results between different clinical trials of the same product candidate due to numerous factors, including changes in trial procedures set forth in protocols, differences in the size and type of the patient populations, changes in and adherence to the dosing regimen and other clinical trial protocols and the rate of dropout among clinical trial participants. In addition, preclinical and clinical data are often susceptible to various interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy profile despite having progressed through nonclinical studies and initial clinical trials. A number of companies in the pharmaceutical and biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier studies, and we cannot be certain that we will not face similar setbacks. Even if early-stage clinical trials are successful, we may need to conduct additional clinical trials of our product candidates in additional patient populations or under different treatment conditions before we are able to seek approvals from the FDA and regulatory authorities outside the United States to market and sell these product candidates. Our failure to obtain marketing approval for our product candidates would substantially harm our business, prospects, financial condition and results of operations. For example, if BBP-265 is first approved for ATTR-CM on the basis of efficacy endpoints other than for reduction in mortality or hospitalization, BBP-265 might be limited to a second-line claim until such data were available. Any of these events could limit the commercial potential of BBP-265 and have a material adverse effect on our business, prospects, financial condition and results of operations.

Additionally, some of the clinical trials performed to date were generated from open-label studies and were conducted at a limited number of clinical sites on a limited number of patients. An “open-label” clinical trial is one where both the patient and investigator know whether the patient is receiving the investigational product

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candidate or either an existing approved drug or placebo. Most typically, open-label clinical trials test only the investigational product candidate and sometimes may do so at different dose levels. Open-label clinical trials are subject to various limitations that may exaggerate any therapeutic effect as patients in open-label clinical trials are aware when they are receiving treatment. Open-label clinical trials may be subject to a “patient bias” where patients perceive their symptoms to have improved merely due to their awareness of receiving an experimental treatment. Moreover, patients selected for early clinical studies often include the most severe sufferers and their symptoms may have been bound to improve notwithstanding the new treatment. In addition, open-label clinical trials may be subject to an “investigator bias” where those assessing and reviewing the physiological outcomes of the clinical trials are aware of which patients have received treatment and may interpret the information of the treated group more favorably given this knowledge. Given that our Phase 2 clinical trial of BBP-265 includes an open-label clinical trial extension, the results from this clinical trial may not be predictive of future clinical trial results with this or other product candidates for which we include an open-label clinical trial when studied in a controlled environment with a placebo or active control.

***We may encounter difficulties enrolling patients in clinical trials, and clinical development activities could thereby be delayed or otherwise adversely affected.***

The timely completion of clinical trials in accordance with their protocols depends, among other things, on our ability to enroll a sufficient number of patients who remain in the trial until its conclusion. The indications for which we plan to evaluate our current product candidates represent a rare disease or condition with limited patient populations from which to draw participants in clinical trials. Due to our focus on the development of product candidates for the treatment of Mendelian diseases and genetically driven cancers, many of which are rare conditions, we may not be able to identify and enroll a sufficient number of patients, or those with required or desired characteristics and criteria, in a timely manner.

We may experience difficulties in patient enrollment in our clinical trials for a variety of reasons, including:

- the size and nature of a patient population;
- the patient eligibility criteria defined in the applicable clinical trial protocols, which may limit the patient populations eligible for clinical trials to a greater extent than competing clinical trials for the same indication;
- the size of the study population required for analysis of the trial’s primary endpoints;
- the severity of the disease under investigation;
- the proximity of patients to a trial site;
- the design of the trial;
- the ability to recruit clinical trial investigators with the appropriate competencies and experience;
- the approval or concurrent enrollment of clinical trials involving competing product candidates currently under development for Mendelian diseases or genetically driven cancers, including Vyndamax (tafamidis) and Vyndaqel (tafamidis meglumine), for which Pfizer Inc. has been approved for the treatment of ATTR-CM in the United States and Japan (Vyndaqel only) and is approved in certain countries outside the United States for the treatment of ATTR-PN (Vyndaqel only), or competing clinical trials for similar therapies or targeting patient populations meeting our patient eligibility criteria;
- clinicians’ and patients’ perceptions as to the potential advantages and side effects of the product candidate being studied in relation to other available therapies and product candidates;
- the ability to obtain and maintain patient consents; and
- the risk that patients enrolled in clinical trials will not complete such trials, for any reason.

If we have difficulty enrolling sufficient numbers of patients to conduct clinical trials as planned, we may need to delay or terminate ongoing or planned clinical trials, either of which would have an adverse effect on our business.

***Use of our product candidates could be associated with side effects, adverse events or other properties or safety risks, which could delay or halt their clinical development, prevent their regulatory approval, cause us to suspend or discontinue clinical trials, abandon a product candidate, limit their commercial potential, if approved, or result in other significant negative consequences that could severely harm our business, prospects, operating results and financial condition.***

As is the case with pharmaceuticals generally, it is likely that there may be side effects and AEs associated with our product candidates' use. Results of our clinical trials could reveal a high and unacceptable severity and prevalence of side effects or unexpected characteristics. Undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or comparable foreign regulatory authorities. The drug-related side effects could affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims. Any of these occurrences may harm our business, financial condition and prospects significantly.

Moreover, if our product candidates are associated with undesirable side effects in preclinical studies or clinical trials or have characteristics that are unexpected, we may elect to abandon their development or limit their development to more narrow uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective, which may limit the commercial expectations for the product candidate if approved. We may also be required to modify or terminate our study plans based on findings in our preclinical studies or clinical trials. For instance, in our Phase 2 clinical trial of BPP-831 for the treatment of FGFR-driven cancers, the most commonly reported treatment emergent adverse event of any grade was hyperphosphatemia, which is an electrolyte disorder in which there is an elevated level of phosphate in the blood. Many product candidates that initially show promise in early-stage testing may later be found to cause side effects that prevent further development. In addition, in ongoing IND-enabling toxicology studies, we have observed toxicity in a non-rodent species for BBP-671. We believe the toxicity observed is consistent with a species and chemotype-specific mechanism and we are now instead pursuing two backup BBP-671 compounds. As we work to advance existing product candidates and to identify new product candidates, we cannot be certain that later testing or trials of product candidates that initially showed promise in early testing will not be found to cause similar or different unacceptable side effects that prevent their further development.

It is possible that as we test our product candidates in larger, longer and more extensive clinical trials, or as the use of these product candidates becomes more widespread if they receive regulatory approval, illnesses, injuries, discomforts and other AEs that were observed in earlier trials, as well as conditions that did not occur or went undetected in previous trials, will be reported by subjects. If such side effects become known later in development or upon approval, if any, such findings may harm our business, financial condition and prospects significantly.

Additionally, adverse developments in clinical trials of pharmaceutical and biopharmaceutical products conducted by others may cause the FDA or other regulatory oversight bodies to suspend or terminate our clinical trials or to change the requirements for approval of any of our product candidates.

In addition to side effects caused by the product candidate, the administration process or related procedures also can cause adverse side effects. If any such AEs occur, our clinical trials could be suspended or terminated. If we are unable to demonstrate that any AEs were caused by the administration process or related procedures, the FDA, the European Commission, the European Medicines Agency, or the EMA, or other regulatory authorities could order us to cease further development of, or deny approval of, a product candidate for any or all targeted indications. Even if we can demonstrate that all future serious adverse events, or SAEs, are not product-related, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the trial. Moreover, if we elect, or are required, to not initiate, delay, suspend or terminate any future clinical trial of any of our product candidates, the commercial prospects of such product candidates may be harmed and our ability to generate

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product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates, and may harm our business, financial condition and prospects significantly.

Additionally, if any of our product candidates receives marketing approval, the FDA could impose a boxed warning in the labeling of our product and could require us to adopt a risk evaluation and mitigation strategy, or REMS, and could apply elements to assure safe use to ensure that the benefits of the product outweigh its risks, which may include, among other things, a Medication Guide outlining the risks of the product for distribution to patients and a communication plan to health care practitioners. Furthermore, if we or others later identify undesirable side effects caused by our product candidates once approved, several potentially significant negative consequences could result, including:

- regulatory authorities may suspend or withdraw approvals of such product candidate;
- regulatory authorities may require additional warnings on the label;
- we may be required by the FDA to implement a REMS;
- we may be required to change the way a product candidate is administered or conduct additional clinical trials;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these occurrences could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, and may harm our business, financial condition and prospects significantly.

***Certain of our product candidates under development for the treatment of patient populations with significant comorbidities that may result in deaths or serious adverse or unacceptable side effects and require us to abandon or limit our clinical development activities.***

Patients in certain of our ongoing and planned clinical trials of product candidates in genetically driven cancers, including clinical trials of BBP-831 of FGFR-driven cancers, as well as patients who may undergo treatment with other product candidates that we may develop, may also receive chemotherapy, radiation, and/or other high dose or myeloablative treatments in the course of treatment of their disease, and may therefore experience side effects or AEs, including death, that are unrelated to our product candidates. While these side effects or AEs may be unrelated to our product candidates, they may still affect the success of our clinical trials. The inclusion of critically ill patients in our clinical trials may also result in deaths or other adverse medical events due to underlying disease or to other therapies or medications that such patients may receive. Any of these events could prevent us from advancing our product candidates through clinical development, and from obtaining regulatory approval, and would impair our ability to commercialize our product candidates. Any inability to advance our product candidates through clinical development would have a material adverse effect on our business, and the value of our common stock would decline.

***We may in the future conduct clinical trials for product candidates outside the United States, and the FDA and comparable foreign regulatory authorities may not accept data from such trials.***

We may in the future choose to conduct one or more clinical trials outside the United States, including in Europe. For instance, our clinical trials of BBP-831 and BBP-870 each included patients outside of the United States and our Phase 3 clinical trials of BBP-265 will include patients outside of the United States. The acceptance of study data from clinical trials conducted outside the United States or another jurisdiction by the FDA or comparable foreign regulatory authority may be subject to certain conditions or may not be accepted at all. In cases where data from foreign clinical trials are intended to serve as the basis for marketing approval in the United States, the FDA will generally not approve the application on the basis of foreign data alone unless (i) the data are

applicable to the U.S. population and U.S. medical practice; and (ii) the trials were performed by clinical investigators of recognized competence and pursuant to GCP regulations. Additionally, the FDA's clinical trial requirements, including sufficient size of patient populations and statistical powering, must be met. Many foreign regulatory authorities have similar approval requirements. In addition, such foreign trials would be subject to the applicable local laws of the foreign jurisdictions where the trials are conducted. There can be no assurance that the FDA or any comparable foreign regulatory authority will accept data from trials conducted outside of the United States or the applicable jurisdiction, including from our ongoing and planned Phase 3 clinical trials of BBP-265, for which we plan to enroll cohorts outside the United States. If the FDA or any comparable foreign regulatory authority does not accept such data, it would result in the need for additional trials, which would be costly and time-consuming and delay aspects of our business plan, and which may result in product candidates that we may develop not receiving approval or clearance for commercialization in the applicable jurisdiction.

***Even if we obtain FDA approval for product candidates that we may identify and pursue in the United States, we may never obtain approval to commercialize any product candidates outside of the United States, which would limit our ability to realize their full market potential.***

In order to market any products outside of the United States, we must establish and comply with numerous and varying regulatory requirements of other countries regarding safety and effectiveness. Clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not mean that regulatory approval will be obtained in any other country. Approval processes vary among countries and can involve additional product testing and validation and additional or different administrative review periods from those in the United States, including additional preclinical studies or clinical trials, as clinical trials conducted in one jurisdiction may not be accepted by regulatory authorities in other jurisdictions. In many jurisdictions outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that jurisdiction. In some cases, the price that we intend to charge for our products is also subject to approval.

Seeking foreign regulatory approval could result in difficulties and costs and require additional nonclinical studies or clinical trials which could be costly and time-consuming. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our product candidates in those countries. The foreign regulatory approval process may include all of the risks associated with obtaining FDA approval. We do not have any product candidates approved for sale in any jurisdiction, including international markets, and we do not have experience in obtaining regulatory approval in international markets. If we fail to comply with regulatory requirements in international markets or to obtain and maintain required approvals, or if regulatory approval in international markets is delayed, our target market will be reduced and our ability to realize the full market potential of our products will be harmed.

***Interim, "top-line," and preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available or as additional analyses are conducted, and as the data are subject to audit and verification procedures that could result in material changes in the final data.***

From time to time, we may publish interim, "top-line," or preliminary data from our clinical studies. Interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Preliminary or "top-line" data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, interim and preliminary data should be viewed with caution until the final data are available. Material adverse changes between preliminary, "top-line," or interim data and final data could significantly harm our business prospects.



***Even though we may apply for orphan drug designation for our product candidates, we may not be able to obtain orphan drug marketing exclusivity.***

Our business strategy focuses on the development of product candidates for the treatment of genetic diseases, which may be eligible for FDA or EMA orphan drug designation. Regulatory authorities in some jurisdictions, including the United States and European Union, may designate drugs or biologics for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a drug as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals annually in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In order to obtain orphan drug designation, the request must be made before submitting an NDA or BLA. In the United States, orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages, and user-fee waivers. After the FDA grants orphan drug designation, the generic identity of the drug and its potential orphan use are disclosed publicly by the FDA. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

If a product that has orphan drug designation subsequently receives the first FDA approval of that particular product for the disease for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications, including an NDA or BLA, to market the same drug or biologic for the same indication for seven years, except in limited circumstances such as a showing of clinical superiority to the product with orphan drug exclusivity or if FDA finds that the holder of the orphan drug exclusivity has not shown that it can assure the availability of sufficient quantities of the orphan drug to meet the needs of patients with the disease or condition for which the drug was designated. As a result, even if one of our product candidates receives orphan exclusivity, the FDA can still approve other drugs or biologics for use in treating the same indication or disease or the same biologic for a different indication or disease during the exclusivity period. Furthermore, the FDA can waive orphan exclusivity if we are unable to manufacture sufficient supply of our product or if a subsequent applicant demonstrates clinical superiority over our product.

In the European Union, the Committee for Orphan Medicinal Products of the EMA grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10,000 persons in the European Union (or where it is unlikely that the development of the medicine would generate sufficient return to justify the investment) and for which no satisfactory method of diagnosis, prevention, or treatment is authorized or, if a method exists, the product would be of significant benefit to those affected by the condition.

We have obtained from the FDA orphan drug designation for BBP-009 for treatment of nevoid basal cell carcinoma syndrome, or Gorlin syndrome, BBP-265 for the treatment of transthyretin amyloidosis, BBP-589 for the treatment of dystrophic epidermolysis bullosa, BBP-631 for the treatment of CAH 21OHD, BBP-587 for the treatment of dystrophic epidermolysis bullosa and BBP-870 for treatment of molybdenum cofactor deficiency type A. We have obtained from the EMA orphan drug designation for BBP-009 for treatment of nevoid basal cell carcinoma syndrome (Gorlin syndrome), BBP-265 for the treatment of ATTR amyloidosis, BBP-589 for the treatment of epidermolysis bullosa and BBP-870 for treatment of molybdenum cofactor deficiency type A. We may seek orphan drug designation for certain other of our product candidates. Even if we obtain orphan drug designation, exclusive marketing rights in the United States may be limited if we seek approval for an indication broader than the orphan designated indication and may be lost if the FDA later determines that the request for designation was materially defective or if we are unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition, or if a subsequent applicant demonstrates clinical superiority over our products, if approved. In addition, although we may seek orphan drug designation for other product candidates, we may never receive such designations.

***Certain of our product candidates, including our protein therapeutic and gene therapy product candidates are novel, complex and difficult to manufacture. We could experience manufacturing problems that result in delays in our development or commercialization programs or otherwise harm our business.***

The manufacturing processes our CMOs use to produce our product candidates, including our protein therapeutic and gene therapy product candidates, are complex, novel and have not been validated for commercial use. Several factors could cause production interruptions, including equipment malfunctions, facility contamination, raw material shortages or contamination, natural disasters, disruption in utility services, human error or disruptions in the operations of our suppliers.

Several of our small molecule product candidates are particularly complex and difficult to manufacture, in some cases due to the number of steps required, the process complexity and the toxicity of end or intermediate-stage products.

Our protein therapeutic and gene therapy product candidates require processing steps that are more complex than those required for most small molecule drugs. Moreover, unlike small molecules, the physical and chemical properties of biologics such as ours generally cannot be fully characterized. As a result, assays of the finished product may not be sufficient to ensure that the product is consistent from lot-to-lot or will perform in the intended manner. Accordingly, our CMOs must employ multiple steps to control the manufacturing process to assure that the process is reproducible and the product candidate is made strictly and consistently in compliance with the process. Problems with the manufacturing process, even minor deviations from the normal process, could result in product defects or manufacturing failures that result in lot failures, product recalls, product liability claims or insufficient inventory to conduct clinical trials or supply commercial markets. We may encounter problems achieving adequate quantities and quality of clinical-grade materials that meet the FDA, the EMA or other applicable standards or specifications with consistent and acceptable production yields and costs.

In addition, the FDA, the EMA and other foreign regulatory authorities may require us to submit samples of any lot of any approved product together with the protocols showing the results of applicable tests at any time. Under some circumstances, the FDA, the EMA or other foreign regulatory authorities may require that we not distribute a lot until the agency authorizes its release. Slight deviations in the manufacturing process, including those affecting quality attributes and stability, may result in unacceptable changes in the product that could result in lot failures or product recalls. Lot failures or product recalls could cause us to delay product launches or clinical trials, which could be costly to us and otherwise harm our business, financial condition, results of operations and prospects.

Our CMOs also may encounter problems hiring and retaining the experienced scientific, quality assurance, quality-control and manufacturing personnel needed to operate our manufacturing processes, which could result in delays in production or difficulties in maintaining compliance with applicable regulatory requirements.

Any problems in our CMOs' manufacturing process or facilities could result in delays in planned clinical trials and increased costs, and could make us a less attractive collaborator for potential partners, including larger biotechnology companies and academic research institutions, which could limit access to additional attractive development programs. Problems in our manufacturing process could restrict our ability to meet potential future market demand for products.

***Certain of our product candidates are based on a novel AAV, gene therapy technology with which there is limited clinical or regulatory experience to date, which makes it difficult to predict the time and cost of product candidate development and subsequently obtaining regulatory approval.***

Certain of our product candidates are based on gene therapy technology and our future success depends on the successful development of this novel therapeutic approach. We cannot assure you that any development problems we or other gene therapy companies experience in the future related to gene therapy technology will not cause

significant delays or unanticipated costs in the development of our product candidates, or that such development problems can be solved. In addition, the clinical study requirements of the FDA, EMA and other regulatory agencies and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential products. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or extensively studied therapeutic modalities. Further, as we are developing novel treatments for diseases in which there is limited clinical experience with new endpoints and methodologies, there is heightened risk that the FDA, EMA or comparable foreign regulatory bodies may not consider the clinical trial endpoints to provide clinically meaningful results, and the resulting clinical data and results may be more difficult to analyze. To date, few gene therapy products have been approved by the FDA or comparable foreign regulatory authorities, which makes it difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in the United States, the European Union or other jurisdictions. Further, approvals by one regulatory agency may not be indicative of what other regulatory agencies may require for approval.

Regulatory requirements governing gene therapy products have evolved and may continue to change in the future. For example, the FDA established the Office of Tissues and Advanced Therapies within its Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. These and other regulatory review agencies, committees and advisory groups and the requirements and guidelines they promulgate may lengthen the regulatory review process, require us to perform additional preclinical studies or clinical trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these treatment candidates or lead to significant post-approval limitations or restrictions.

The FDA, National Institutes of Health, or NIH, other regulatory agencies at both the federal and state level in the United States, U.S. congressional committees, and the EMA and other foreign governments, have expressed interest in further regulating the biotechnology industry, including gene therapy and genetic testing. For example, the EMA advocates a risk-based approach to the development of a gene therapy product. Any such further regulation may delay or prevent commercialization of some or all of our product candidates. For example, in 1999, a patient died during a gene therapy clinical trial that utilized an adenovirus vector and it was later discovered that adenoviruses could generate an extreme immune system reaction that can be life-threatening. In January 2000, the FDA halted that trial and began investigating 69 other gene therapy trials underway in the United States, 13 of which required remedial action. In 2003, the FDA suspended 27 additional gene therapy trials involving several hundred patients after learning that some patients treated in a clinical trial in France had subsequently developed leukemia. While the new AAV vectors that we use across our portfolio of gene therapy product candidates have been designed and developed to help reduce these side effects, gene therapy is still a relatively new approach to disease treatment and past as well as different adverse side effects could develop.

Regulatory requirements in the United States and abroad governing gene therapy products have changed frequently and may continue to change in the future. For example, in addition to the submission of an IND, to the FDA, before initiation of a clinical trial in the United States, certain human clinical trials for cell therapy products and gene therapy had historically been subject to review by the NIH Office of Biotechnology Activities' Recombinant DNA Advisory Committee, or the RAC, pursuant to the NIH Guidelines for Research Involving Recombinant DNA Molecules, or NIH Guidelines. Following an initial review, RAC members would make a recommendation as to whether the protocol raises important scientific, safety, medical, ethical or social issues that warrant in-depth discussion at the RAC's quarterly meetings. Although the FDA decides whether individual gene therapy protocols may proceed under an IND, the RAC's recommendations were shared with the FDA and, the RAC public review process, if undertaken, could have impeded or delayed the initiation of a clinical trial, even if the FDA has reviewed the trial and approved its initiation or has notified the sponsor that the study may begin. Conversely, the FDA can put an IND on clinical hold even if the RAC provided a favorable review or has recommended against an in-depth, public review.

On August 17, 2018, the NIH issued a notice in the Federal Register and issued a public statement proposing changes to the oversight framework for gene therapy trials, including changes to the applicable NIH Guidelines to modify the roles and responsibilities of the RAC with respect to human clinical trials of gene therapy products, and requesting public comment on its proposed modifications. During the public comment period, which closed on October 16, 2018, the NIH had announced that it would no longer accept new human gene transfer protocols for review as part of the protocol registration process under the existing NIH Guidelines or convene the RAC to review individual clinical protocols. In April 2019, NIH announced the updated guidelines, which reflect these proposed changes, and clarify that these trials will remain subject to the FDA's oversight and other clinical trial regulations, and oversight at the local level will continue as otherwise set forth in the NIH Guidelines. Specifically, under the NIH Guidelines, supervision of human gene transfer trials includes evaluation and assessment by an IBC, a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. The IBC assesses the safety of the research and identifies any potential risk to the public health or the environment, and such review may result in some delay before initiation of a clinical trial. While the NIH Guidelines are not mandatory unless the research in question is being conducted at or sponsored by institutions receiving NIH funding of recombinant or synthetic nucleic acid molecule research, many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them. Even though we may not be required to submit a protocol for our gene therapy product candidates through the NIH for RAC review, we will still be subject to significant regulatory oversight by the FDA, and in addition to the government regulators, the applicable IBC and IRB, of each institution at which we or our collaborators conduct clinical trials of our product candidates, or a central IRB if appropriate, would need to review and approve the proposed clinical trial.

Similarly, the EMA governs the development of gene therapies in the European Union and may issue new guidelines concerning the development and marketing authorization for gene therapy products and require that we comply with these new guidelines.

These regulatory review committees and advisory groups and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies or trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our product candidates or lead to significant post-approval limitations or restrictions. As we advance our product candidates, we will be required to consult with these regulatory and advisory groups and comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of such product candidates. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. Delays as a result of an increased or lengthier regulatory approval process or further restrictions on the development of our product candidates can be costly and could negatively impact our ability to complete clinical trials and commercialize our current and future product candidates in a timely manner, if at all.

***Our product candidates based on gene therapy technology may cause undesirable and unforeseen side effects or be perceived by the public as unsafe, which could delay or prevent their advancement into clinical trials or regulatory approval, limit the commercial potential or result in significant negative consequences.***

Public attitudes may be influenced by claims that gene therapy technology is unsafe, unethical, or immoral, and, consequently, our product candidates may not gain the acceptance of the public or the medical community. Adverse public attitudes may adversely impact our ability to enroll clinical trials. Moreover, our success will depend upon physicians prescribing, and their patients being willing to receive, treatments that involve the use of product candidates we may develop in lieu of, or in addition to, existing treatments with which they are already familiar and for which greater clinical data may be available. For example, there have been several significant adverse side effects in prior clinical trials of gene therapy product candidates, including reported cases of leukemia and death seen in other trials using other vectors. While new AAV vectors have been developed to reduce these side effects, gene therapy is still a relatively new approach to disease treatment and additional adverse side effects could develop. There also is the potential risk of delayed AEs following exposure to gene therapy products due to persistent biologic activity of the genetic material or other components of products used to carry the genetic material.

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Possible adverse side effects that could occur with treatment with gene therapy products include an immunologic reaction early after administration which could be detrimental to the patient's health or substantially limit the effectiveness and durability of the treatment. For example, an increasingly anticipated side effect of AAV gene therapy is the development of a T-cell immunological response, most often seen affecting the liver.

***The FDA has granted rare pediatric disease designation to BBP-870 for the treatment of molybdenum cofactor deficiency type A. However, a marketing application for BBP-870, if approved, may not meet the eligibility criteria for a priority review voucher.***

The FDA has granted rare pediatric disease designation to BBP-870 for the treatment of molybdenum cofactor deficiency type A, or MoCD Type A. Designation of a drug as a drug for a rare pediatric disease does not guarantee that an NDA for such drug will meet the eligibility criteria for a rare pediatric disease priority review voucher at the time the application is approved. Under the Federal Food, Drugs, and Cosmetic Act, or FDCA, we will need to request a rare pediatric disease priority review voucher in our original NDA for BBP-870. The FDA may determine that an NDA for BBP-870, if approved, does not meet the eligibility criteria for a priority review voucher, including for the following reasons:

- MoCD Type A no longer meets the definition of a rare pediatric disease;
- the NDA contains an active ingredient (including any ester or salt of the active ingredient) that has been previously approved in an NDA;
- the NDA is not deemed eligible for priority review;
- the NDA does not rely on clinical data derived from studies examining a pediatric population and dosages of the drug intended for that population (that is, if the NDA does not contain sufficient clinical data to allow for adequate labeling for use by the full range of affected pediatric patients); or
- the NDA is approved for a different adult indication than the rare pediatric disease for which BBP-870 is designated (for example, if BBP-870 is approved for an indication based on specific genetic alterations that would be inclusive of, but not limited to, BBP-870).

The authority for the FDA to award rare pediatric disease priority review vouchers for drugs that have received rare pediatric disease designation prior to September 30, 2020 currently expires on September 30, 2022. If the NDA for BBP-870 is not approved prior to September 30, 2022 for any reason, regardless of whether it meets the criteria for a rare pediatric disease priority review voucher, it will not be eligible for a priority review voucher. However, it is also possible the authority for FDA to award rare pediatric disease priority review vouchers will be further extended through Federal lawmaking.

***We may not elect or be able to take advantage of any expedited development or regulatory review and approval processes available to product candidates granted breakthrough therapy or fast track designation by the FDA.***

We intend to evaluate and continue ongoing discussions with the FDA on regulatory strategies that could enable us to take advantage of expedited development pathways for certain of our product candidates, although we cannot be certain that our product candidates will qualify for any expedited development pathways or that regulatory authorities will grant, or allow us to maintain, the relevant qualifying designations. Potential expedited development pathways that we could pursue include breakthrough therapy and fast track designation.

Breakthrough therapy designation is intended to expedite the development and review of product candidates that are designed to treat serious or life-threatening diseases when "preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development." The designation of a product candidate as a breakthrough therapy provides potential benefits that include more frequent meetings with FDA to discuss the development plan for the product candidate and ensure collection of appropriate data needed

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to support approval; more frequent written correspondence from FDA about such things as the design of the proposed clinical trials and use of biomarkers; intensive guidance on an efficient drug development program, beginning as early as Phase 1; organizational commitment involving senior managers; and eligibility for rolling review and priority review.

Fast track designation is designed for product candidates intended for the treatment of a serious or life-threatening disease or condition, where nonclinical or clinical data demonstrate the potential to address an unmet medical need for this disease or condition.

Although BBP-589 has received fast track designation for the treatment of dystrophic epidermolysis bullosa, or DEB, BBP-870 has received breakthrough therapy designation for MoCD and BBP-009 has received breakthrough therapy designation for the reduction of life-long, serious clinical morbidity and disease burden of persistently developing BCCs in patients with basal cell nevus syndrome, or BCNS, which is also known as Gorlin Syndrome, we may elect not to pursue either of breakthrough therapy or fast track designation for our other product candidates, and the FDA has broad discretion whether or not to grant these designations.

Accordingly, even if we believe a particular product candidate is eligible for breakthrough therapy or fast track designation, we cannot assure you that the FDA would decide to grant it. Breakthrough therapy designation and fast track designation do not change the standards for product approval, and there is no assurance that such designation or eligibility will result in expedited review or approval or that the approved indication will not be narrower than the indication covered by the breakthrough therapy designation or fast track designation. Thus, even if we do receive breakthrough therapy or fast track designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may withdraw breakthrough therapy or fast track designation if it believes that the product no longer meets the qualifying criteria. Our business may be harmed if we are unable to avail ourselves of these or any other expedited development and regulatory pathways.

***If we are unable to successfully validate, develop and obtain regulatory approval for companion diagnostic tests for our drug candidates that require or would commercially benefit from such tests, or experience significant delays in doing so, we may not realize the full commercial potential of these drug candidates.***

In connection with the clinical development of our drug candidates for certain indications, we may work with collaborators to develop or obtain access to *in vitro* companion diagnostic tests to identify patient subsets within a disease category who may derive selective and meaningful benefit from our drug candidates. For example, we are currently developing a companion diagnostic for BBP-831 in patients with CCA in collaboration with Foundation Medicine, or FMI. Such companion diagnostics would be used during our clinical trials as well as in connection with the commercialization of our product candidates. To be successful, we or our collaborators will need to address a number of scientific, technical, regulatory and logistical challenges. The FDA and comparable foreign regulatory authorities regulate *in vitro* companion diagnostics as medical devices and, under that regulatory framework, will likely require the conduct of clinical trials to demonstrate the safety and effectiveness of any diagnostics we may develop, which we expect will require separate regulatory clearance or approval prior to commercialization.

We may rely on third parties for the design, development and manufacture of companion diagnostic tests for our therapeutic drug candidates that may require such tests. If we enter into such collaborative agreements, we will be dependent on the sustained cooperation and effort of our future collaborators in developing and obtaining approval for these companion diagnostics. It may be necessary to resolve issues such as selectivity/specificity, analytical validation, reproducibility, or clinical validation of companion diagnostics during the development and regulatory approval processes. Moreover, even if data from preclinical studies and early clinical trials appear to support development of a companion diagnostic for a product candidate, data generated in later clinical trials may fail to support the analytical and clinical validation of the companion diagnostic. We and our future collaborators may encounter difficulties in developing, obtaining regulatory approval for, manufacturing and commercializing companion diagnostics similar to those we face with respect to our therapeutic candidates themselves, including issues with achieving regulatory clearance or approval, production of sufficient quantities at commercial scale and with appropriate quality standards, and in gaining market acceptance. If we are unable to

successfully develop companion diagnostics for these therapeutic drug candidates, or experience delays in doing so, the development of these therapeutic drug candidates may be adversely affected, these therapeutic drug candidates may not obtain marketing approval, and we may not realize the full commercial potential of any of these therapeutics that obtain marketing approval. As a result, our business, results of operations and financial condition could be materially harmed. In addition, a diagnostic company with whom we contract may decide to discontinue selling or manufacturing the companion diagnostic test that we anticipate using in connection with development and commercialization of our product candidates or our relationship with such diagnostic company may otherwise terminate. We may not be able to enter into arrangements with another diagnostic company to obtain supplies of an alternative diagnostic test for use in connection with the development and commercialization of our product candidates or do so on commercially reasonable terms, which could adversely affect and/or delay the development or commercialization of our therapeutic candidates.

***If approved, our investigational products regulated as biologics may face competition from biosimilars approved through an abbreviated regulatory pathway.***

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively the ACA, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCIA, which created an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product. Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a BLA for the competing product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity, and potency of the other company's product. The law is complex and is still being interpreted and implemented by the FDA. As a result, its ultimate impact, implementation, and meaning are subject to uncertainty.

We believe that any of our product candidates approved as a biological product under a BLA should qualify for the 12-year period of exclusivity. However, there is a risk that this exclusivity could be shortened due to congressional action or otherwise, or that the FDA will not consider our investigational medicines to be reference products for competing products, potentially creating the opportunity for generic competition sooner than anticipated. Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. Moreover, the extent to which a biosimilar, once licensed, will be substituted for any one of our reference products in a way that is similar to traditional generic substitution for non-biological products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing.

If competitors are able to obtain marketing approval for biosimilars referencing our products, our products may become subject to competition from such biosimilars, with the attendant competitive pressure and consequences.

***Even if we obtain regulatory approval for a product candidate, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with our product candidates.***

If any of our product candidates are approved, they will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping, conduct of post-marketing studies, and submission of safety, efficacy, and other post-market information, including both federal and state requirements in the United States and requirements of comparable foreign regulatory authorities.

Manufacturers and manufacturers' facilities are required to comply with extensive requirements imposed by the FDA and comparable foreign regulatory authorities, including ensuring that quality control and manufacturing

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procedures conform to current good manufacturing practices, or cGMP, regulations. As such, we and our CMOs will be subject to continual review and inspections to assess compliance with cGMP and adherence to commitments made in any NDA, BLA or marketing authorization application, or MAA. Accordingly, we and others with whom we work must continue to expend time, money, and effort in all areas of regulatory compliance, including manufacturing, production and quality control.

Any regulatory approvals that we may receive for our product candidates will be subject to limitations on the approved indicated uses for which the product may be marketed and promoted or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials and surveillance to monitor the safety and efficacy of the product candidate. We will be required to report certain adverse reactions and production problems, if any, to the FDA and comparable foreign regulatory authorities. Any new legislation addressing drug safety issues could result in delays in product development or commercialization, or increased costs to assure compliance.

The FDA and other agencies, including the Department of Justice, closely regulate and monitor the post-approval marketing, labeling, advertising and promotion of products to ensure that they are manufactured, marketed and distributed only for the approved indications and in accordance with the provisions of the approved label. We will have to comply with requirements concerning advertising and promotion for our products. Promotional communications with respect to prescription drugs are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved label. As such, we may not promote our products for indications or uses for which they do not have approval.

The holder of an approved NDA, BLA or MAA must submit new or supplemental applications and obtain approval for certain changes to the approved product, product labeling, or manufacturing process. We could also be asked to conduct post-marketing clinical trials to verify the safety and efficacy of our products in general or in specific patient subsets. If original marketing approval was obtained via the accelerated approval pathway, we could be required to conduct a successful post-marketing clinical trial to confirm clinical benefit for our products. An unsuccessful post-marketing study or failure to complete such a study could result in the withdrawal of marketing approval.

If a regulatory agency discovers previously unknown problems with a product, such as AEs of unanticipated severity or frequency, or problems with the facility where the product is manufactured, or disagrees with the promotion, marketing or labeling of a product, such regulatory agency may impose restrictions on that product or us, including requiring withdrawal of the product from the market. If we fail to comply with applicable regulatory requirements, a regulatory agency or enforcement authority may, among other things:

- issue warning letters that would result in adverse publicity;
- impose civil or criminal penalties;
- suspend or withdraw regulatory approvals;
- suspend any of our ongoing clinical trials;
- refuse to approve pending applications or supplements to approved applications submitted by us;
- impose restrictions on our operations, including closing our CMOs' facilities;
- seize or detain products; or
- require a product recall.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response, and could generate negative publicity. Any failure to comply with ongoing regulatory requirements may significantly and adversely affect our ability to commercialize and generate revenue from our products. If regulatory sanctions are applied or if regulatory approval is withdrawn, the value of our company and our operating results will be adversely affected.



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The FDA's and other regulatory authorities' policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates.

We also cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. For example, certain policies of the Trump administration may impact our business and industry. Namely, the Trump administration has taken several executive actions, including the issuance of a number of Executive Orders, that could impose significant burdens on, or otherwise materially delay, the FDA's ability to engage in routine regulatory and oversight activities such as implementing statutes through rulemaking, issuance of guidance, and review and approval of marketing applications. If these executive actions impose constraints on FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted.

### ***The FDA and other regulatory agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses.***

If any of our product candidates are approved and we are found to have improperly promoted off-label uses of those products, we may become subject to significant liability. The FDA and other regulatory agencies strictly regulate the promotional claims that may be made about prescription products, if approved. In particular, while FDA permits the dissemination of truthful and non-misleading information about an approved product, a manufacturer may not promote a product for uses that are not approved by the FDA or such other regulatory agencies as reflected in the product's approved labeling. If we are found to have promoted such off-label uses, we may become subject to significant liability. The federal government has levied large civil and criminal fines against companies for alleged improper promotion of off-label use and has enjoined several companies from engaging in off-label promotion. The FDA has also requested that companies enter into consent decrees, corporate integrity agreements or permanent injunctions under which specified promotional conduct must be changed or curtailed. If we cannot successfully manage the promotion of our product candidates, if approved, we could become subject to significant liability, which would materially adversely affect our business and financial condition.

### **Risks Related to Reliance on Third Parties**

***We expect to rely on third parties to conduct our clinical trials and some aspects of our research and preclinical testing, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials, research, or testing.***

We currently rely and expect to continue to rely on third parties, such as CROs, clinical data management organizations, medical institutions, and clinical investigators, to conduct some aspects of research and preclinical testing and clinical trials. Any of these third parties may terminate their engagements with us or be unable to fulfill their contractual obligations. If any of our relationships with these third parties terminate, we may not be able to enter into arrangements with alternative third parties on commercially reasonable terms, or at all. If we need to enter into alternative arrangements, it would delay product development activities.

Our reliance on these third parties for research and development activities reduces control over these activities but does not relieve us of our responsibilities. For example, we remain responsible for ensuring that each of our respective clinical trials is conducted in accordance with the general investigational plan and protocols for the trial and applicable legal, regulatory, and scientific standards, and our reliance on third parties does not relieve us of our regulatory responsibilities. In addition, the FDA and comparable foreign regulatory authorities require compliance with GCPs for conducting, recording, and reporting the results of clinical trials to assure that data and reported results are credible, reproducible and accurate and that the rights, integrity, and confidentiality of trial participants are protected. Regulatory authorities enforce these GCPs through periodic inspections of trial sponsors, principal investigators, and trial sites. If we or any of these third parties fail to comply with applicable GCP regulations, some or all of the clinical data generated in our clinical trials may be deemed unreliable and the

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FDA or comparable foreign regulatory authorities may require us to perform additional nonclinical or clinical trials or to enroll additional patients before approving our marketing applications. We cannot be certain that, upon inspection, such regulatory authorities will determine that any of our clinical trials complies with the GCP regulations. For any violations of laws and regulations during the conduct of clinical trials, we could be subject to untitled and warning letters or enforcement action that may include civil penalties up to and including criminal prosecution. We also are required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database within certain timeframes. Failure to do so can result in fines, adverse publicity, and civil and criminal sanctions.

If these third parties do not successfully carry out their contractual duties, meet expected deadlines, or conduct clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, marketing approvals for any product candidates we may develop and will not be able to, or may be delayed in our efforts to, successfully commercialize our medicines. Our failure or the failure of these third parties to comply applicable regulatory requirements or our stated protocols could also subject us to enforcement action.

We also expect to rely on other third parties to store and distribute drug supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing approval of any product candidates we may develop or commercialization of our medicines, producing additional losses and depriving us of potential product revenue.

***We rely entirely on third parties for the manufacturing of our product candidates or other product candidates that we may develop for preclinical studies and clinical trials and expect to continue to do so for commercialization. Our business could be harmed if those third parties fail to provide us with sufficient quantities of drug product, or fail to do so at acceptable quality levels or prices.***

We do not currently have, nor do we plan to acquire, the infrastructure or capability internally to manufacture drug supplies for our ongoing clinical trials or any future clinical trials that we may conduct, and we lack the resources to manufacture any product candidates on a commercial scale. We rely, and expect to continue to rely, on third-party manufacturers to produce our current product candidates or other product candidates that we may identify for clinical trials, as well as for commercial manufacture if any product candidates that receive marketing approval. Although we generally do not begin a clinical trial unless we believe we have a sufficient supply of a product candidate to complete the trial, any significant delay or discontinuity in the supply of a product candidate, or the raw material components thereof, for an ongoing clinical trial due to the need to replace a third-party manufacturer could considerably delay the clinical development and potential regulatory approval of our product candidates, which could harm our business and results of operations. We also expect to rely primarily on third parties for the manufacturing of commercial supply of our product candidates, if approved.

We may be unable to identify and appropriately qualify third-party manufacturers or establish agreements with third-party manufacturers or do so on acceptable terms. Even if we are able to establish agreements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

- reliance on the third party for sourcing of raw materials, components, and such other goods as may be required for execution of its manufacturing processes and the oversight by the third party of its suppliers;
- reliance on the third party for regulatory compliance and quality assurance for the manufacturing activities each performs;
- the possible breach of the manufacturing agreement by the third party;
- the possible misappropriation of proprietary information, including trade secrets and know-how; and
- the possible termination or non-renewal of the agreement by the third party at a time that is costly or inconvenient for us.

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Furthermore, all of our CMOs are engaged with other companies to supply and/or manufacture materials or products for such companies, which exposes our manufacturers to regulatory risks for the production of such materials and products. The facilities used by our contract manufacturers to manufacture our product candidates are subject to review by the FDA pursuant to inspections that will be conducted after we submit an NDA or BLA to the FDA. We do not control the manufacturing process of, and are completely dependent on, our contract manufacturing partners for compliance with the regulatory requirements, known as current good manufacturing practice, or cGMP, requirements for manufacture of drug and biologic products. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or others, we will not be able to secure or maintain regulatory approval for our product candidates manufactured at these manufacturing facilities. In addition, we have no control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or a comparable foreign regulatory agency does not approve these facilities for the manufacture of our product candidates or if any agency withdraws its approval in the future, we may need to find alternative manufacturing facilities, which would negatively impact the ability to develop, obtain regulatory approval for or market our product candidates, if approved.

Our product candidates may compete with other product candidates and marketed drugs for access to manufacturing facilities. Any performance failure on the part of our existing or future manufacturers could delay clinical development, marketing approval or commercialization. Our current and anticipated future dependence upon others for the manufacturing of our product candidates may adversely affect our future profit margins and our ability to commercialize any product candidates that receive marketing approval on a timely and competitive basis.

***The drug substance and drug product for certain of our product candidates are currently acquired from single-source suppliers. The loss of these suppliers, or their failure to supply us with the drug substance or drug product, could materially and adversely affect our business.***

The drug substance and drug product for certain of our product candidates, including Veratrum californicum, or corn lily, from which we obtain cyclopamine for BBP-009, are grown or manufactured by single-source suppliers or CMOs under development and manufacturing contracts and services and quality agreements and purchase orders. We do not currently have any other suppliers for the drug substance or drug product of these product candidates and, although we believe that there are alternate sources of supply that could satisfy our clinical and commercial requirements, we cannot assure you that identifying alternate sources and establishing relationships with such sources would not result in significant delay in the development of our product candidates.

Our dependence on single-source suppliers exposes us to certain risks, including the following:

- our suppliers may cease or reduce production or deliveries, raise prices or renegotiate terms;
- delays caused by supply issues may harm our reputation; and
- our ability to progress our business could be materially and adversely impacted if our single-source suppliers upon which we rely were to experience a significant business challenges, disruption or failures due to issues such as financial difficulties or bankruptcy, issues relating regulatory or quality compliance issues, or other legal or reputational issues.

Additionally, we may not be able to enter into supply arrangements with alternative suppliers on commercially reasonable terms, or at all. A delay in the development of our product candidates or having to enter into a new agreement with a different third party on less favorable terms than we have with our current suppliers could have a material adverse impact upon on our business.

***If the contract manufacturing facilities on which we rely do not continue to meet regulatory requirements or are unable to meet our supply demands, our business will be harmed.***

All entities involved in the preparation of product candidates for clinical trials or commercial sale, including our existing CMOs for all of our product candidates, are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in late-stage clinical trials must be manufactured in accordance with cGMP, or similar regulatory requirements outside the United States. These regulations govern manufacturing processes and procedures, including recordkeeping, and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of contaminants or to inadvertent changes in the properties or stability of our product candidates. Our failure, or the failure of third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, suspension of production, seizures or recalls of product candidates or marketed drugs, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect clinical or commercial supplies of our product candidates.

We or our CMOs must supply all necessary documentation in support of an NDA, BLA or MAA on a timely basis and must adhere to regulations enforced by the FDA and other regulatory agencies through their facilities inspection program. Some of our CMOs have never produced a commercially approved pharmaceutical product and therefore have not obtained the requisite regulatory authority approvals to do so. The facilities and quality systems of some or all of our third-party contractors must pass a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of our product candidates or any of our other potential products. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our product candidates or our other potential products or the associated quality systems for compliance with the regulations applicable to the activities being conducted. Although we oversee the CMOs, we cannot control the manufacturing process of, and are completely dependent on, our CMO partners for compliance with the regulatory requirements. If these facilities do not pass a pre-approval plant inspection, regulatory approval of the products may not be granted or may be substantially delayed until any violations are corrected to the satisfaction of the regulatory authority, if ever.

The regulatory authorities also may, at any time following approval of a product for sale, audit the manufacturing facilities of our third-party contractors. If any such inspection or audit identifies a failure to comply with applicable regulations or if a violation of our product specifications or applicable regulations occurs independent of such an inspection or audit, we or the relevant regulatory authority may require remedial measures that may be costly and/or time consuming for us or a third party to implement, and that may include the temporary or permanent suspension of a clinical study or commercial sales or the temporary or permanent closure of a facility. Any such remedial measures imposed upon us or third parties with whom we contract could materially harm our business.

Additionally, if supply from one approved manufacturer is interrupted, an alternative manufacturer would need to be qualified through an NDA, BLA supplement or MAA variation, or equivalent foreign regulatory filing, which could result in further delay. The regulatory agencies may also require additional studies if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and is likely to result in a delay in our desired clinical and commercial timelines.

These factors could cause us to incur higher costs and could cause the delay or termination of clinical trials, regulatory submissions, required approvals, or commercialization of our product candidates. Furthermore, if our suppliers fail to meet contractual requirements and we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our clinical trials may be delayed or we could lose potential revenue.

***Collaborative relationships with third parties could cause us to expend significant resources and incur substantial business risk with no assurance of financial return.***

We anticipate relying upon strategic collaborations for marketing and commercializing our existing product candidates, and we may rely even more on strategic collaborations for R&D of other product candidates. We may sell product offerings through strategic partnerships with pharmaceutical and biotechnology companies. If we are unable to establish or manage such strategic collaborations on terms favorable to us in the future, our R&D efforts and potential to generate revenue may be limited.

If we enter into R&D collaborations during the early phases of product development, success will in part depend on the performance of research collaborators. We will not directly control the amount or timing of resources devoted by research collaborators to activities related to product candidates. Research collaborators may not commit sufficient resources to our R&D programs. If any research collaborator fails to commit sufficient resources, the preclinical or clinical development programs related to the collaboration could be delayed or terminated. Also, collaborators may pursue existing or other development-stage products or alternative technologies in preference to those being developed in collaboration with us. Finally, if we fail to make required milestone or royalty payments to collaborators or to observe other obligations in agreements with them, the collaborators may have the right to terminate or stop performance of those agreements.

Establishing strategic collaborations is difficult and time-consuming. Our discussions with potential collaborators may not lead to the establishment of collaborations on favorable terms, if at all. Potential collaborators may reject collaborations based upon their assessment of our financial, regulatory or intellectual property position. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators. Even if we successfully establish new collaborations, these relationships may never result in the successful development or commercialization of product candidates or the generation of sales revenue. To the extent that we enter into collaborative arrangements, the related product revenues are likely to be lower than if we directly marketed and sold products. Such collaborators may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for any future product candidate.

Management of our relationships with collaborators will require:

- significant time and effort from our management team;
- coordination of our marketing and R&D programs with the marketing and R&D priorities of our collaborators; and
- effective allocation of our resources to multiple projects.

***We are parties to and may seek to enter into additional collaborations, licenses and other similar arrangements and may not be successful in maintaining existing arrangements or entering into new ones, and even if we are, we may not realize the benefits of such relationships.***

The success of our collaboration arrangements will depend heavily on the efforts and activities of our collaborators. Collaborations are subject to numerous risks, which may include risks that:

- collaborators may have significant discretion in determining the efforts and resources that they will apply to collaborations;
- collaborators may not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in their strategic focus due to their acquisition of competitive products or their internal development of competitive products, availability of funding or other external factors, such as a business combination that diverts resources or creates competing priorities;

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- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial, abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our products or product candidates;
- a collaborator with marketing, manufacturing and distribution rights to one or more products may not commit sufficient resources to or otherwise not perform satisfactorily in carrying out these activities;
- we could grant exclusive rights to our collaborators that would prevent us from collaborating with others;
- collaborators may not properly maintain or defend our intellectual property rights or may use our intellectual property or proprietary information in a way that gives rise to actual or threatened litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential liability;
- disputes may arise between us and a collaborator that cause the delay or termination of the research, development or commercialization of our current or future product candidates or that results in costly litigation or arbitration that diverts management attention and resources;
- collaborations may be terminated, which may result in a need for additional capital to pursue further development or commercialization of the applicable current or future product candidates;
- collaborators may own or co-own intellectual property covering products that result from our collaboration with them, and in such cases, we would not have the exclusive right to develop or commercialize such intellectual property;
- disputes may arise with respect to the ownership of any intellectual property developed pursuant to our collaborations; and
- a collaborator's sales and marketing activities or other operations may not be in compliance with applicable laws resulting in civil or criminal proceedings.

Additionally, we may seek to enter into additional collaborations, joint ventures, licenses and other similar arrangements for the development or commercialization of our product candidates, due to capital costs required to develop or commercialize the product candidate or manufacturing constraints. We may not be successful in our efforts to establish such collaborations for our product candidates because our research and development pipeline may be insufficient, our product candidates may be deemed to be at too early of a stage of development for collaborative effort or third parties may not view our product candidates as having the requisite potential to demonstrate safety and efficacy or significant commercial opportunity. In addition, we face significant competition in seeking appropriate strategic partners, and the negotiation process can be time consuming and complex. Further, any future collaboration agreements may restrict us from entering into additional agreements with potential collaborators. We cannot be certain that, following a strategic transaction or license, we will achieve an economic benefit that justifies such transaction.

Even if we are successful in our efforts to establish such collaborations, the terms that we agree upon may not be favorable to us, and we may not be able to maintain such collaborations if, for example, development or approval of a product candidate is delayed, the safety of a product candidate is questioned or sales of an approved product candidate are unsatisfactory.

In addition, any potential future collaborations may be terminable by our strategic partners, and we may not be able to adequately protect our rights under these agreements. Furthermore, strategic partners may negotiate for certain rights to control decisions regarding the development and commercialization of our product candidates, if approved, and may not conduct those activities in the same manner as we do. Any termination of collaborations we enter into in the future, or any delay in entering into collaborations related to our product candidates, could

delay the development and commercialization of our product candidates and reduce their competitiveness if they reach the market, which could have a material adverse effect on our business, financial condition and results of operations.

### **Risks Related to our Intellectual Property**

***If we are unable to obtain and maintain sufficient intellectual property protection for BBP-265, BBP-831, BBP-454, BBP-631 or other product candidates that we may identify, or if the scope of the intellectual property protection obtained is not sufficiently broad, our competitors could develop and commercialize product candidates similar or identical to ours, and our ability to successfully commercialize BBP-265, BBP-831, BBP-454, BBP-631 and other product candidates that we may pursue may be impaired.***

As is the case with other pharmaceutical and biopharmaceutical companies, our success depends in large part on our ability to obtain and maintain protection of the intellectual property we may own solely and jointly with others, particularly patents, in the United States and other countries with respect to our product candidates and technology. We seek to protect our proprietary position by filing patent applications in the United States and abroad related to BBP-265, BBP-831, BBP-454, BBP-631 or other product candidates that we may identify.

Obtaining and enforcing pharmaceutical and biopharmaceutical patents is costly, time consuming and complex, and we may not be able to file and prosecute all necessary or desirable patent applications, or maintain, enforce and license any patents that may issue from such patent applications, at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. We may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the rights to patents licensed to third parties. Therefore, these patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal, technological and factual questions and has in recent years been the subject of much litigation. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States, or vice versa. Further, we may not be aware of all third-party intellectual property rights potentially relating to our product candidates. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing or, in some cases, not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions. Furthermore, the scope of a patent claim is determined by an interpretation of the law, the written disclosure in a patent and the patent's prosecution history and can involve other factors such as expert opinion. Our analysis of these issues, including interpreting the relevance or the scope of claims in a patent or a pending application, determining applicability of such claims to our proprietary technologies or product candidates, predicting whether a third party's pending patent application will issue with claims of relevant scope, and determining the expiration date of any patent in the United States or abroad that we consider relevant may be incorrect, which may negatively impact our ability to develop and market our product candidates. We do not always conduct independent reviews of pending patent applications of and patents issued to third parties. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued that protect our product candidates, in whole or in part, or which effectively prevent others from commercializing competitive product candidates. Even if our patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our patents by developing similar or alternative product candidates in a non-infringing manner.

Our ability to enforce patent rights also depends on our ability to detect infringement. It may be difficult to detect infringers who do not advertise the components or methods that are used in connection with their products and

services. Moreover, it may be difficult or impossible to obtain evidence of infringement in a competitor's or potential competitor's product or service. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded if we were to prevail may not be commercially meaningful. If we initiate lawsuits to protect or enforce our patents, or litigate against third-party claims, such proceedings would be expensive and would divert the attention of our management and technical personnel. Such proceedings could also provoke third parties to assert claims against us, including that some or all of the claims in one or more of our patents are invalid or otherwise unenforceable.

Moreover, we may be subject to a third-party preissuance submission of prior art to the United States Patent and Trademark Office, or the USPTO, or become involved in opposition, derivation, reexamination, inter partes review, post-grant review or interference proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our product candidates and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize drugs without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, regardless of the outcome, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

In addition, the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical product candidates to ours, or limit the duration of the patent protection of our product candidates. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our patent portfolio may not provide us with sufficient rights to exclude others from commercializing drugs similar or identical to ours.

Furthermore, our intellectual property rights may be subject to a reservation of rights by one or more third parties. For example, the research resulting in certain of our patent rights and technology was funded in part by the U.S. government. As a result, the government has certain rights, including march-in rights, to such patent rights and technology. When new technologies are developed with government funding, the government generally obtains certain rights in any resulting patents, including a non-exclusive license authorizing the government to use the invention or to have others use the invention on its behalf. These rights may permit the government to disclose our information to third parties and to exercise march-in rights to use or allow third parties to use our technology. The government can exercise its march-in rights if it determines that action is necessary because we fail to achieve practical application of the government-funded technology, because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations, or to give preference to U.S. industry. In addition, our rights in such inventions may be subject to certain requirements to manufacture products embodying such inventions in the United States. Any exercise by the government of such rights or by any third party of its reserved rights could harm our competitive position, business, financial condition, results of operations, and prospects.

***Our rights to develop and commercialize our product candidates are subject in part to the terms and conditions of licenses granted to us by others, and the patent protection, prosecution and enforcement for some of our product candidates may be dependent on our licensors.***

We currently are reliant upon licenses of certain intellectual property rights and proprietary technology from third parties that are important or necessary to the development of our proprietary technology, including technology related to our product candidates. These licenses, and other licenses we may enter into in the future, may not provide adequate rights to use such intellectual property rights and proprietary technology in all relevant fields of use or in all territories in which we may wish to develop or commercialize technology and product



candidates in the future. Licenses to additional third-party proprietary technology or intellectual property rights that may be required for our development programs may not be available in the future or may not be available on commercially reasonable terms. In that event, we may be required to expend significant time and resources to redesign our proprietary technology or product candidates or to develop or license replacement technology, which may not be feasible on a technical or commercial basis. If we are unable to do so, we may not be able to develop and commercialize technology and product candidates in fields of use and territories for which we are not granted rights pursuant to such licenses, which could harm our competitive position, business, financial condition, results of operations and prospects significantly.

In some circumstances, we may not have the right to control the preparation, filing, prosecution and enforcement of patent applications, or to maintain the patents, covering technology that we license from third parties. In addition, some of our agreements with our licensors require us to obtain consent from the licensor before we can enforce patent rights, and our licensor may withhold such consent or may not provide it on a timely basis. Therefore, we cannot be certain that our licensors or collaborators will prosecute, maintain, enforce and defend such intellectual property rights in a manner consistent with the best interests of our business, including by taking reasonable measures to protect the confidentiality of know-how and trade secrets, or by paying all applicable prosecution and maintenance fees related to intellectual property registrations for any of our product candidates. We also cannot be certain that our licensors have drafted or prosecuted the patents and patent applications licensed to us in compliance with applicable laws and regulations, which may affect the validity and enforceability of such patents or any patents that may issue from such applications. This could cause us to lose rights in any applicable intellectual property that we in-license, and as a result our ability to develop and commercialize product candidates may be adversely affected and we may be unable to prevent competitors from making, using and selling competing products.

In addition, our licensors may own or control intellectual property that has not been licensed to us and, as a result, we may be subject to claims, regardless of their merit, that we are infringing or otherwise violating the licensor's rights. In addition, while we cannot currently determine the amount of the royalty obligations we would be required to pay on sales of future products, if any, the amounts may be significant. The amount of our future royalty obligations will depend on the technology and intellectual property we use in product candidates that we successfully develop and commercialize, if any. Therefore, even if we successfully develop and commercialize product candidates, we may be unable to achieve or maintain profitability. In addition, we may seek to obtain additional licenses from our licensors and, in connection with obtaining such licenses, we may agree to amend our existing licenses in a manner that may be more favorable to the licensors, including by agreeing to terms that could enable third parties (potentially including our competitors) to receive licenses to a portion of the intellectual property rights that are subject to our existing licenses. Any of these events could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

***If we fail to comply with our obligations in the agreements under which we license intellectual property rights from third parties or these agreements are terminated or we otherwise experience disruptions to our business relationships with our licensors, we could lose intellectual property rights that are important to our business.***

We are party to various agreements that we depend on to operate our business, and our rights to use currently licensed intellectual property, or intellectual property to be licensed in the future, are or will be subject to the continuation of and our compliance with the terms of these agreements. For example, we are a party to an exclusive license agreement with the Board of Trustees of the Leland Stanford Junior University, or Stanford, and may need to obtain additional licenses from others to advance our research and development activities to allow the commercialization of BBP-265 or any other product candidates we may identify and pursue. Our license agreement with Stanford imposes, and we expect that future license agreements will impose, various development, diligence, commercialization, and other obligations on us. For example, under our license agreement with Stanford, we are required to use commercially reasonable efforts to engage in various development and commercialization activities with respect to licensed products, and must satisfy specified

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milestone and royalty payment obligations. We are also a party to a license agreement with Novartis International Pharmaceutical Ltd. for BBP-831 under which we are required to use commercially reasonable efforts to develop BBP-831, and to obtain regulatory approval for and commercialize at least one therapeutic product incorporating BBP-831 in the United States and the European Union.

In spite of our efforts, our licensors might conclude that we have materially breached our obligations under such license agreements and might therefore terminate the license agreements, thereby removing or limiting our ability to develop and commercialize products and technology covered by these license agreements. For example, if our license agreement with Stanford is terminated, competitors or other third parties would have the freedom to seek regulatory approval of, and to market, products identical to BBP-265 and we may be required to cease our development and commercialization of BBP-265. Any of the foregoing could have a material adverse effect on our competitive position, business, financial conditions, results of operations and prospects.

Moreover, disputes may arise regarding intellectual property subject to a licensing agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our product candidates, technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights under our collaborative development relationships;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the inventorship and ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- the priority of invention of patented technology.

In addition, certain provisions in our license agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the agreement, either of which could have a material adverse effect on our business, financial condition, results of operations and prospects. Moreover, if disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates, which could have a material adverse effect on our competitive position, business, financial conditions, results of operations and prospects.

### ***Third-party claims of intellectual property infringement may prevent or delay our development and commercialization efforts.***

Our commercial success depends in part on our avoiding infringement of the patents and proprietary rights of third parties. However, our research, development and commercialization activities may be subject to claims that we infringe or otherwise violate patents or other intellectual property rights owned or controlled by third parties. There is a substantial amount of litigation, both within and outside the United States, involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits, interferences, oppositions and inter partes reexamination proceedings before the USPTO, and corresponding foreign patent offices. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are pursuing development candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that BBP-265, BBP-831, BBP-454, BBP-631 or other product candidates that we may identify may be subject to claims of infringement of the patent rights of third parties.

For example, in December 2018, the Children's Hospital and Research Center at Oakland d/b/a UC Benioff Children's Hospital-Oakland, or CHRCO, filed a complaint in the U.S. District Court of the Northern District of

California alleging, among other things, that PellePharm infringed certain patent rights of CHRCO.

Other third parties may assert that we are employing their proprietary technology without authorization. There may be other third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of BBP-265, BBP-831, BBP-454, BBP-631 or other product candidates that we may identify. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that BBP-265, BBP-831, BBP-454, BBP-631 or other product candidates that we may identify may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of BBP-265, BBP-831, BBP-454, BBP-631 or other product candidates that we may identify, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until such patents expire.

Similarly, if any third-party patents, including any patents that may issue from the '257 application, were held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, including combination therapy, the holders of any such patents may be able to block our ability to develop and commercialize the applicable product candidate unless we obtained a license or until such patent expires. In either case, such a license may not be available on commercially reasonable terms or at all, or it may be non-exclusive, which could result in our competitors gaining access to the same intellectual property.

Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize BBP-265, BBP-831, BBP-454, BBP-631 or other product candidates that we may identify. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, pay royalties, redesign our infringing products or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure.

Parties making claims against us, including CHRCO, may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation or administrative proceedings, there is a risk that some of our confidential information could be compromised by disclosure. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have material adverse effect on ability to raise additional funds or otherwise have a material adverse effect on our business, results of operations, financial condition and prospects.

***Patent terms may be inadequate to protect our competitive position on product candidates for an adequate amount of time.***

Patents have a limited lifespan. In the United States, if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional or international patent application filing date. Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired, we may be open to competition from competitive products, including generics or biosimilars. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

***If we are not able to obtain patent term extension or non-patent exclusivity in the United States under the Hatch-Waxman Act and in foreign countries under similar legislation, thereby potentially extending the marketing exclusivity term of our product candidates, our business may be materially harmed.***

Depending upon the timing, duration and specifics of FDA marketing approval of our product candidates, one of the U.S. patents covering each of such product candidates or the use thereof may be eligible for up to five years of patent term extension under the Hatch-Waxman Act. The Hatch-Waxman Act allows a maximum of one patent to be extended per FDA approved product as compensation for the patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only those claims covering such approved drug product, a method for using it or a method for manufacturing it may be extended. Patent term extension also may be available in certain foreign countries upon regulatory approval of our product candidates. Nevertheless, we may not be granted patent term extension either in the United States or in any foreign country because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the term of extension, as well as the scope of patent protection during any such extension, afforded by the governmental authority could be less than we request.

If we are unable to obtain patent term extension or restoration, or the term of any such extension is less than we request, the period during which we will have the right to exclusively market our product may be shortened and our competitors may obtain approval of competing products following our patent expiration sooner, and our revenue could be reduced, possibly materially.

It is possible that we will not obtain patent term extension under the Hatch-Waxman Act for a U.S. patent covering a product candidate even where that patent is eligible for patent term extension, or if we obtain such an extension, it may be for a shorter period than we had sought. Further, for certain of our licensed patents, we do not have the right to control prosecution, including filing with the USPTO, a petition for patent term extension under the Hatch-Waxman Act. Thus, if one of our licensed patents is eligible for patent term extension under the Hatch-Waxman Act, we may not be able to control whether a petition to obtain a patent term extension is filed, or obtained, from the USPTO.

Also, there are detailed rules and requirements regarding the patents that may be submitted to the FDA for listing in the Approved Drug Products with Therapeutic Equivalence Evaluations, or the Orange Book. We may be unable to obtain patents covering our product candidates that contain one or more claims that satisfy the requirements for listing in the Orange Book. Even if we submit a patent for listing in the Orange Book, the FDA may decline to list the patent, or a manufacturer of generic drugs may challenge the listing. If one of product candidates is approved and a patent covering that product candidate is not listed in the Orange Book, a manufacturer of generic drugs would not have to provide advance notice to us of any abbreviated new drug application, or ANDA, filed with the FDA to obtain permission to sell a generic version of such product candidate.

***If we are unable to protect the confidentiality of our trade secrets, the value of our technology could be materially adversely affected and our business would be harmed.***

We seek to protect our confidential proprietary information, in part, by confidentiality agreements and invention assignment agreements with our employees, consultants, scientific advisors, contractors and collaborators. These agreements are designed to protect our proprietary information. However, we cannot be certain that such agreements have been entered into with all relevant parties, and we cannot be certain that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. For example, any of these parties may breach the agreements and disclose proprietary information, including trade secrets, and we may not be able to obtain adequate remedies for such breaches. We also seek to preserve the integrity and

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confidentiality of our confidential proprietary information by maintaining physical security of our premises and physical and electronic security of our information technology systems, but it is possible that these security measures could be breached. If any of our confidential proprietary information were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent such competitor from using that technology or information to compete with us, which could harm our competitive position.

Unauthorized parties may also attempt to copy or reverse engineer certain aspects of our products that we consider proprietary. We may not be able to obtain adequate remedies in the event of such unauthorized use. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret can be difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. Trade secrets will also over time be disseminated within the industry through independent development, the publication of journal articles and the movement of personnel skilled in the art from company to company or academic to industry scientific positions. Though our agreements with third parties typically restrict the ability of our advisors, employees, collaborators, licensors, suppliers, third-party contractors and consultants to publish data potentially relating to our trade secrets, our agreements may contain certain limited publication rights. In addition, if any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent such competitor from using that technology or information to compete with us, which could harm our competitive position. Despite employing the contractual and other security precautions described above, the need to share trade secrets increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. If any of these events occurs or if we otherwise lose protection for our trade secrets, the value of this information may be greatly reduced and our competitive position, business, financial condition, results of operations, and prospects would be harmed.

***If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.***

Our registered or unregistered trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential collaborators or customers in our markets of interest. At times, competitors may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. We may license our trademarks and trade names to third parties, such as distributors. Though these license agreements may provide guidelines for how our trademarks and trade names may be used, a breach of these agreements or misuse of our trademarks and tradenames by our licensees may jeopardize our rights in or diminish the goodwill associated with our trademarks and trade names. Our efforts to enforce or protect our proprietary rights related to trademarks, trade names, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely affect our competitive position, business, financial condition, results of operations and prospects.

***We may become involved in lawsuits to protect or enforce our patents or other intellectual property, which could be expensive, time consuming and unsuccessful.***

Competitors may infringe our patents or other intellectual property. If we were to initiate legal proceedings against a third party to enforce a patent covering one or more of our product candidates, the defendant could counterclaim that the patent covering our product candidate is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements,

including novelty, nonobviousness, written description or enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO, or made a misleading statement, during prosecution. The outcome following legal assertions of invalidity and unenforceability is unpredictable. Interference or derivation proceedings provoked by third parties or brought by us or declared by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms or at all, or if a non-exclusive license is offered and our competitors gain access to the same technology. Our defense of litigation or interference or derivation proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. In addition, the uncertainties associated with litigation could have a material adverse effect on our ability to raise the funds necessary to continue clinical trials, continue research programs, license necessary technology from third parties, or enter into development partnerships that would help us bring product candidates to market. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions, or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our common stock.

***We may be subject to claims challenging the inventorship of our patents and other intellectual property.***

Our agreements with employees and our personnel policies provide that any inventions conceived by an individual in the course of rendering services to us shall be our exclusive property. Although our policy is to have all such individuals complete these agreements, we may not obtain these agreements in all circumstances, and individuals with whom we have these agreements may not comply with their terms. The assignment of intellectual property may not be automatic upon the creation of an invention and despite such agreement, such inventions may become assigned to third parties. In the event of unauthorized use or disclosure of our trade secrets or proprietary information, these agreements, even if obtained, may not provide meaningful protection, particularly for our trade secrets or other confidential information.

We or our licensors may be subject to claims that former employees, collaborators or other third parties have an interest in our owned or in-licensed patents, trade secrets, or other intellectual property as an inventor or co-inventor. For example, we or our licensors may have inventorship disputes arise from conflicting obligations of employees, consultants or others who are involved in developing our product candidates. Litigation may be necessary to defend against these and other claims challenging inventorship or our or our licensors' ownership of our owned or in-licensed patents, trade secrets or other intellectual property. If we or our licensors fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, intellectual property that is important to our product candidates. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

Any of the foregoing could have a material adverse effect on our competitive position, business, financial condition, results of operations and prospects.

***Issued patents covering our product candidates could be found invalid or unenforceable if challenged in court.***

If we or one of our licensing partners initiated legal proceedings against a third party to enforce a patent covering one or more of our product candidates, the defendant could counterclaim that the patent covering the relevant product candidate is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including novelty, nonobviousness,

written description or enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO, or made a misleading statement, during prosecution. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review, and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). Such proceedings could result in revocation or amendment to our patents in such a way that they no longer cover our product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates. Such a loss of patent protection would have a material adverse impact on our business.

***We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of third parties or that our employees have wrongfully used or disclosed alleged trade secrets of their former employers.***

As is common in the biotechnology and pharmaceutical industry, we employ individuals who were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and independent contractors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed intellectual property, including trade secrets or other proprietary information, of any of our employee's former employer or other third parties. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel, which could adversely impact our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

***Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.***

Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on patents and/or applications will be due to be paid to the USPTO and various governmental patent agencies outside of the United States in several stages over the lifetime of the patents and/or applications. We have systems in place to remind us to pay these fees, and we employ an outside firm and rely on outside counsel to pay these fees due to non-U.S. patent agencies. However, we cannot guarantee that our licensors have similar systems and procedures in place to pay such fees. The USPTO and various non-U.S. governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. We employ reputable law firms and other professionals to help us comply, and in many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. However, there are situations in which non-compliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, our competitors might be able to enter the market and this circumstance would have a material adverse effect on our business.

***We may not be able to protect our intellectual property rights throughout the world.***

Filing, prosecuting and defending patents on our product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we

may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and may also export infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets, and other intellectual property protection, particularly those relating to biotechnology and pharmaceutical products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions, whether or not successful, could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

***Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our products.***

Changes in either the patent laws or interpretation of the patent laws in the United States could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. Assuming that other requirements for patentability are met, prior to March 2013, in the United States, the first to invent the claimed invention was entitled to a patent, while outside the United States, the first to file a patent application was entitled to the patent. After March 2013, under the Leahy-Smith America Invents Act, or the America Invents Act, enacted in September 2011, the United States transitioned to a first inventor to file system in which, assuming that other requirements for patentability are met, the first inventor to file a patent application will be entitled to the patent on an invention regardless of whether a third party was the first to invent the claimed invention. A third party that files a patent application in the USPTO after March 2013, but before us could therefore be awarded a patent covering an invention of ours even if we had made the invention before it was made by such third party. This will require us to be cognizant of the time from invention to filing of a patent application. Since patent applications in the United States and most other countries are confidential for a period of time after filing or until issuance, we cannot be certain that we or our licensors were the first to either (i) file any patent application related to our product candidates or (ii) invent any of the inventions claimed in our or our licensor's patents or patent applications.

The America Invents Act also includes a number of significant changes that affect the way patent applications are prosecuted and also may affect patent litigation. These include allowing third party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent by USPTO administered post-grant proceedings, including post-grant review, inter partes review, and derivation proceedings. Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in United States federal courts necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action. Accordingly, a third party may attempt to use the USPTO procedures to invalidate our patent claims that would not have been invalidated if first challenged by the third party as a defendant in a district court action. Therefore, the America Invents Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our owned or in-licensed patent applications and the enforcement or defense of our owned or in-licensed issued



patents, all of which could have a material adverse effect on our competitive position, business, financial condition, results of operations, and prospects.

In addition, the patent positions of companies in the development and commercialization of pharmaceuticals are particularly uncertain. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. This combination of events has created uncertainty with respect to the validity and enforceability of patents, once obtained. Depending on future actions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that could have a material adverse effect on our existing patent portfolio and our ability to protect and enforce our intellectual property in the future.

### **Risks Related to Commercialization**

***Even if any product candidates we develop receive marketing approval, they may fail to achieve the degree of market acceptance by physicians, patients, healthcare payors, and others in the medical community necessary for commercial success.***

The commercial success of our product candidates will depend upon their degree of market acceptance by physicians, patients, third-party payors, and others in the medical community. Even if any product candidates we may develop receive marketing approval, they may nonetheless fail to gain sufficient market acceptance by physicians, patients, healthcare payors, and others in the medical community. The degree of market acceptance of any product candidates we may develop, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and safety of such product candidates as demonstrated in pivotal clinical trials and published in peer-reviewed journals;
- the potential and perceived advantages compared to alternative treatments, including any similar generic treatments;
- the ability to offer these products for sale at competitive prices;
- the ability to offer appropriate patient access programs, such as co-pay assistance;
- convenience and ease of dosing and administration compared to alternative treatments;
- the clinical indications for which the product candidate is approved by FDA or comparable regulatory agencies;
- product labeling or product insert requirements of the FDA or other comparable foreign regulatory authorities, including any limitations, contraindications or warnings contained in a product's approved labeling;
- restrictions on how the product is distributed;
- the timing of market introduction of competitive products;
- publicity concerning these products or competing products and treatments;
- the strength of marketing and distribution support;
- favorable third-party coverage and sufficient reimbursement; and
- the prevalence and severity of any side effects or AEs.

Sales of medical products also depend on the willingness of physicians to prescribe the treatment, which is likely to be based on a determination by these physicians that the products are safe, therapeutically effective and cost effective. In addition, the inclusion or exclusion of products from treatment guidelines established by various physician groups and the viewpoints of influential physicians can affect the willingness of other physicians to

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prescribe the treatment. We cannot predict whether physicians, physicians' organizations, hospitals, other healthcare providers, government agencies or private insurers will determine that our product is safe, therapeutically effective and cost effective as compared with competing treatments. If any product candidates we develop do not achieve an adequate level of acceptance, we may not generate significant product revenue, and we may not become profitable.

***If, in the future, we are unable to establish sales and marketing capabilities or enter into agreements with third parties to sell and market any product candidates we may develop, we may not be successful in commercializing those product candidates if and when they are approved.***

We do not have a sales or marketing infrastructure and have little experience in the sale, marketing, or distribution of pharmaceutical products. To achieve commercial success for any approved product for which we retain sales and marketing responsibilities, we must either develop a sales and marketing organization or outsource these functions to third parties. In the future, we may choose to build a focused sales, marketing, and commercial support infrastructure to market and sell our product candidates, if and when they are approved. We may also elect to enter into collaborations or strategic partnerships with third parties to engage in commercialization activities with respect to selected product candidates, indications or geographic territories, including territories outside the United States, although there is no guarantee we will be able to enter into these arrangements even if the intent is to do so.

There are risks involved with both establishing our own commercial capabilities and entering into arrangements with third parties to perform these services. For example, recruiting and training a sales force or reimbursement specialists is expensive and time consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing and other commercialization capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition commercialization personnel.

Factors that may inhibit our efforts to commercialize any approved product on our own include:

- the inability to recruit and retain adequate numbers of effective sales, marketing, reimbursement, customer service, medical affairs, and other support personnel;
- the inability of sales personnel to obtain access to physicians or persuade adequate numbers of physicians to prescribe any future approved products;
- the inability of reimbursement professionals to negotiate arrangements for formulary access, reimbursement, and other acceptance by payors;
- the inability to price products at a sufficient price point to ensure an adequate and attractive level of profitability;
- restricted or closed distribution channels that make it difficult to distribute our products to segments of the patient population;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent commercialization organization.

If we enter into arrangements with third parties to perform sales, marketing, commercial support, and distribution services, our product revenue or the profitability of product revenue may be lower than if we were to market and sell any products we may develop internally. In addition, we may not be successful in entering into arrangements with third parties to commercialize our product candidates or may be unable to do so on terms that are favorable to us or them. We may have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively or may expose us to legal and

regulatory risk by not adhering to regulatory requirements and restrictions governing the sale and promotion of prescription drug products, including those restricting off-label promotion. If we do not establish commercialization capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates, if approved.

***The insurance coverage and reimbursement status of newly-approved products is uncertain. Our product candidates may become subject to unfavorable pricing regulations, third-party coverage and reimbursement practices, or healthcare reform initiatives, which would harm our business. Failure to obtain or maintain adequate coverage and reimbursement for new or current products could limit our ability to market those products and decrease our ability to generate revenue.***

The regulations that govern marketing approvals, pricing, coverage, and reimbursement for new drugs vary widely from country to country. In the United States, recently enacted legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenue we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if any product candidates we may develop obtain marketing approval.

Our ability to successfully commercialize our product candidates also will depend in part on the extent to which coverage and adequate reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers, and other organizations. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. The availability of coverage and extent of reimbursement by governmental and private payors is essential for most patients to be able to afford treatments such as gene therapy products. Sales of these or other product candidates that we may identify will depend substantially, both domestically and abroad, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or reimbursed by government health administration authorities, private health coverage insurers and other third-party payors. If coverage and adequate reimbursement is not available, or is available only to limited levels, we may not be able to successfully commercialize our product candidates. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on our investment.

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In general, the prices of medicines under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for medicines, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for products may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenues and profits.

There is also significant uncertainty related to the insurance coverage and reimbursement of newly approved products and coverage may be more limited than the purposes for which the medicine is approved by the FDA or comparable foreign regulatory authorities. In the United States, the principal decisions about reimbursement for new medicines are typically made by the Centers for Medicare & Medicaid Services, or CMS, an agency within

the U.S. Department of Health and Human Services. CMS decides whether and to what extent a new medicine will be covered and reimbursed under Medicare and private payors tend to follow CMS to a substantial degree. No uniform policy of coverage and reimbursement for products exists among third-party payors and coverage and reimbursement levels for products can differ significantly from payor to payor. As a result, the coverage determination process is often a time consuming and costly process that may require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance. It is difficult to predict what CMS will decide with respect to reimbursement for fundamentally novel products such as ours, as there is no body of established practices and precedents for these new products. Reimbursement agencies in Europe may be more conservative than CMS. For example, a number of cancer drugs have been approved for reimbursement in the United States and have not been approved for reimbursement in certain European countries. Moreover, eligibility for reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale, and distribution. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. Our inability to promptly obtain coverage and profitable payment rates from both government-funded and private payors for any approved products we may develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize product candidates, and our overall financial condition.

Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. Our inability to promptly obtain coverage and profitable reimbursement rates third-party payors for any approved products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that reimbursement will be available for any product candidate that we commercialize and, if reimbursement is available, the level of reimbursement. Reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. In order to obtain reimbursement, physicians may need to show that patients have superior treatment outcomes with our products compared to standard of care drugs, including lower-priced generic versions of standard of care drugs. We expect to experience pricing pressures in connection with the sale of any of our product candidates, due to the trend toward managed healthcare, the increasing influence of health maintenance organizations and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products.

Additionally, we may develop companion diagnostic tests for use with our product candidates. For instance, we are partnered with FMI to develop a companion diagnostic for use in our planned NDA submission for BBP-831 for second-line CCA. We, or our collaborators, may be required to obtain coverage and reimbursement for these tests separate and apart from the coverage and reimbursement we seek for our product candidates, once approved. Even if we obtain regulatory approval or clearance for such companion diagnostics, there is significant uncertainty regarding our ability to obtain coverage and adequate reimbursement for the same reasons applicable to our product candidates. Medicare reimbursement methodologies, whether under Part A, Part B, or clinical laboratory fee schedule may be amended from time to time, and we cannot predict what effect any change to these methodologies would have on any product candidate or companion diagnostic for which we receive approval. Our inability to promptly obtain coverage and adequate reimbursement from both third-party payors for the companion diagnostic tests that we develop and for which we obtain regulatory approval could have a material and adverse effect on our business, financial condition, results of operations and prospects.

***If we fail to comply with healthcare laws, we could face substantial penalties and our business, operations and financial conditions could be adversely affected.***

Healthcare providers, physicians and third-party payors in the United States and elsewhere play a primary role in the recommendation and prescription of pharmaceutical products. Arrangements with third-party payors and customers can expose pharmaceutical manufacturers to broadly applicable fraud and abuse and other healthcare laws and regulations, including, without limitation, the federal Anti-Kickback Statute and the federal False Claims Act, which may constrain the business or financial arrangements and relationships through which such companies sell, market and distribute pharmaceutical products. In particular, the promotion, sales and marketing of healthcare items and services, as well as certain business arrangements in the healthcare industry, are subject to extensive laws designed to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of ownership, pricing, discounting, marketing and promotion, structuring and commission(s), certain customer incentive programs and other business arrangements generally. Activities subject to these laws also involve the improper use of information obtained in the course of patient recruitment for clinical trials. The applicable federal and state healthcare laws and regulations laws that may affect our ability to operate include, but are not limited to:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe, or rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce, or in return for, either the referral of an individual, or the purchase, lease, order or recommendation of any good, facility, item or service for which payment may be made, in whole or in part, under a federal healthcare program, such as the Medicare and Medicaid programs. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. Violations are subject to civil and criminal fines and penalties up to \$100,000 for each violation, plus up to three times the remuneration involved, imprisonment of up to ten years, and exclusion from government healthcare programs. In addition, the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act. There are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution;
- federal civil and criminal false claims laws and civil monetary penalty laws, including the False Claims Act, which impose criminal and civil penalties, including through civil “qui tam” or “whistleblower” actions, against individuals or entities for, among other things, knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid, or other federal health care programs that are false or fraudulent; knowingly making or causing a false statement material to a false or fraudulent claim or an obligation to pay money to the federal government; or knowingly concealing or knowingly and improperly avoiding or decreasing such an obligation. Manufacturers can be held liable under the FCA even when they do not submit claims directly to government payors if they are deemed to “cause” the submission of false or fraudulent claims. The FCA also permits a private individual acting as a “whistleblower” to bring actions on behalf of the federal government alleging violations of the FCA and to share in any monetary recovery. When an entity is determined to have violated the federal civil False Claims Act, the government may impose civil fines and penalties ranging from \$11,181 to \$22,363 for each false claim, plus treble damages, and exclude the entity from participation in Medicare, Medicaid and other federal healthcare programs;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payor (e.g., public or private) and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters. Similar to the federal Anti-Kickback Statute, a person or entity can be found guilty of violating HIPAA without actual knowledge of the statute or specific intent to violate it;

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- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their respective implementing regulations, which impose requirements on certain covered healthcare providers, health plans, and healthcare clearinghouses as well as their respective business associates that perform services for them that involve the use, or disclosure of, individually identifiable health information, relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization. HITECH also created new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorneys' fees and costs associated with pursuing federal civil actions;
- the federal Physician Payments Sunshine Act, created under the ACA, and its implementing regulations, which require manufacturers of drugs, devices, biologicals and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program to report annually to the U.S. Department of Health and Human Services under the Open Payments Program, information related to payments or other transfers of value made to physicians, certain other healthcare professionals, and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members;
- federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers; and
- analogous state and foreign laws and regulations, such as state and foreign anti-kickback, false claims, consumer protection and unfair competition laws which may apply to pharmaceutical business practices, including but not limited to, research, distribution, sales and marketing arrangements as well as submitting claims involving healthcare items or services reimbursed by any third-party payer, including commercial insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government that otherwise restricts payments that may be made to healthcare providers and other potential referral sources; state laws that require drug manufacturers to file reports with states regarding pricing and marketing information, such as the tracking and reporting of gifts, compensations and other remuneration and items of value provided to healthcare professionals and entities; state and local laws requiring the registration of pharmaceutical sales representatives; and state and foreign laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of the statutory exceptions and regulatory safe harbors available, it is possible that some of our business activities, including compensation of physicians with stock or stock options, could, despite efforts to comply, be subject to challenge under one or more of such laws. Additionally, FDA or foreign regulators may not agree that we have mitigated any risk of bias in our clinical trials due to payments or equity interests provided to investigators or institutions which could limit a regulator's acceptance of those clinical trial data in support of a marketing application. Moreover, efforts to ensure that our business arrangements will comply with applicable healthcare laws may involve substantial costs. It is possible that governmental and enforcement authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law interpreting applicable fraud and abuse or other healthcare laws and regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant civil, criminal and administrative penalties, damages, disgorgement, monetary fines, exclusion from participation in Medicare, Medicaid and other federal healthcare programs, integrity and oversight agreements to resolve allegations of non-compliance, contractual damages, reputational harm, diminished profits and future earnings, and curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations. In addition, the approval and commercialization of any of our product candidates outside the United States will also likely subject us to foreign equivalents of the healthcare laws mentioned above, among other foreign laws.

***Failure to comply with health and data protection laws and regulations could lead to government enforcement actions (which could include civil or criminal penalties), private litigation, and/or adverse publicity and could negatively affect our operating results and business.***

We and any potential collaborators may be subject to federal, state, and foreign data protection laws and regulations (i.e., laws and regulations that address privacy and data security). In the United States, numerous federal and state laws and regulations, including federal health information privacy laws, state data breach notification laws, state health information privacy laws, and federal and state consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act), that govern the collection, use, disclosure and protection of health-related and other personal information could apply to our operations or the operations of our collaborators. In addition, we may obtain health information from third parties (including research institutions from which we obtain clinical trial data) that are subject to privacy and security requirements under HIPAA, as amended by HITECH. Depending on the facts and circumstances, we could be subject to civil, criminal, and administrative penalties if we knowingly obtain, use, or disclose individually identifiable health information maintained by a HIPAA-covered entity in a manner that is not authorized or permitted by HIPAA.

Compliance with U.S. and international data protection laws and regulations could require us to take on more onerous obligations in our contracts, restrict our ability to collect, use and disclose data, or in some cases, impact our ability to operate in certain jurisdictions. Failure to comply with these laws and regulations could result in government enforcement actions (which could include civil, criminal and administrative penalties), private litigation, and/or adverse publicity and could negatively affect our operating results and business. Moreover, clinical trial subjects, employees and other individuals about whom we or our potential collaborators obtain personal information, as well as the providers who share this information with us, may limit our ability to collect, use and disclose the information. Claims that we have violated individuals' privacy rights, failed to comply with data protection laws, or breached our contractual obligations, even if we are not found liable, could be expensive and time-consuming to defend and could result in adverse publicity that could harm our business.

***European data collection is governed by restrictive regulations governing the use, processing and cross-border transfer of personal information.***

In the event we decide to conduct clinical trials or continue to enroll subjects in our ongoing or future clinical trials in the European Union, we may be subject to additional privacy restrictions. The collection and use of personal health data in the European Union is governed by the provisions of the General Data Protection Regulation 2016/679, or GDPR. This directive imposes several requirements relating to the consent of the individuals to whom the personal data relates, the information provided to the individuals, notification of data processing obligations to the competent national data protection authorities and the security and confidentiality of the personal data. The GDPR also imposes strict rules on the transfer of personal data out of the European Union to the United States. Failure to comply with the requirements of the Data Protection Directive, which governs the collection and use of personal health data in the European Union, the GDPR, and the related national data protection laws of the European Union Member States may result in fines and other administrative penalties. The GDPR introduced new data protection requirements in the European Union and substantial fines for breaches of the data protection rules. The GDPR regulations may impose additional responsibility and liability in relation to personal data that we process and we may be required to put in place additional mechanisms ensuring compliance with these and/or new data protection rules. This may be onerous and adversely affect our business, financial condition, prospects and results of operations.

***Healthcare legislative measures aimed at reducing healthcare costs may have a material adverse effect on our business and results of operations.***

The United States and many foreign jurisdictions have enacted or proposed legislative and regulatory changes affecting the healthcare system that could prevent or delay marketing approval of our product candidates or any future product candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any

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product for which we obtain marketing approval. Changes in regulations, statutes or the interpretation of existing regulations could impact our business in the future by requiring, for example: (i) changes to our manufacturing arrangements; (ii) additions or modifications to product labeling; (iii) the recall or discontinuation of our products; or (iv) additional record-keeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business.

In the United States, there have been and continue to be a number of legislative initiatives to contain healthcare costs. For example, in March 2010, the ACA was passed, which substantially changed the way healthcare is financed by both governmental and private insurers, and significantly impacted the U.S. pharmaceutical industry. The ACA, among other things, subjects biological products to potential competition by lower-cost biosimilars, addresses a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected, increases the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program and extends the rebate program to individuals enrolled in Medicaid managed care organizations, establishes annual fees and taxes on manufacturers of certain branded prescription drugs, and creates a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 70% (as of 2019) point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D.

There have been a number of significant changes to the ACA and its implementation. The Tax Cuts and Jobs Act of 2017, or Tax Act, includes a provision that repealed effective January 1, 2019 the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate." On December 14, 2018, a U.S. District Court Judge in the Northern District of Texas, or the Texas District Court Judge, ruled that the individual mandate is a critical and inseparable feature of the ACA, and therefore, because it was repealed as part of the Tax Act, the remaining provisions of the ACA are invalid as well. While the Trump Administration and CMS have both stated that the ruling will have no immediate effect, and on December 30, 2018 the Texas District Court Judge issued an order staying the judgment pending appeal, it is unclear how this decision, subsequent appeals and other efforts to repeal and replace the ACA will impact the ACA and our business.

On January 20, 2017, President Trump signed an Executive Order directing federal agencies with authorities and responsibilities under the ACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the ACA that would impose a fiscal burden on states or a cost, fee, tax, penalty or regulatory burden on individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. On October 13, 2017, President Trump signed an Executive Order terminating the cost-sharing subsidies that reimburse insurers under the ACA. Several state Attorneys General filed suit to stop the administration from terminating the subsidies, but their request for a restraining order was denied by a federal judge in California on October 25, 2017. On June 14, 2018, U.S. Court of Appeals for the Federal Circuit ruled that the federal government was not required to pay more than \$12 billion in ACA risk corridor payments to third-party payors who argued were owed to them. The effects of this gap in reimbursement on third-party payors, the viability of the ACA marketplace, providers, and potentially our business, are not yet known.

Moreover, on January 22, 2018, President Trump signed a continuing resolution on appropriations for fiscal year 2018 that delayed the implementation of certain ACA-mandated fees, including the so called "Cadillac" tax on certain high cost employer-sponsored insurance plans, the annual fee imposed on certain health insurance providers based on market share, and the medical device excise tax on non-exempt medical devices. The Bipartisan Budget Act of 2018, also amended the ACA, effective January 1, 2019, by increasing the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D and closing the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole." In July 2018, CMS published a final rule permitting further collections and payments to and from certain ACA qualified health plans and health insurance issuers under the ACA risk adjustment program in response to the outcome of federal district court litigation regarding the method CMS uses to determine this risk adjustment. In addition, CMS has recently published a final rule that would give states greater flexibility, starting in 2020, in setting benchmarks



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for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the ACA for plans sold through such marketplaces.

In addition, other legislative changes have been proposed and adopted in the United States since the ACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least \$1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers of 2% per fiscal year, which went into effect in 2013, and, due to subsequent legislative amendments, will remain in effect through 2027 unless additional Congressional action is taken. The American Taxpayer Relief Act of 2012 further reduced Medicare payments to several providers, including hospitals and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

There has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices. Specifically, there have been several recent U.S. Congressional inquiries and proposed federal and state legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs. At the federal level, the Trump administration's budget for fiscal year 2019 contains further drug price control measures that could be enacted during the 2019 legislative session, or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. Additionally, the Trump administration released a "Blueprint" to lower drug prices and reduce out of pocket costs of drugs that contains additional proposals to increase manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products and reduce the out of pocket costs of drug products paid by consumers. The U.S. Department of HHS, has already started the process of soliciting feedback on some of these measures and, at the same time, is immediately implementing others under its existing authority. For example, in September 2018, CMS announced that it will allow Medicare Advantage Plans the option to use step therapy for Part B drugs beginning January 1, 2019. Additionally, on May 10, 2019, the Centers for Medicare and Medicaid Services announced a new pricing transparency rule, which goes into effect on July 9, 2019. This final rule requires direct-to-consumer television advertisements for prescription drugs and biological products for which reimbursement is available, directly or indirectly, through or under Medicare or Medicaid to include the list price of that product, except for a prescription drug or biological product that has a list price of less than \$35 per month for a 30-day supply or typical course of treatment. The pricing transparency rule could have a negative effect on our business. Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

There have been, and likely will continue to be, legislative and regulatory proposals at the foreign, federal and state levels directed at containing or lowering the cost of healthcare. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our product. Such reforms could have an adverse effect on anticipated revenue from product candidates that we may successfully develop and for which we may obtain regulatory approval and may affect our overall financial condition and ability to develop product candidates. We cannot predict the initiatives that may be adopted in the future. The continuing efforts of the government, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce costs of healthcare and/or impose price controls may adversely affect:

- the demand for our product candidates, if approved;

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- our ability to receive or set a price that we believe is fair for our products;
- our ability to generate revenue and achieve or maintain profitability;
- the amount of taxes that we are required to pay; and
- the availability of capital.

We expect that the ACA, as well as other healthcare reform measures that may be adopted in the future, may result in additional reductions in Medicare and other healthcare funding, more rigorous coverage criteria, lower reimbursement, and new payment methodologies. This could lower the price that we receive for any approved product. Any denial in coverage or reduction in reimbursement from Medicare or other government-funded programs may result in a similar denial or reduction in payments from private payors, which may prevent us from being able to generate sufficient revenue, attain profitability or commercialize our product candidates, if approved.

***We face significant competition in an environment of rapid technological and scientific change, and there is a possibility that our competitors may achieve regulatory approval before us or develop therapies that are safer, more advanced or more effective than ours, which may negatively impact our ability to successfully market or commercialize any product candidates we may develop and ultimately harm our financial condition.***

The development and commercialization of new drug products is highly competitive. We may face competition with respect to any product candidates that we seek to develop or commercialize in the future from major pharmaceutical companies, specialty pharmaceutical companies, and biotechnology companies worldwide. Potential competitors also include academic institutions, government agencies, and other public and private research organizations that conduct research, seek patent protection, and establish collaborative arrangements for research, development, manufacturing, and commercialization.

There are a number of large pharmaceutical and biotechnology companies that are currently pursuing the development and commercialization of products for the treatment of the indications that our four key drivers are pursuing, including: tafamidis, a TTR tetramer stabilizer, a competitor to BBP-265; pemigatinib, a small molecule FGFR inhibitor, a competitor to BBP-831; NBI-74788, a corticotropin releasing factor receptor antagonist, a competitor to BBP-631; and MRTX849, a KRAS G12C inhibitor, a competitor to BBP-454. If any of these competitors or competitors for our other product candidates receive FDA approval before we do, our product candidates would not be the first treatment on the market, and our market share may be limited. In addition to competition from other companies targeting our target indications, any products we may develop may also face competition from other types of therapies.

Many of our current or potential competitors, either alone or with their strategic partners, have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals, and marketing approved products than we do.

Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient, or are less expensive than any products that we may develop. Furthermore, currently approved products could be discovered to have application for treatment of our targeted disease indications or similar indications, which could give such products significant regulatory and market timing advantages over our product candidates. Our competitors also may obtain FDA or other regulatory

approval for their products more rapidly than we may obtain approval for ours and may obtain orphan product exclusivity from the FDA for indications that we are targeting, which could result in our competitors establishing a strong market position before we are able to enter the market. Additionally, products or technologies developed by our competitors may render our potential product candidates uneconomical or obsolete and we may not be successful in marketing any product candidates we may develop against competitors.

In addition, we could face litigation or other proceedings with respect to the scope, ownership, validity and/or enforceability of our patents relating to our competitors' products and our competitors may allege that our products infringe, misappropriate or otherwise violate their intellectual property. The availability of our competitors' products could limit the demand, and the price we are able to charge, for any products that we may develop and commercialize. See "Risks related to our intellectual property."

***If the market opportunities for our product candidates are smaller than we believe they are, our revenue may be adversely affected, and our business may suffer. Our ability to successfully identify patients and acquire a significant market share will be necessary for us to achieve profitability and growth.***

We focus research and product development on treatments for Mendelian diseases and genetically driven cancers, many of which are rare or orphan indications. Our projections of both the number of individuals who are affected by our target disease indications and have the potential to benefit from treatment with our product candidates, are based on our beliefs and estimates. These estimates have been derived from a variety of sources, including the scientific literature, and may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of these diseases. The number of patients may turn out to be lower than expected. The effort to identify patients with diseases we seek to treat is in early stages, and we cannot accurately predict the number of patients for whom treatment might be possible. Additionally, the potentially addressable patient population for BBP-265, BBP-831, BBP-454, BBP-631 or other product candidates that we may identify may be limited or may not be amenable to treatment with BBP-265, BBP-831, BBP-454, BBP-631 or other product candidates that we may identify, and new patients may become increasingly difficult to identify or gain access to, which would adversely affect our results of operations and our business. Further, even if we obtain significant market share for BBP-265, BBP-831, BBP-454, BBP-631 or other product candidates that we may identify, because the potential target populations are small, we may never achieve profitability despite obtaining such significant market share. In addition, market share could be limited by the availability of other treatments including Vyndamaz (tafamidis) and Vyndaqel (tafamidis meglumine), for which Pfizer Inc. has been approved for the treatment of ATTR-CM in the United States and Japan (Vyndaqel only). As a result, BBP-265 is not the first treatment on the market for ATTR-CM.

#### **Risks related to our business and industry**

***Our future success depends on our ability to retain key employees, directors, consultants and advisors and to attract, retain and motivate qualified personnel.***

We are highly dependent on the management, research and development, clinical, financial and business development expertise of our executive officers, our directors, our Management Committee as well as the other members of our scientific and clinical teams. However, some of these executive officers, directors and other personnel split their time between BridgeBio and certain of our other subsidiaries. For instance, Neil Kumar serves as chief executive officer and a director both to us and Eidos; Uma Sinha serves as chief scientific officer to us and Eidos; Ali Satvat serves as a director both to us and Eidos; Eric David serves as chief executive officer of both Adrenas Therapeutics, Inc. and Aspa Therapeutics, Inc.; Neil Kirby serves as chief operating officer of Origin Biosciences, Inc. and chief executive officer of Phoenix Tissue Repair, Inc.; and both James Montazee and Ali Satvat serve as members of our board of directors and as executive officers of Kohlberg Kravis Roberts & Co. L.P. As a result, these executive officers, directors and members of our Management Committee may not be able to devote their full attention to us, which could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy.

While we believe that we have put in place policies and procedures to identify such conflicts and any such policies and procedures were negotiated at arm's length in conformity with fiduciary duties, such conflicts of interest may nonetheless arise. The existence and consequences of such potential conflicts could expose us to loss of profits, claims by our investors and creditors, and harm our business and our results of operations. The risks related to our dependence upon Dr. Kumar are compounded by Dr. Kumar's significant ownership percentage and Dr. Kumar's role in both our company and our subsidiaries, including Eidos. If we were to lose Dr. Kumar or any of our other executives or key personnel, we may not be able to find appropriate replacements on a timely basis. In addition, because certain of our employees provide a centralized source of support across multiple subsidiaries, the loss of any of these employees could negatively affect the operations of the affected subsidiaries, and our financial condition and results of operations could be materially adversely affected.

Furthermore, each of our executive officers may terminate their employment with us at any time. We do not maintain "key person" insurance for any of our executives or employees. Recruiting and retaining qualified scientific and clinical personnel and, if we progress the development of our drug pipeline toward scaling up for commercialization, sales and marketing personnel, will also be critical to our success. The loss of the services of our executive officers or other key employees could impede the achievement of research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval for and commercialize our product candidates. Competition to hire qualified personnel in our industry is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. Furthermore, to the extent we hire personnel from competitors, we may be subject to allegations that they have been improperly solicited or that they have divulged proprietary or other confidential information, or that their former employers own their research output. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions.

In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

***Our reliance on a central team consisting of a limited number of employees who provide various administrative, research and development and other services across our organization, and on dedicated teams at the subsidiary level presents operational challenges that may adversely affect our business.***

As of March 31, 2019, we had 19 employees who are employed by our wholly-owned subsidiary, BridgeBio Services, Inc., upon which we rely for various administrative, research and development and other support services shared among us. While we believe this structure enables us to reduce certain infrastructure costs, the small size of our central team may cause us to be unable to devote adequate personnel, time and resources to support the operations of all of our subsidiaries, including their research and development activities, employee recruiting and retention efforts and the management financial and accounting and reporting matters. From time to time, members of our central team may not have access to adequate information regarding aspects of the business and operations of our subsidiaries to sufficiently manage these affairs. Additionally, because our dedicated subsidiary-level employees and management are primarily incentivized at the subsidiary level, these employees and management team members may not be sufficiently incentivized to maximize the overall value of our entire organization. If our central team fails to provide adequate administrative, research and development or other services across our entire organization, or our subsidiary-level employees and management do not perform in a manner that aligns with the interests of our entire organization, our business, financial condition and results of operations could be harmed.

***Changes in funding for the FDA, the SEC and other government agencies could hinder their ability to hire and retain key leadership and other personnel, prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal functions on which the operation of our business may rely, which could negatively impact our business.***

The ability of the FDA to review and approve new products or take action with respect to other regulatory matters can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept payment of user fees, and statutory, regulatory, and policy changes. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of the SEC and other government agencies on which our operations may rely, including those that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other agencies may also slow the time necessary for new drugs to be reviewed and/or approved, or for other actions to be taken, by relevant government agencies, which would adversely affect our business. For example, over the last several years, including for 35 days beginning on December 22, 2018, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA and the SEC, have had to furlough critical FDA, SEC and other government employees and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Similarly, a prolonged government shutdown could prevent the timely review of our patent applications by the United States Patent and Trademark Office, or USPTO, which could delay the issuance of any U.S. patents to which we might otherwise be entitled. Further, upon completion of this offering and in our operations as a public company, future government shutdowns could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

***We will need to expand our organization and we may experience difficulties in managing this growth, which could disrupt our operations.***

As of March 31, 2019, we had 152 full-time employees. As we mature, we expect to expand our full-time employee base and to hire more consultants and contractors. Our management may need to divert a disproportionate amount of its attention away from our day-to-day activities and devote a substantial amount of time toward managing these growth activities. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and/or grow revenues could be reduced, and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize product candidates and compete effectively will depend, in part, on our ability to effectively manage any future growth.

***Because we have multiple programs and product candidates in our development pipeline and are pursuing a variety of target indications and treatment modalities, we may expend our limited resources to pursue a particular product candidate and fail to capitalize on development opportunities or product candidates that may be more profitable or for which there is a greater likelihood of success.***

We focus on the development of product candidates to address Mendelian diseases and genetically driven cancers, regardless of the treatment modality or the particular target indication within this space. Because we have limited financial and personnel resources, we may forgo or delay pursuit of opportunities with potential target indications or product candidates that later prove to have greater commercial potential than our current and planned development programs and product candidates. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future

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research and development programs and other future product candidates for specific indications may not yield any commercially viable future product candidates. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may be required to relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such future product candidates.

Additionally, we may pursue additional in-licenses or acquisitions of development-stage assets or programs, which entails additional risk to us. Identifying, selecting and acquiring promising product candidates requires substantial technical, financial and human resources expertise. Efforts to do so may not result in the actual acquisition or license of a successful product candidate, potentially resulting in a diversion of our management's time and the expenditure of our resources with no resulting benefit. For example, if we are unable to identify programs that ultimately result in approved products, we may spend material amounts of our capital and other resources evaluating, acquiring and developing products that ultimately do not provide a return on our investment.

### ***Product liability lawsuits against us could cause us to incur substantial liabilities and could limit commercialization of any product candidates that we may develop.***

We face an inherent risk of product liability exposure related to the testing of product candidates in human clinical trials and will face an even greater risk if we commercially sell any medicines that we may develop. If we cannot successfully defend ourselves against claims that our product candidates or medicines caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates or medicines that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue; and
- the inability to commercialize our product candidates.

Although we maintain product liability insurance, including coverage for clinical trials that we sponsor, it may not be adequate to cover all liabilities that we may incur. We anticipate that we will need to increase our insurance coverage as we commence additional clinical trials and if we successfully commercialize any product candidates. The market for insurance coverage is increasingly expensive, and the costs of insurance coverage will increase as our clinical programs increase in size. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

### ***Our employees, independent contractors, consultants, commercial partners and vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.***

We are exposed to the risk of fraud, misconduct or other illegal activity by our employees, independent contractors, consultants, commercial partners and vendors. Misconduct by these parties could include intentional, reckless and negligent conduct that fails to: comply with the laws of the FDA and comparable foreign regulatory authorities; provide true, complete and accurate information to the FDA and comparable foreign regulatory authorities; comply with manufacturing standards we have established; comply with healthcare fraud and abuse laws in the United States and similar foreign fraudulent misconduct laws; or report financial information or data

accurately or to disclose unauthorized activities. If we obtain FDA approval of our product candidates and begin commercializing those products in the United States, our potential exposure under such laws will increase significantly, and our costs associated with compliance with such laws are also likely to increase. In particular, research, sales, marketing, education and other business arrangements in the healthcare industry are subject to extensive laws designed to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, educating, marketing and promotion, sales and commission, certain customer incentive programs and other business arrangements generally. Activities subject to these laws also involve the improper use of information obtained in the course of patient recruitment for clinical trials, which could result in regulatory sanctions and cause serious harm to our reputation. We intend to adopt a code of business conduct and ethics, but it is not always possible to identify and deter misconduct by employees and third parties, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

***If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.***

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biological, hazardous or radioactive materials.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Our failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

***Unfavorable global economic conditions could adversely affect our business, financial condition or results of operations.***

Our ability to invest in and expand our business and meet our financial obligations, to attract and retain third-party contractors and collaboration partners and to raise additional capital depends on our operating and financial performance, which, in turn, is subject to numerous factors, including the prevailing economic and political conditions and financial, business and other factors beyond our control, such as the rate of unemployment, the number of uninsured persons in the United States, political influences and inflationary pressures. For example, an overall decrease in or loss of insurance coverage among individuals in the United States as a result of unemployment, underemployment or the repeal of certain provisions of the ACA, may decrease the demand for healthcare services and pharmaceuticals. If fewer patients are seeking medical care because they do not have insurance coverage, we may experience difficulties in any eventual commercialization of our product candidates and our business, results of operations, financial condition and cash flows could be adversely affected.

In addition, our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets upon which pharmaceutical and biopharmaceutical companies such as us are dependent for sources of capital. In the past, global financial crises have caused extreme volatility and disruptions in the capital and credit markets. A severe or prolonged economic downturn could result in a variety of risks to our business, including a reduced ability to raise additional capital when needed on acceptable terms, if at all, and weakened demand for our product candidates. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

***Our internal computer systems, or those used by our third-party research institution collaborators, CROs or other contractors or consultants, may fail or suffer security breaches.***

Despite the implementation of security measures, our internal computer systems and those of our future CROs and other contractors and consultants may be vulnerable to damage from computer viruses and unauthorized access. Although to our knowledge we have not experienced any such material system failure or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of development programs and business operations. For example, the loss of clinical trial data from completed, ongoing or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Likewise, we rely on third parties for research and development, the manufacture and supply of drug product and drug substance and to conduct clinical trials, and similar events relating to their computer systems could also have a material adverse effect on our business. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or systems, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development and commercialization of our product candidates could be delayed.

Certain data breaches must also be reported to affected individuals and the government, and in some cases to the media, under provisions of HIPAA, as amended by HITECH, other U.S. federal and state law, and requirements of non-U.S. jurisdictions, including the European Union Data Protection Directive, and financial penalties may also apply.

Our insurance policies may not be adequate to compensate us for the potential losses arising from breaches, failures or disruptions of our infrastructure, catastrophic events and disasters or otherwise. In addition, such insurance may not be available to us in the future on economically reasonable terms, or at all. Further, our insurance may not cover all claims made against us and defending a suit, regardless of its merit, could be costly and divert management's attention.

Furthermore, the loss of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data.

***We or the third parties upon whom we depend may be adversely affected by earthquakes or other natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.***

Earthquakes or other natural disasters could severely disrupt our operations, and have a material adverse effect on our business, results of operations, financial condition and prospects. If a natural disaster, power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure, such as the manufacturing facilities of our third-party CMOs, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time. The disaster recovery and business continuity plans we have in place currently are limited and are unlikely to prove adequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which, particularly when taken together with our lack of earthquake insurance, could have a material adverse effect on our business.



***Our anticipated international operations may expose us to business, regulatory, political, operational, financial, pricing and reimbursement and economic risks associated with doing business outside of the United States.***

We currently have no international operations, but our business strategy incorporates potential international expansion to target patient populations outside the United States. If we receive regulatory approval for and commercialize any of our product candidates in patient populations outside the United States, we may hire sales representatives and conduct physician and patient association outreach activities outside of the United States. Doing business internationally involves a number of risks, including, but not limited to:

- multiple, conflicting, and changing laws and regulations such as privacy regulations, tax laws, export and import restrictions, employment laws, regulatory requirements, and other governmental approvals, permits, and licenses;
- failure by us to obtain and maintain regulatory approvals for the use of our products in various countries;
- additional potentially relevant third-party patent rights;
- complexities and difficulties in obtaining protection and enforcing our intellectual property;
- difficulties in staffing and managing foreign operations;
- complexities associated with managing multiple payor reimbursement regimes, government payors, or patient self-pay systems;
- limits in our ability to penetrate international markets;
- financial risks, such as longer payment cycles, difficulty collecting accounts receivable, the impact of local and regional financial crises on demand and payment for our products, and exposure to foreign currency exchange rate fluctuations;
- natural disasters, political and economic instability, including wars, terrorism, and political unrest, outbreak of disease, boycotts, curtailment of trade, and other business restrictions;
- certain expenses including, among others, expenses for travel, translation, and insurance; and
- regulatory and compliance risks that relate to maintaining accurate information and control over sales and activities that may fall within the purview of the U.S. Foreign Corrupt Practices Act, its books and records provisions, or its anti-bribery provisions.

Any of these factors could significantly harm our potential international expansion and operations and, consequently, our results of operations.

***We have identified material weaknesses in our internal control over financial reporting. If our remediation of the material weaknesses is not effective, or if we experience additional material weaknesses in the future or otherwise fail to maintain an effective system of internal controls in the future, we may not be able to accurately or timely report our financial condition or results of operations, which may adversely affect investor confidence in us and, as a result, the value of our common stock.***

After the completion of this offering, we will be subject to the reporting requirements of the Securities Exchange Act of 1934, as amended, or the Exchange Act, the Sarbanes-Oxley Act and the rules and regulations of the Nasdaq Stock Market. The Sarbanes-Oxley Act requires, among other things, that we maintain effective disclosure controls and procedures and internal controls over financial reporting. Prior to the completion of this offering, we have been a private company with limited accounting personnel to adequately execute our accounting processes and other supervisory resources with which to address our internal control over financial reporting and, as a result, we may experience difficulty in meeting these reporting requirements in a timely manner. To date, we have never conducted a review of our internal control for the purpose of providing the reports required by the Sarbanes-Oxley Act. During our review and testing, we may identify deficiencies and be unable to remediate them before we must provide the required reports.

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In connection with the preparation of our 2017 combined and consolidated financial statements, we and our independent auditors identified material weaknesses in our internal control over financial reporting. A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of our annual or interim consolidated financial statements will not be prevented or detected on a timely basis.

These material weaknesses related to the following:

- We do not have sufficient staffing to enable segregation of duties within accounting functions and do not have sufficient written policies and procedures for accounting and financial reporting. These factors contributed to the lack of a formalized process or controls for our management's timely review and approval of journal entries and related financial statement analysis.
- We do not have finance and accounting staff with the appropriate U.S. GAAP technical expertise to identify, evaluate and account for complex and non-routine transactions. As a result, we did not design and maintain formal accounting policies, processes and controls related to complex transactions necessary for an effective financial reporting process.

As the hiring of additional finance and accounting personnel becomes economically feasible, we intend to take appropriate and reasonable steps to remediate these material weaknesses through the implementation of appropriate segregation of duties and formalization of accounting policies and controls. However, we cannot assure you that these measures will significantly improve or remediate the material weaknesses described above. As of December 31, 2018, the material weaknesses have not been remediated.

In addition, in connection with the audit of the financial statements for the year ended December 31, 2018 of our subsidiary Eidos, which is a public company subject to the reporting requirements of the Exchange Act and the rules and regulations of the Nasdaq Stock Market, Eidos and its independent registered public accounting firm identified a material weakness in Eidos' internal control over financial reporting related to a deficiency in the operation of Eidos' internal controls over the accounting for complex debt and equity transactions and ineffective disclosure controls. While Eidos intends to implement a plan to remediate the material weakness, it has not completed the implementation of this plan and can give no assurance that its current and planned implementation will remediate this deficiency in internal control or that additional material weaknesses or significant deficiencies in its internal control over financial reporting will not be identified in the future.

We may discover additional weaknesses in our system of internal financial and accounting controls and procedures that could result in a material misstatement of our combined and consolidated financial statements. Our internal control over financial reporting will not prevent or detect all errors and all fraud. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system's objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected.

If we are not able to comply with the requirements of the Sarbanes-Oxley Act in a timely manner, or if we are unable to maintain proper and effective internal controls over financial reporting, we may not be able to produce timely and accurate financial statements. If that were to happen, our investors could lose confidence in our reported financial information, the market price of our stock could decline, and we could be subject to sanctions or investigations by the SEC or other regulatory authorities.

***If we fail to maintain an effective system of disclosure controls and internal control over financial reporting, our ability to produce timely and accurate financial statements or comply with applicable regulations could be impaired.***

As a public company, we will be required to maintain internal control over financial reporting and to report any material weaknesses in such internal controls. The Sarbanes-Oxley Act, requires that we evaluate and determine

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the effectiveness of our internal control over financial reporting and, beginning with our second annual report following this offering, provide a management report on internal control over financial reporting. However, while we remain an emerging growth company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm.

Any failure to develop or maintain effective controls, or any difficulties encountered in their implementation or improvement, could harm our results of operations, cause us to fail to meet our reporting obligations, result in a restatement of our financial statements for prior periods, or adversely affect the results of management evaluations and independent registered public accounting firm audits of our internal control over financial reporting that we will eventually be required to include in our periodic reports that will be filed with the SEC. In addition, to the extent we acquire or establish additional consolidated subsidiaries and VIEs, the financial statements of such entities may not be initially prepared by us, and we will not have direct control over their financial statement preparation. As a result, we will, for our financial reporting, depend on what these entities report to us, which could result in our adding monitoring and audit processes, and increase the difficulty of implementing and maintaining adequate controls over our financial processes and reporting in the future, which could lead to delays in our external reporting. In particular, this may occur where we are establishing such entities with partners that do not have sophisticated financial accounting processes in place, or where we are entering into new relationships at a rapid pace, straining our integration capacity. Furthermore, during the course of the audit of Eidos' financial statements for the fiscal year ended December 31, 2018, Eidos discovered certain errors related to the accounting for complex debt and equity transactions, which required Eidos to restate its unaudited financial information for the quarterly periods ended March 31, 2018, June 30, 2018 and September 30, 2018. If we or any of our publicly listed subsidiaries are required to restate previously issued financial statements for any additional periods, our reputation could be impaired which could cause a loss of investor confidence and adversely materially affect our business, operating results and financial condition. Additionally, if we do not receive the information from the consolidated subsidiaries or controlled VIEs on a timely basis, it could cause delays in our external reporting. Ineffective disclosure controls and procedures and internal controls over financial reporting could also cause investors to lose confidence in our reported financial and other information, which would likely have a negative effect on the trading price of our common stock.

Historically, we have relied upon and expect to continue to rely upon third-party contracted service providers to assist with our financial reporting. We are in the process of designing and implementing the internal control over financial reporting required to comply with the Sarbanes-Oxley Act. This process will be time consuming, costly, and complicated. If we are unable to assert that our internal control over financial reporting is effective or when required in the future, if our independent registered public accounting firm issues an adverse opinion on the effectiveness of our internal control over financial reporting, investors may lose confidence in the accuracy and completeness of our financial reports, the market price of our common stock could be adversely affected and we could become subject to investigations by the stock exchange on which our securities are listed, the SEC, or other regulatory authorities, which could require additional financial and management resources.

### **Risks Related to our Equity Securities and this Offering**

***We are an “emerging growth company,” and we cannot be certain if the reduced reporting requirements applicable to emerging growth companies will make our common stock less attractive to investors.***

We are an “emerging growth company” as defined in the JOBS Act, and we intend to take advantage of some of the exemptions from reporting requirements that are applicable to other public companies that are not emerging growth companies, including:

- being permitted to provide only two years of audited financial statements prior to our first filing of our Annual Report on Form 10-K, in addition to any required unaudited interim financial statements, with correspondingly reduced “Management’s discussion and analysis of financial condition and results of operations” disclosure;

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- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements;
- reduced disclosure obligations regarding executive compensation; and
- not being required to hold a non-binding advisory vote on executive compensation or obtain stockholder approval of any golden parachute payments not previously approved.

We cannot predict if investors will find our common stock less attractive because we will rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile. We may take advantage of these reporting exemptions until we are no longer an emerging growth company. We will remain an emerging growth company until the earlier of (1) the last day of the fiscal year (a) following the fifth anniversary of the completion of this offering, (b) in which we have total annual gross revenue of at least \$1.07 billion or (c) in which we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by non-affiliates exceeds \$700 million as of the prior June 30th and (2) the date on which we have issued more than \$1.0 billion in non-convertible debt during the prior three-year period.

Under Section 107(b) of the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards until such time as those standards apply to private companies. Pursuant to Section 107(b) of the JOBS Act, we have elected to use the extended transition period for complying with new or revised accounting standards under Section 102(b)(2) of The JOBS Act. This election allows us to delay the adoption of new or revised accounting standards that have different effective dates for public and private companies until those standards apply to private companies. As a result, our financial statements may not be comparable to companies that comply with public company effective dates, and our stockholders and potential investors may have difficulty in analyzing our operating results if comparing us to such companies.

***The market price of our common stock may be highly volatile, and you may not be able to resell your shares at or above the initial public offering price.***

The market price of our common stock is likely to be volatile. Our stock price could be subject to wide fluctuations in response to a variety of factors, including the following:

- adverse results or delays in our preclinical studies or clinical trials;
- reports of AEs or other negative results in clinical trials of third parties' product candidates that target our product candidates' target indications;
- Inability for us to obtain additional funding on reasonable terms or at all;
- any delay in filing an IND, BLA or NDA for our product candidates and any adverse development or perceived adverse development with respect to the FDA's review of that IND, BLA or NDA;
- failure to develop successfully and commercialize our product candidates;
- announcements we make regarding our current product candidates, acquisition of potential new product candidates and companies and/or in-licensing;
- failure to maintain our existing license arrangements or enter into new licensing and collaboration agreements;
- failure by us or our licensors to prosecute, maintain or enforce our intellectual property rights;
- changes in laws or regulations applicable to future products;

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- inability to obtain adequate clinical or commercial supply for our product candidates or the inability to do so at acceptable prices;
- adverse regulatory decisions, including failure to reach agreement with applicable regulatory authorities on the design or scope of our planned clinical trials;
- failure to obtain and maintain regulatory exclusivity for our product candidates;
- regulatory approval or commercialization of new products or other methods of treating our target disease indications by our competitors;
- failure to meet or exceed financial projections we may provide to the public or to the investment community;
- the perception of the pharmaceutical industry by the public, legislatures, regulators and the investment community;
- announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us, our strategic collaboration partner or our competitors;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- additions or departures of our key scientific or management personnel;
- significant lawsuits, including patent or stockholder litigation, against us;
- changes in the market valuations of similar companies;
- sales or potential sales of substantial amounts of our common stock; and
- trading volume of our common stock.

In addition, companies trading in the stock market in general, and Nasdaq, in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance.

***If you purchase our common stock in this offering, you will incur immediate and substantial dilution in the book value of your shares.***

Investors purchasing shares of common stock in this offering will pay a price per share that substantially exceeds the pro forma as adjusted book value per share of our tangible assets after subtracting our liabilities.

As a result, investors purchasing shares of common stock in this offering will incur immediate dilution of \$10.53 per share, based on the assumed initial public offering price of \$15.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, and our pro forma adjusted net tangible book value as of March 31, 2019. Further, based on these assumptions, investors purchasing shares of common stock in this offering will contribute approximately 31.8% of the total amount invested by stockholders since our inception, but will own only approximately 13.0% of the shares of common stock outstanding. For information on how the foregoing amounts were calculated, see “Dilution” located elsewhere in this prospectus.

This dilution is due to the substantially lower price paid by our investors who purchased shares prior to this offering as compared to the price offered to the public in this offering, and the conversion of common units and management incentive units granted to our employees and non-employees. As a result of the dilution to investors purchasing shares in this offering, as well as other factors, investors may receive significantly less than the purchase price paid in this offering, if anything, in the event of our liquidation.

***Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our equity incentive plans, would result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to fall.***

We will need additional capital in the future to continue our planned operations. To the extent we raise additional capital by issuing equity securities, our stockholders may experience substantial dilution. We may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. If we sell common stock, convertible securities or other equity securities in more than one transaction, investors may be materially diluted by subsequent sales. These sales may also result in material dilution to our existing stockholders, and new investors could gain rights superior to our existing stockholders.

Pursuant to our 2019 Stock Option and Incentive Plan, or the 2019 Plan, which will become effective upon the effectiveness of the registration statement of which this prospectus is a part, we are authorized to grant stock options and other equity-based awards to our employees, directors and consultants. If our board of directors elects to increase the number of shares available for future grant and our stockholders approve of such an increase at our annual meeting, our stockholders may experience additional dilution, and our stock price may fall.

***A significant portion of our total outstanding shares is restricted from immediate resale but may be sold into the market in the near future, which could cause the market price of our common stock to decline significantly.***

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, upon the expiration of the market standoff and lock-up agreements, the early release of these agreements, or the perception in the market that the holders of a large number of shares of common stock intend to sell shares, could reduce the market price of our common stock. After this offering and based on the assumed initial public offering price of \$15.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, upon the completion of this offering, we will have 114,999,967 shares of common stock outstanding assuming the exchange of all outstanding units of BridgeBio Pharma LLC into 99,999,967 shares of our common stock upon the Reorganization, as if the Reorganization has occurred as of March 31, 2019. Of these shares, the 15,000,000 shares we are selling in this offering (excluding any shares sold to our directors and officers in the directed share program) may be resold in the public market immediately, unless purchased by our affiliates. The remaining 99,999,967 shares, or 87.0% of our outstanding shares after this offering, are currently prohibited or otherwise restricted under securities laws, market standoff agreements entered into by our stockholders with us or lock-up agreements entered into by our stockholders with the underwriters; however, subject to applicable securities law restrictions and excluding shares of restricted stock that will remain unvested, these shares will be able to be sold in the public market beginning 180 days after the date of this prospectus. J.P. Morgan Securities LLC and Goldman Sachs & Co. LLC may, in their sole discretion, release all or some portion of the shares subject to lock-up agreements at any time and for any reason. In addition, the 99,999,967 shares of unvested restricted stock and common stock issued and outstanding as of March 31, 2019 will become available for sale immediately upon the vesting of such shares, as applicable, and the expiration of any applicable market standoff or lock-up agreements. Shares issued upon the exercise of stock options outstanding under our equity incentive plans or pursuant to future awards granted under those plans will become available for sale in the public market to the extent permitted by the provisions of applicable vesting schedules, any applicable market standoff and lock-up agreements, and Rule 144 and Rule 701 under the Securities Act of 1933, as amended, or the Securities Act. See the section titled “Shares eligible for future sale” for additional information.

Moreover, after the completion of this offering, based on the assumed initial public offering price of \$15.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, holders of an aggregate of 99,999,967 shares of our common stock will have rights, subject to conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. We also plan to register all shares of common stock that we may issue under our equity compensation plans. Once we register these shares, they can be freely sold in the public market upon

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issuance and once vested, subject to volume limitations applicable to affiliates and the lock-up agreements described in the section titled “Underwriting (Conflicts of Interest)” in this prospectus. If any of these additional shares are sold, or if it is perceived that they will be sold, in the public market, the market price of our common stock could decline.

***If securities analysts do not publish research or reports about our business or if they publish negative evaluations of our stock, the price of our stock could decline.***

The trading market for our common stock will rely in part on the research and reports that industry or financial analysts publish about us or our business. We do not currently have and may never obtain research coverage by industry or financial analysts. If no or few analysts commence coverage of us, the trading price of our stock could decrease. Even if we do obtain analyst coverage, if one or more of the analysts covering our business downgrade their evaluations of our stock, the price of our stock could decline. If one or more of these analysts cease to cover our stock, we could lose visibility in the market for our stock, which in turn could cause our stock price to decline.

***Our principal stockholders and certain members of our management own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.***

When this offering is completed, KKR Genetic Disorder L.P., or together with its affiliates, KKR, will beneficially own approximately 29.9% of our outstanding shares of common stock, and KKR, together with Viking Global Opportunities Illiquid Investments Sub-Master LP and Neil Kumar, our chief executive officer, will beneficially own approximately 56.6% of our outstanding shares of common stock (assuming no exercise of the underwriters’ over-allotment option). Therefore, even after this offering, these stockholders will have the ability to influence us through their ownership positions. These stockholders may be able to determine all matters requiring stockholder approval. For example, these stockholders, acting together, may be able to control elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may believe are in your best interest as one of our stockholders.

***We have broad discretion in the use of the net proceeds from this offering and may not use them effectively.***

Our management will have broad discretion in the application of the net proceeds from this offering, including for any of the purposes described in the section entitled “Use of Proceeds” located elsewhere in this prospectus, and you will not have the opportunity as part of your investment decision to assess whether the net proceeds are being used appropriately. Because of the number and variability of factors that will determine our use of the net proceeds from this offering, their ultimate use may vary substantially from their currently intended use. The failure by our management to apply these funds effectively could harm our business. Pending their use, we may invest the net proceeds from this offering in short-term, investment-grade, interest-bearing securities. These investments may not yield a favorable return to our stockholders.

***Provisions in our amended and restated certificate of incorporation and amended and restated bylaws, as well as provisions of Delaware law, could make it more difficult for a third party to acquire us or increase the cost of acquiring us, even if doing so would benefit our stockholders or remove our current management.***

Our amended and restated certificate of incorporation and amended and restated bylaws that will become effective immediately prior to the completion of this offering and Delaware law contain provisions that may have the effect of delaying or preventing a change in control of us or changes in our management. Our amended and restated certificate of incorporation and amended and restated bylaws, which will become effective upon the completion of this offering, include provisions that:

- authorize “blank check” preferred stock, which could be issued by our board of directors without stockholder approval and may contain voting, liquidation, dividend and other rights superior to our common stock;

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- create a classified board of directors whose members serve staggered three-year terms;
- specify that special meetings of our stockholders can be called only by our board of directors or stockholders holding at least 25% of our outstanding voting stock;
- prohibit stockholder action by written consent;
- establish an advance notice procedure for stockholder approvals to be brought before an annual meeting of our stockholders, including proposed nominations of persons for election to our board of directors;
- provide that vacancies on our board of directors may be filled only by a majority of directors then in office, even if less than a quorum, or by the holders of a majority of the outstanding shares of capital stock then entitled to vote at an election of directors;
- specify that no stockholder is permitted to cumulate votes at any election of directors;
- expressly authorize our board of directors to modify, alter or repeal our amended and restated bylaws; and
- require supermajority votes of the holders of our common stock to amend specified provisions of our amended and restated certificate of incorporation and amended and restated bylaws.

These provisions, alone or together, could delay or prevent hostile takeovers and changes in control or changes in our management.

In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which limits the ability of stockholders owning in excess of 15% of our outstanding voting stock to merge or combine with us. Any provision of our amended and restated certificate of incorporation or amended and restated bylaws or Delaware law that has the effect of delaying or deterring a change in control could limit the opportunity for our stockholders to receive a premium for their shares of our common stock, and could also affect the price that some investors are willing to pay for our common stock.

***Our amended and restated bylaws will designate specific courts as the exclusive forum for certain litigation that may be initiated by our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us.***

Pursuant to our amended and restated bylaws, which will be in effect upon the completion of this offering, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware (or, if the Chancery Court does not have jurisdiction, the federal district court for the District of Delaware or other state courts of the State of Delaware) will be the sole and exclusive forum for state law claims for (i) any derivative action or proceeding brought on our behalf; (ii) any action asserting a claim of breach of a fiduciary duty or other wrongdoing by any of our directors, officers, employees or agents to us or our stockholders; (iii) any action asserting a claim against us arising pursuant to any provision of the Delaware General Corporation Law or our certificate of incorporation or bylaws; (iv) any action to interpret, apply, enforce or determine the validity of our certificate of incorporation or bylaws; or (v) any action asserting a claim governed by the internal affairs doctrine. This exclusive forum provision will not apply to any causes of action arising under the Securities Act or the Exchange Act. The forum selection clauses in our amended and restated bylaws, which will be in effect upon the completion of this offering, may limit our stockholders' ability to obtain a favorable judicial forum for disputes with us.

***We are party to a loan and security agreement that contains operating and financial covenants that may restrict our business and financing activities.***

In June 2018, we entered into a loan and security agreement, or the Loan and Security Agreement, with Hercules Capital, Inc., or Hercules, pursuant to which we were extended a term loan in the aggregate principal amount of up to \$35.0 million. In December 2018, we entered into an amendment to the Loan and Security Agreement with



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Hercules, pursuant to which we were extended an additional term loan in the aggregate principal amount of up to \$20.0 million. In May 2019, we entered into a second amendment to the Loan and Security Agreement with Hercules, pursuant to which we were extended a second additional term loan in the aggregate principal amount of up to \$20.0 million, increasing the total principal amount outstanding to \$75.0 million under the Loan and Security Agreement, as amended to date, or the Amended and Restated Loan and Security Agreement. The Amended and Restated Loan and Security Agreement may restrict our ability, among other things, to:

- sell, transfer or otherwise dispose of any of our business or property, subject to limited exceptions;
- make material changes to our business;
- enter into transactions resulting in significant changes to the voting control of our stock;
- make certain changes to our organizational structure;
- consolidate or merge with other entities or acquire other entities;
- incur additional indebtedness or create encumbrances on our assets;
- pay dividends, or make distributions on and, in certain cases, repurchase our stock;
- enter into transactions with our affiliates;
- repay subordinated indebtedness; or
- make certain investments.

In addition, we are required under our Amended and Restated Loan and Security Agreement to comply with various operating covenants and default clauses that may restrict our ability to finance our operations, engage in business activities or expand or fully pursue our business strategies. A breach of any of these covenants or clauses could result in a default under the Amended and Restated Loan and Security Agreement, which could cause all of the outstanding indebtedness under the facility to become immediately due and payable.

If we are unable to generate sufficient cash to repay our debt obligations when they become due and payable, we may not be able to obtain additional debt or equity financing on favorable terms, if at all, which may negatively affect our business operations and financial condition.

Under the Amended and Restated Loan and Security Agreement, we also have an obligation to pledge our equity interests in our subsidiaries. In addition, certain of our non-operating subsidiaries, which are subsidiaries other than those predominantly involved in advancing our development programs are also obligated to enter into a joinder agreement, whereby they shall also agree to comply with the terms of the Amended and Restated Loan and Security Agreement.

***Our operating results may fluctuate significantly, which makes our future operating results difficult to predict and could cause our operating results to fall below expectations or our guidance.***

Our quarterly and annual operating results may fluctuate significantly in the future, which makes it difficult for us to predict our future operating results. Our operating results may fluctuate due to a variety of factors, many of which are outside of our control and may be difficult to predict, including the following:

- the timing, results and cost of, and level of investment in, our clinical development activities for BBP-265, BBP-831, BBP-454 and BBP-631, and any other product candidates we may identify and pursue, which may change from time to time;
- the cost of manufacturing BBP-009 and the related materials or other product candidates that we may identify, which may vary depending on the quantity of production and the terms of agreements with manufacturers;

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- our ability to conduct clinical trials of BBP-265, BBP-831, BBP-454 and BBP-631 in accordance with our plans and to obtain regulatory approval for BBP-265, BBP-831, BBP-454 and BBP-631 or other product candidates that we may identify, and the timing and scope of any such approvals we may receive;
- the timing and success or failure of clinical trials for competing product candidates, or any other change in the competitive landscape of our industry, including consolidation among our competitors or partners;
- expenditures that we or will or may incur to acquire or develop additional product candidates and technologies;
- Our ability to attract, hire and retain qualified personnel;
- the level of demand for BBP-265, BBP-831, BBP-454, BBP-631 or other product candidates that we may identify, should they receive approval, which may vary significantly;
- future accounting pronouncements or changes in our accounting policies;
- the risk/benefit profile, cost and reimbursement policies with respect to BBP-265, BBP-831, BBP-454, BBP-631 or other product candidates that we may identify, if approved, and existing and potential future drugs that compete with our product candidates; and
- the changing and volatile U.S., European and global economic environments.

The cumulative effects of these factors could result in large fluctuations and unpredictability in our quarterly and annual operating results. As a result, comparing our operating results on a period-to-period basis may not be meaningful. This variability and unpredictability could also result in our failing to meet the expectations of industry or financial analysts or investors for any period. If our operating results fall below the expectations of analysts or investors or below any forecasts we may provide to the market, or if the forecasts we provide to the market are below the expectations of analysts or investors, the price of our common stock could decline substantially.

### ***Our future ability to utilize our net operating loss carryforwards and certain other tax attributes may be limited.***

We have incurred substantial losses during our history and we do not expect to become profitable in the near future and we may never achieve profitability. To the extent that we continue to generate taxable losses, unused losses will carry forward to offset a portion of future taxable income, if any, subject to expiration of such carryforwards in the case of carryforwards generated prior to 2018. In addition, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or the Code, if a corporation undergoes an “ownership change,” generally defined as a greater than 50 percentage point change (by value) in its equity ownership over a three-year period, the corporation’s ability to use its pre-change net operating loss carryforwards, or NOLs, and other pre-change tax attributes (such as research and development tax credits) to offset its post-change income or taxes may be limited. Our prior equity offerings and other changes in our stock ownership may have resulted in such ownership changes. In addition, we may experience ownership changes in the future as a result of this offering or subsequent shifts in our stock ownership, some of which are outside of our control. As a result, if we earn net taxable income, our ability to use our pre-change NOLs to offset U.S. federal taxable income may be subject to limitations, which could potentially result in increased future tax liability to us. Additional limitations on our ability to utilize our NOLs to offset future taxable income may arise as a result of our corporate structure whereby NOLs generated by certain of our subsidiaries or controlled entities may not be available to offset taxable income earned by other subsidiaries, controlled entities or BridgeBio. In addition, under the Tax Act, the amount of post-2017 NOLs that we are permitted to deduct in any taxable year is limited to 80% of our taxable income in such year. The Tax Act generally eliminates the ability to carry back any NOLs to prior taxable years, while allowing post-2017 unused NOLs to be carried forward indefinitely. There is a risk that due to changes under the Tax Act, regulatory changes, or other unforeseen reasons, our existing NOLs could expire or otherwise be unavailable to offset future income tax liabilities. At the state level, there may also be periods during which

the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed. For these reasons, we may not be able to realize a tax benefit from the use of our NOLs, even if we attain profitability.

***Comprehensive tax reform legislation could adversely affect our business and financial condition.***

On December 22, 2017, the Tax Act was signed into law. The Tax Act, among other things, contains significant changes to corporate taxation, including (i) reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, (ii) limitation of the tax deduction for interest expense to 30% of adjusted earnings (except for certain small businesses), (iii) limitation of the deduction for net operating losses to 80% of current year taxable income in respect of net operating losses generated during or after 2018 and elimination of net operating loss carrybacks, (iv) one-time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, (v) immediate deductions for certain new investments instead of deductions for depreciation expense over time, and (vi) modifying or repealing many business deductions and credits. Any federal net operating loss incurred in 2018 and in future years may now be carried forward indefinitely pursuant to the Tax Act. It is uncertain if and to what extent various states will conform to the newly enacted federal tax law. We will continue to examine the impact the Tax Act may have on our business.

***We have never and do not currently intend to pay dividends on our common stock, and, consequently, our stockholders' ability to achieve a return on their investment will depend on appreciation in the price of our common stock.***

We have never paid cash dividends on any of our capital stock and do not currently intend to pay any cash dividends on our common stock for the foreseeable future. In addition, pursuant to the Amended and Restated Loan and Security Agreement with Hercules, we are not permitted to declare or pay any cash dividends or make cash distributions on any class of our capital stock or any other equity interest, except in limited circumstances. We currently intend to invest our future earnings, if any, to fund our growth. Therefore, you are not likely to receive any dividends on your common stock for the foreseeable future. Since we do not intend to pay dividends, your ability to receive a return on your investment will depend on any future appreciation in the market value of our common stock. There is no guarantee that our common stock will appreciate or even maintain the price at which our holders have purchased it.

***An active trading market for our common stock may not develop.***

Prior to this offering, there has been no public market for shares of our common stock. Although we have applied to list our common stock on the Nasdaq Global Market, an active trading market for our shares may never develop or be sustained following this offering. The initial public offering price of our common stock was determined through negotiations between us and the underwriters. This initial public offering price may not be indicative of the market price of our common stock after this offering. In the absence of an active trading market for our common stock, investors may not be able to sell their common stock at or above the initial public offering price or at the time that they would like to sell.

***We will incur significant costs as a result of operating as a new public company, and our management will devote substantial time to new compliance initiatives.***

As a public company, we will incur significant legal, accounting and other expenses that we did not incur as a private company. We will be subject to the reporting requirements of the Exchange Act, which will require, among other things, that we file with the SEC, annual, quarterly and current reports with respect to our business and financial condition and that of our consolidated subsidiaries. In addition, the Sarbanes-Oxley Act, as well as rules subsequently adopted by the SEC and Nasdaq to implement provisions of the Sarbanes-Oxley Act, impose significant requirements on public companies, including requiring establishment and maintenance of effective disclosure and financial controls and changes in corporate governance practices.

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Further, in July 2010, the Dodd-Frank Wall Street Reform and Consumer Protection Act, or the Dodd-Frank Act, was enacted. There are significant corporate governance and executive compensation-related provisions in the Dodd-Frank Act that require the SEC to adopt additional rules and regulations in these areas such as “say on pay” and proxy access. Recent legislation permits emerging growth companies to implement many of these requirements over a longer period and up to five years from the pricing of this offering. We intend to take advantage of this new legislation, but cannot guarantee that we will not be required to implement these requirements sooner than budgeted or planned and thereby incur unexpected expenses. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate.

We expect the rules and regulations applicable to public companies to substantially increase our legal and financial compliance costs and to make some activities more time-consuming and costly. If these requirements divert the attention of our management and personnel from other business concerns, they could have a material adverse effect on our business, financial condition and results of operations. The increased costs will decrease our net income or increase our net loss, and may require us to reduce costs in other areas of our business, including our subsidiaries. For example, we expect these rules and regulations to make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain the same or similar coverage. We cannot predict or estimate the amount or timing of additional costs we may incur to respond to these requirements. The impact of these requirements could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers.

## SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus, including the sections entitled “Prospectus Summary,” “Risk Factors,” “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and “Business,” contains forward-looking statements, within the meaning of the Private Securities Litigation Reform Act of 1995, concerning our business, operations and financial performance and condition, as well as our plans, objectives and expectations for our business operations and financial performance and condition that are based on our management’s belief and assumptions and on information currently available to our management. Although we believe that the expectations reflected in these forward-looking statements are reasonable, these statements relate to our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans, objectives of management and expected market growth, and involve known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to be materially different from any future results, levels of activity, performance or achievements expressed or implied by these forward-looking statements. Forward-looking statements in this prospectus include, but are not limited to, statements about:

- the success, cost and timing of our clinical development of our product candidates, including the progress of, and results from, our ongoing and planned Phase 3 clinical trials of BBP-265 and our clinical trials of BBP-831;
- our ability to initiate, recruit and enroll patients in and conduct our clinical trials at the pace that we project;
- the timing of our submissions to the FDA and any review or comments on data that we will need to generate to file our NDAs;
- our plans to implement certain development strategies;
- our ability to obtain and maintain regulatory approval of our product candidates, and any related restrictions, limitations or warnings in the label of any of our product candidates, if approved;
- our ability to compete with companies currently marketing or engaged in the development of treatments that our product candidates are designed to target;
- our reliance on third parties to conduct our clinical trials and to manufacture drug substance for use in our clinical trials;
- the size and growth potential of the markets for BBP-265, BBP-831, BBP-631, BBP-454 and any of our current product candidates or other product candidates we may identify and pursue, and our ability to serve those markets;
- our ability to identify and advance through clinical development any additional product candidates;
- the commercialization of our current product candidates and any other product candidates we may identify and pursue, if approved, including our ability to successfully build a specialty sales force and commercial infrastructure to market our current product candidates and any other product candidates we may identify and pursue;
- our ability to retain and recruit key personnel;
- our ability to obtain and maintain adequate intellectual property rights;
- our expectations regarding government and third-party payor coverage and reimbursement;
- our estimates of our expenses, ongoing losses, capital requirements and our needs for or ability to obtain additional financing;
- our expected uses of the net proceeds to us from this offering;
- our expectations regarding the time during which we will be an emerging growth company under the JOBS Act;
- our financial performance; and
- developments and projections relating to our competitors or our industry.

In some cases, you can identify forward-looking statements by terminology such as “may,” “will,” “should,” “expects,” “intends,” “plans,” “anticipates,” “believes,” “estimates,” “predicts,” “potential,” “continue” or the

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negative of these terms or other comparable terminology. These statements are only predictions. You should not place undue reliance on forward-looking statements because they involve known and unknown risks, uncertainties and other factors, which are, in some cases, beyond our control and which could materially affect results. Factors that may cause actual results to differ materially from current expectations include, among other things, those listed under “Risk Factors” and elsewhere in this prospectus. If one or more of these risks or uncertainties occur, or if our underlying assumptions prove to be incorrect, actual events or results may vary significantly from those implied or projected by the forward-looking statements. No forward-looking statement is a guarantee of future performance. You should read this prospectus and the documents that we reference in this prospectus and have filed with the SEC as exhibits to the registration statement, of which this prospectus is a part, completely and with the understanding that our actual future results may be materially different from any future results expressed or implied by these forward-looking statements.

The forward-looking statements in this prospectus represent our views as of the date of this prospectus. We anticipate that subsequent events and developments will cause our views to change. However, while we may elect to update these forward-looking statements at some point in the future, we assume no obligation to update or revise any forward-looking statements except to the extent required by applicable law. You should, therefore, not rely on these forward-looking statements as representing our views as of any date subsequent to the date of this prospectus.

In addition, statements that “we believe” and similar statements reflect our beliefs and opinions on the relevant subject. These statements are based upon information available to us as of the date of this prospectus, and while we believe such information forms a reasonable basis for such statements, such information may be limited or incomplete, and our statements should not be read to indicate that we have conducted an exhaustive inquiry into, or review of, all potentially available relevant information. These statements are inherently uncertain and you are cautioned not to rely unduly upon these statements.

## **MARKET, INDUSTRY AND OTHER DATA**

We obtained the industry, market and competitive position data used throughout this prospectus from our own internal estimates and research, as well as from independent market research, industry and general publications and surveys, governmental agencies and publicly available information in addition to research, surveys and studies conducted by third parties. Internal estimates are derived from publicly available information released by industry analysts and third-party sources, our internal research and our industry experience, and are based on assumptions made by us based on such data and our knowledge of our industry and market, which we believe to be reasonable. In some cases, we do not expressly refer to the sources from which this data is derived. In that regard, when we refer to one or more sources of this type of data in any paragraph, you should assume that other data of this type appearing in the same paragraph is derived from the same sources, unless otherwise expressly stated or the context otherwise requires. In addition, while we believe the industry, market and competitive position data included in this prospectus is reliable and based on reasonable assumptions, such data involve risks and uncertainties and are subject to change based on various factors, including those discussed in “Risk Factors.” These and other factors could cause results to differ materially from those expressed in the estimates made by the independent parties or by us.

## USE OF PROCEEDS

We estimate that our net proceeds from the sale of 15,000,000 shares of our common stock in this offering will be approximately \$203.7 million, or \$235.1 million if the underwriters exercise in full their option to purchase 2,250,000 additional shares, assuming an initial public offering price of \$15.00 per share, the midpoint of the price range set forth on the cover page of this prospectus, and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

A \$1.00 increase (decrease) in the assumed initial public offering price of \$15.00 per share, the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) the net proceeds to us from this offering by \$14.0 million, assuming the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. An increase (decrease) of 1,000,000 shares in the number of shares offered by us, as set forth on the cover page of this prospectus, would increase (decrease) our net proceeds from this offering by \$14.0 million, assuming no change in the assumed initial public offering price per share and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

We intend to use the net proceeds from this offering, together with our current capital resources of \$226.0 million, which excludes \$147.1 million of cash and cash equivalents held by Eidos as of March 31, 2019, as follows:

- approximately \$190.0 million to \$210.0 million to fund the continued development, pre-commercialization, and potential commercialization costs of our key value drivers, BBP-831, BBP-454 and BBP-631, including: with respect to BBP-831, the completion of our Phase 2 clinical trial and potential NDA submission as a treatment for CCA in the second-line or later setting, as well as the advancement of our planned clinical trials in achondroplasia, first-line CCA, adjuvant UC, and tumors with FGFR fusions; with respect to BBP-454, the anticipated nomination of a development candidate and initiation of IND-enabling studies; and with respect to BBP-631, the anticipated filing of an IND and initiation of a first-in-human clinical trial;
- approximately \$45.0 million to \$55.0 million to fund the continued development, pre-commercialization, and potential commercialization costs of our development programs that are currently in clinical development, BBP-870 and BBP-589, including: with respect to BBP-870, the completion of our ongoing Phase 2/3 clinical trial and potential NDA submission as a treatment for MoCD Type A; and with respect to BBP-589, the completion of our ongoing Phase 1/2 clinical trial;
- approximately \$75.0 million to \$85.0 million to fund continued development of the other programs in our pipeline, including designing and conducting preclinical studies and clinical trials, as well as funding discovery, manufacturing, research and development;
- approximately \$45.0 million to \$55.0 million to fund the acquisition of and drug development activities related to new programs; although we have no material agreements, commitments or understandings with respect to any in-license or acquisition, we have and plan to continue to evaluate such opportunities and engage in related discussions with other business entities from time to time; and
- the remainder to fund working capital and for general corporate purposes.

From time to time, to maintain or increase our ownership position in our subsidiaries, we may make additional investments in or purchase equity in our subsidiaries.

We estimate that our current capital resources, along with the net proceeds from this offering, will be sufficient for us to fund our operating expenses and capital expenditure requirements through the next 24 months.

This expected use of the net proceeds from this offering represents our intentions based upon our current plans and business conditions. We cannot predict with certainty all of the particular uses for the net proceeds to be received upon the completion of this offering or the amounts that we will actually spend on the uses set forth above.



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The amounts and timing of our actual expenditures and the extent of our research and development activities may vary significantly depending on numerous factors, including the progress of our development efforts, the status of and results from any preclinical studies or clinical trials we may commence in the future, our ability to take advantage of expedited programs or to obtain regulatory approval for any other product candidates we may identify and pursue, the timing and costs associated with the manufacture and supply of any other product candidates we may identify and pursue for clinical development or commercialization, and any unforeseen cash needs. As a result, our management will retain broad discretion over the allocation of the net proceeds from this offering.

We intend to invest the net proceeds in a variety of capital preservation investments, including short-term, investment-grade, interest-bearing instruments and U.S. government securities.

## **DIVIDEND POLICY**

We have never declared or paid any cash dividends on our capital stock. We do not anticipate paying any dividends on our capital stock in the foreseeable future. We currently intend to retain all available funds and any future earnings to fund the development and growth of our business. Any future determination to declare dividends will be subject to the discretion of our board of directors and will depend on various factors, including applicable laws, our results of operations, financial condition, future prospects and any other factors deemed relevant by our board of directors. Investors should not purchase our common stock with the expectation of receiving cash dividends.

In addition, pursuant to the Amended and Restated Loan and Security Agreement with Hercules, we are not permitted to declare or pay any cash dividends or make cash distributions on any class of our capital stock or any other equity interest, except in limited circumstances.

## REORGANIZATION

### Existing LLC (BridgeBio Pharma LLC)

Currently, the capital structure of BridgeBio Pharma LLC consists of seven classes of membership units: management incentive units; common units; founder units; Series A preferred units; Series B preferred units; Series C preferred units; and Series D preferred units. The LLC entity is the direct parent company of the various subsidiaries of the LLC entity. The subsidiaries of the LLC entity are focused on identifying, acquiring, developing and, if approved, commercializing our product candidates under development.

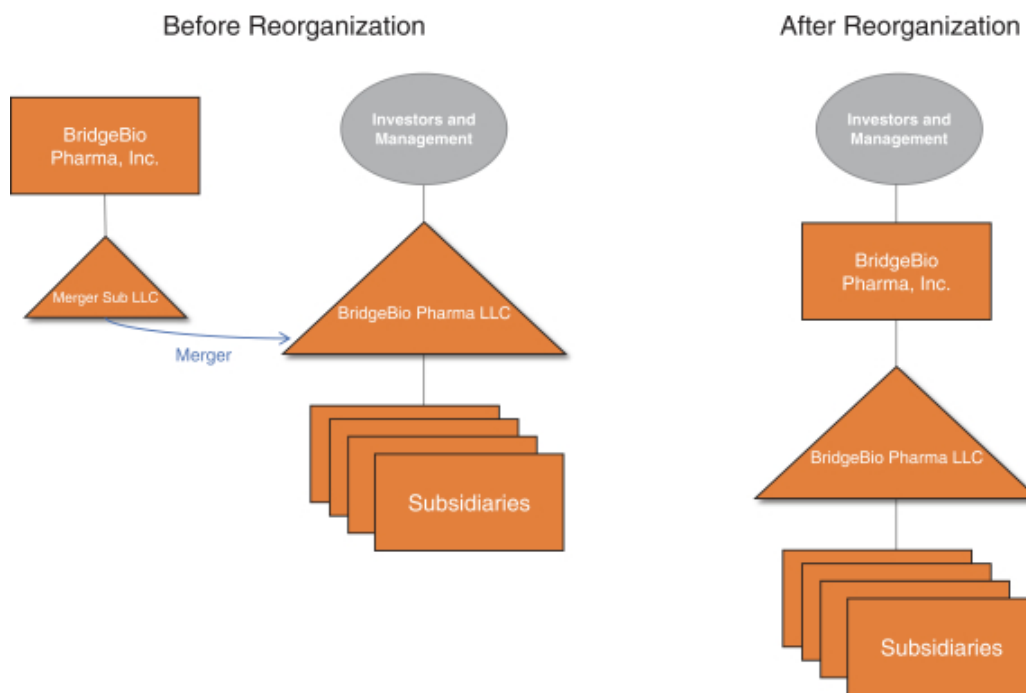
### Corporate Reorganization

Prior to the completion of this offering, we intend to engage in a series of transactions, which we refer to collectively as the Reorganization. As a result of the Reorganization, we anticipate BridgeBio Pharma LLC will ultimately become a wholly-owned subsidiary of the Corporation, the Corporation will become the registrant for purposes of this offering and our combined and consolidated financial statements will be reported from the Corporation.

On May 17, 2019, we formed the Corporation as a stand-alone entity. We believe the remaining steps to the Reorganization will include:

- the Corporation will create BridgeBio Pharma Merger Sub LLC, a Delaware limited liability company, or Merger Sub LLC, which will be a wholly-owned subsidiary of the Corporation;
- Merger Sub LLC will merge with and into BridgeBio Pharma LLC, with BridgeBio Pharma LLC as the surviving entity of the merger; and
- any other steps necessary to effect the Corporation being the registrant for this offering and our combined and consolidated financial statements being reported from the Corporation going forward after the Reorganization.

The chart below shows, on a simplified basis, our organizational structure immediately prior to and immediately following the Reorganization:



As part of the Reorganization, the holders of existing units in the LLC entity will exchange those units for shares of common stock of the Corporation, after which those holders will have received 100% of the outstanding capital stock of the Corporation as of immediately prior to the completion of the offering. The capital stock of the Corporation will be allocated to the holders of existing units in the LLC entity pursuant to the distribution provisions of the Fourth Amended and Restated Limited Liability Company Agreement of the LLC entity, or the LLC Agreement, based upon the liquidation value of the LLC entity, assuming it was liquidated immediately prior to the completion of this offering with a value implied by the assumed initial public offering price of \$15.00 per share, the midpoint of the price range set forth on the cover page of this prospectus. Shares of common stock of the Corporation will be allocated pursuant to the distribution provisions of the LLC Agreement, based on the assumed initial public offering price of \$15.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, as follows:

- holders of Series D preferred units of the LLC entity will first receive 19,949,285 shares of common stock of the Corporation, which is the aggregate distribution equal to the amount such holders are entitled to receive based on their Series D unit value in accordance with the LLC Agreement;
- holders of Series C preferred units and Series B preferred units of the LLC entity will then receive 12,944,871 shares of common stock of the Corporation, which is the aggregate distribution equal to the amount such holders are entitled to receive based on their Series C unit value and Series B unit value, respectively, in accordance with the LLC Agreement;
- holders of Series A preferred units of the LLC entity will then receive 902,778 shares of common stock of the Corporation, which is the aggregate distribution equal to the amount such holders are entitled to receive based on their Series A unit value in accordance with the LLC Agreement;
- holders of founder units and common units of the LLC entity will then receive 742,896 shares of common stock of the Corporation, which is the aggregate distribution equal to the amount such holders

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are entitled to receive based on their founder unit value and common unit value, respectively, in accordance with the LLC Agreement;

- holders of units other than Series D preferred units will then receive 30,406,399 shares of common stock of the Corporation, which is the aggregate distribution equal to the amount such holders are entitled to receive based on the applicable unit cap for such holders in accordance with the LLC Agreement; and
- holders of all units of BridgeBio Pharma LLC will then receive 35,053,738 shares of common stock of the Corporation, which reflects the remaining proceeds following the above distributions.

As a result of the Reorganization, the holders of existing units in BridgeBio Pharma LLC will collectively own 99,999,967 shares of common stock of the Corporation.

Because the exact number of shares of the Corporation's common stock to be issued to holders of units in BridgeBio Pharma LLC in the Reorganization is based on the initial public offering price, to the extent that the actual initial public offering price per share for this offering is greater or less than \$15.00, which is the midpoint of the price range set forth on the cover page of this prospectus, the actual number of shares of common stock to be issued to holders of units in BridgeBio Pharma LLC and the total number of shares of common stock outstanding following the Reorganization will be adjusted accordingly following this offering. See "Pricing Sensitivity Analysis" for how the number of shares to be issued in the Reorganization would be affected by an initial public offering price per share of common stock at the low-, mid- and high-points of the estimated price range set forth on the cover of this prospectus.

### **Treatment of Outstanding Incentive Equity of BridgeBio Pharma LLC**

In connection with the Reorganization, all of the outstanding incentive equity of BridgeBio Pharma LLC, which is currently comprised of management incentive units and common units of BridgeBio Pharma LLC, will be exchanged for shares of the common stock and restricted stock of BridgeBio Pharma, Inc. as provided for in the distribution provisions of the LLC Agreement. The portion of the outstanding management incentive units and common units of BridgeBio Pharma LLC that have vested as of the consummation of the Reorganization will be exchanged for shares of common stock of BridgeBio Pharma, Inc., and the remaining portion of unvested outstanding management incentive units and common units of BridgeBio Pharma LLC will be exchanged for shares of BridgeBio Pharma, Inc.'s restricted common stock. The shares of restricted common stock will be subject to time-based vesting conditions, in accordance with the terms and conditions of the management incentive units and common units of BridgeBio Pharma LLC from which such shares are exchanged.

### **Holding Company Structure**

Following the consummation of the Reorganization, BridgeBio Pharma, Inc. will be a holding company, and its sole material asset will be 100% of the membership units in BridgeBio Pharma LLC. As the sole and managing member of BridgeBio Pharma LLC, BridgeBio Pharma, Inc. will operate and control all of the business and affairs of BridgeBio Pharma LLC and its subsidiaries, through which we conduct our business. BridgeBio Pharma, Inc. will consolidate the financial results of its subsidiaries, including BridgeBio Pharma LLC and its subsidiaries. Pursuant to the LLC Agreement following the Reorganization, BridgeBio Pharma, Inc. will have the right to determine when distributions will be made to BridgeBio Pharma, Inc. and the amount of any such distributions.

In connection with the Reorganization, BridgeBio Pharma, Inc. will hold all property and assets of BridgeBio Pharma LLC and its consolidated subsidiaries and all of the debts and obligations of BridgeBio Pharma LLC and its consolidated subsidiaries will become the debts and obligations of BridgeBio Pharma, Inc. by operation of law. BridgeBio Pharma, Inc. will be governed by a certificate of incorporation filed with the Delaware Secretary of State and bylaws, the material portions of each of which are described under the heading "Description of Capital Stock."

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On the effective date of the Reorganization, the members of the board of managers of BridgeBio Pharma LLC will become the members of BridgeBio Pharma, Inc.'s board of directors and the officers of BridgeBio Pharma LLC will become the officers of BridgeBio Pharma, Inc.

The purpose of the Reorganization is to reorganize our corporate structure so that the entity that is offering common stock to the public in this offering is a corporation rather than a limited liability company and so that our existing investors will own our common stock rather than units in a limited liability company. References in this prospectus to our capitalization and other matters pertaining to our equity and shares prior to the Reorganization relate to the capitalization and equity and units of BridgeBio Pharma LLC, and after the Reorganization, to BridgeBio Pharma, Inc.

## CAPITALIZATION

The following table sets forth our cash and cash equivalents and our capitalization as of March 31, 2019:

- on an actual basis;
- on a pro forma basis to give effect to:
  - the completion of the Reorganization; and
  - the filing and effectiveness of our amended and restated certificate of incorporation; and
- on a pro forma as adjusted basis to give further effect to our issuance and sale of 15,000,000 shares of our common stock in this offering at an assumed initial public offering price of \$15.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

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The pro forma and pro forma as adjusted information below is illustrative only, and our capitalization following the completion of this offering will be adjusted based on the actual initial public offering price and other terms of this offering determined at pricing. You should read this table together with our combined and consolidated financial statements and the related notes appearing at the end of this prospectus and the sections of this prospectus titled “Reorganization,” “Selected Consolidated Financial Data” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations.”

	At March 31, 2019		
	Actual	Pro Forma	Pro Forma As Adjusted <sup>(1)</sup>
	(in thousands, except share, unit, per share and per unit data)		
Cash and cash equivalents	\$ 373,027	\$ 373,027	\$ 577,661
Total long-term debt obligations	\$ 54,861	\$ 54,861	\$ 54,861
Redeemable convertible preferred units (Series A, Series B, Series C and Series D); no par value; 407,955,726 units authorized, issued and outstanding, actual; no units authorized, issued or outstanding, pro forma; no units authorized, issued or outstanding, pro forma as adjusted	\$ 479,044	\$ —	\$ —
Redeemable founder units; no par value; 11,420,741 units authorized, issued and outstanding, actual; no units authorized, issued or outstanding, pro forma; no units authorized, issued or outstanding, pro forma as adjusted	1,754	—	—
Redeemable common units; no par value; 9,098,522 units authorized; 7,533,210 units issued and outstanding, actual; no units authorized, issued or outstanding, pro forma; no units authorized issued or outstanding, pro forma as adjusted	1,645	—	—
Management incentive units; no par value; 72,806,666 units authorized; 21,948,799 units issued and outstanding, actual; no units authorized, issued or outstanding, pro forma; no units authorized, issued and outstanding, pro forma as adjusted	4,431	—	—
Redeemable convertible noncontrolling interests	202	202	202
Members’ equity (deficit), actual; Stockholders’ equity, pro forma and pro forma as adjusted:			
Undesignated preferred stock; \$0.001 par value; no shares authorized, issued or outstanding, actual; 25,000,000 shares authorized, no shares issued and outstanding, pro forma; 25,000,000 shares authorized, no shares issued and outstanding, pro forma as adjusted	—	—	—
Common stock; \$0.001 par value; no shares authorized, issued and outstanding, actual; 500,000,000 shares authorized, 99,999,967 shares issued and outstanding, pro forma; 500,000,000 shares authorized, 114,999,967 shares issued and outstanding, pro forma as adjusted	—	100	115
Additional paid-in capital	—	486,774	690,456
Accumulated deficit	(234,733)	(234,733)	(234,733)
Total BridgeBio Pharma LLC members’ deficit, actual; Total BridgeBio Pharma, Inc. equity, pro forma and pro forma as adjusted	(234,733)	252,141	455,838
Noncontrolling interests	58,318	58,318	58,318
Total members’ deficit, actual; Total stockholders’ equity, pro forma and pro forma as adjusted	(176,415)	310,459	514,156
Total capitalization	\$ 310,661	\$ 310,661	\$ 514,358

(1) A \$1.00 increase (decrease) in the assumed initial public offering price of \$15.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) the pro forma as adjusted amount of



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each of cash and cash equivalents, total stockholders' equity and total capitalization by \$14.0 million, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. An increase (decrease) of 1,000,000 shares in the number of shares offered by us, as set forth on the cover page of this prospectus, would increase (decrease) the pro forma as adjusted amount of each of cash and cash equivalents, total stockholders' equity and total capitalization by \$14.0 million, assuming no change in the assumed initial public offering price per share and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

The number of shares of our common stock to be outstanding after this offering assumes the Reorganization takes place prior to the completion of this offering and is based on (i) an assumed initial public offering price of \$15.00 per share, the midpoint of the price range set forth on the cover page of this prospectus, and (ii) 99,999,967 shares of our common stock (which includes 7,019,309 shares of restricted common stock) outstanding as of March 31, 2019, assuming the exchange of all outstanding units of BridgeBio Pharma LLC as of March 31, 2019 into shares of common stock of BridgeBio Pharma, Inc. in the Reorganization. See "Reorganization".

The table above does not include:

- 11,500,000 shares of our common stock reserved for issuance under our 2019 Stock Option and Incentive Plan, which will become effective in connection with this offering, as well as any future increases in the number of shares of our common stock reserved for issuance under our 2019 Stock Option and Incentive Plan; and
- 2,000,000 shares of our common stock reserved for issuance under our 2019 Employee Stock Purchase Plan, which will become effective in connection with this offering, as well as any future increases in the number of shares of our common stock reserved for issuance under our 2019 Employee Stock Purchase Plan.

## DILUTION

If you invest in our common stock in this offering, your ownership interest will be diluted immediately to the extent of the difference between the initial public offering price per share of our common stock and the pro forma as adjusted net tangible book value per share of our common stock immediately after this offering.

Our historical net tangible book value (deficit) and historical net tangible book value (deficit) per share have not been presented as there were no common shares outstanding as of March 31, 2019.

After giving effect to the Reorganization, our pro forma net tangible book value as of March 31, 2019 was \$309.5 million, or \$3.10 per share of our common stock. Pro forma net tangible book value represents the amount of our total tangible assets less our total liabilities, after giving effect to the Reorganization. Pro forma net tangible book value per share represents pro forma net tangible book value divided by the total number of shares outstanding as of March 31, 2019 after giving effect to the pro forma adjustments described above.

After giving further effect to our issuance and sale of 15,000,000 shares of our common stock in this offering at an assumed initial public offering price of \$15.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us, our pro forma as adjusted net tangible book value as of March 31, 2019 would have been \$514.2 million, or \$4.47 per share. This represents an immediate increase in pro forma as adjusted net tangible book value per share of \$1.37 to existing stockholders and immediate dilution of \$10.53 in pro forma as adjusted net tangible book value per share to new investors purchasing common stock in this offering. Dilution per share to new investors is determined by subtracting pro forma as adjusted net tangible book value per share after this offering from the assumed initial public offering price per share paid by new investors. The following table illustrates this dilution on a per share basis:

Assumed initial public offering price per share	\$15.00
Pro forma net tangible book value per share as of March 31, 2019	\$3.10
Increase in pro forma as adjusted net tangible book value per share attributable to new investors purchasing common stock in this offering	<u>1.37</u>
Pro forma as adjusted net tangible book value per share after this offering	<u>4.47</u>
Dilution per share to new investors purchasing common stock in this offering	<u>\$10.53</u>

A \$1.00 increase (decrease) in the assumed initial public offering price of \$15.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) our pro forma as adjusted net tangible book value per share after this offering by \$0.12 and dilution per share to new investors purchasing common stock in this offering by \$0.88, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. An increase of 1,000,000 shares in the number of shares offered by us, as set forth on the cover page of this prospectus, would increase our pro forma as adjusted net tangible book value per share after this offering by \$0.08 and decrease the dilution per share to new investors purchasing common stock in this offering by \$0.08, assuming no change in the assumed initial public offering price per share and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. A decrease of 1,000,000 shares in the number of shares offered by us, as set forth on the cover page of this prospectus, would decrease our pro forma as adjusted net tangible book value per share after this offering by \$0.08 and increase the dilution per share to new investors purchasing common stock in this offering by \$0.08, assuming no change in the assumed initial public offering price per share and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

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If the underwriters fully exercise their option to purchase 2,250,000 additional shares of common stock in this offering, our pro forma as adjusted net tangible book value per share after this offering would be \$4.65 and the dilution in pro forma as adjusted net tangible book value per share to new investors purchasing common stock in this offering would be \$10.35, assuming no change in the assumed initial public offering price per share and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

The following table summarizes, as of March 31, 2019, on the pro forma as adjusted basis described above, the total number of shares of common stock purchased from us, the total consideration paid or to be paid, and the average price per share paid or to be paid by existing stockholders and by new investors in this offering at an assumed initial public offering price of \$15.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, before deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. As the table shows, new investors purchasing common stock in this offering will pay an average price per share substantially higher than our existing stockholders paid.

	<u>Shares Purchased</u>		<u>Total Consideration</u>		<u>Average Price Per Share</u>
	<u>Number</u>	<u>Percent</u>	<u>Amount</u>	<u>Percent</u>	
Existing stockholders	99,999,967	87.0%	\$482,089,778	68.2%	\$ 4.82
New investors	15,000,000	13.0	225,000,000	31.8	\$15.00
Total	<u>114,999,967</u>	<u>100%</u>	<u>\$707,089,778</u>	<u>100%</u>	

A \$1.00 increase (decrease) in the assumed initial public offering price of \$15.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) the total consideration paid by new investors by \$15.0 million and, in the case of an increase, would increase the percentage of total consideration paid by new investors by 1.4 percentage points and, in the case of a decrease, would decrease the percentage of total consideration paid by new investors by 1.5 percentage points, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same. An increase (decrease) of 1,000,000 shares in the number of shares offered by us, as set forth on the cover page of this prospectus, would increase (decrease) the total consideration paid by new investors by \$15.0 million and, in the case of an increase, would increase the percentage of total consideration paid by new investors by 1.4 percentage points and, in the case of a decrease, would decrease the percentage of total consideration paid by new investors by 1.5 percentage points, assuming no change in the assumed initial public offering price per share.

The table above assumes no exercise of the underwriters' option to purchase additional shares in this offering. If the underwriters' option to purchase additional shares is fully exercised, the number of shares of our common stock held by existing stockholders would be reduced to 85.3% of the total number of shares of our common stock outstanding after this offering, and the number of shares of common stock held by new investors purchasing common stock in this offering would be increased to 14.7% of the total number of shares of our common stock outstanding after this offering.

See "Pricing Sensitivity Analysis" for how some of the information presented above would be affected by an initial public offering price per share of common stock at the low-, mid- and high-points of the estimated price range set forth on the cover of this prospectus or if the underwriters' option to purchase additional shares of common stock is exercised in full.

The table above is based on no shares of common stock outstanding as of March 31, 2019 and gives effect to the Reorganization.

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The table above does not include:

- 11,500,000 shares of our common stock reserved for issuance under our 2019 Stock Option and Incentive Plan, which will become effective in connection with this offering, as well as any future increases in the number of shares of our common stock reserved for issuance under our 2019 Stock Option and Incentive Plan; and
- 2,000,000 shares of our common stock reserved for issuance under our 2019 Employee Stock Purchase Plan, which will become effective in connection with this offering, as well as any future increases in the number of shares of our common stock reserved for issuance under our 2019 Employee Stock Purchase Plan.

If common stock options or share appreciation rights are issued under our equity incentive plans, or if we issue additional shares of common stock in the future, there will be further dilution to investors purchasing common stock in this offering.

The number of shares of common stock of BridgeBio Pharma, Inc. that holders of units of BridgeBio Pharma LLC will receive in the Reorganization will vary depending on the initial public offering price. See “Pricing Sensitivity Analysis” for additional information.

## SELECTED CONSOLIDATED FINANCIAL DATA

The following information is presented for BridgeBio Pharma LLC, which will become a wholly owned subsidiary of BridgeBio Pharma, Inc., the entity whose shares are being offered hereby. You should read the following selected consolidated financial data together with our combined and consolidated financial statements and the related notes appearing at the end of this prospectus and the “Management’s Discussion and Analysis of Financial Condition and Results of Operations” section of this prospectus. We have derived the combined and consolidated statements of operations and comprehensive loss data for the years ended December 31, 2017 and 2018 and our combined and consolidated balance sheet data for the years ended December 31, 2017 and 2018 from our audited combined and consolidated financial statements appearing at the end of this prospectus. We derived the consolidated statements of operations and comprehensive loss data for the three months ended March 31, 2018 and 2019 and the consolidated balance sheet data as of March 31, 2019 from our interim condensed consolidated financial statements appearing at the end of this prospectus. Our interim condensed consolidated financial statements were prepared on the same basis as our audited combined and consolidated financial statements and, in our opinion, reflect all adjustments, consisting only of normal recurring adjustments, that are necessary for the fair statement of our interim condensed consolidated financial statements. Our historical results are not necessarily indicative of the results that may be expected in the future.

	Year Ended December 31,		Three Months Ended March 31,	
	2017	2018	2018	2019
	(in thousands, except share, unit, per share and per unit data)			
<b>Combined and Consolidated Statements of Operations and Comprehensive Loss</b>				
<b>Data:</b>				
Operating expenses:				
Research and development	\$ 30,556	\$ 140,073	\$ 33,831	\$ 44,853
General and administrative	13,302	43,587	8,007	18,899
Total operating expenses	43,858	183,660	41,838	63,752
Loss from operations	(43,858)	(183,660)	(41,838)	(63,752)
Other income (expense), net:				
Interest income	39	2,004	1	2,107
Interest expense	(13)	(2,547)	(7)	(1,671)
Gain on deconsolidation of PellePharm	—	19,327	—	—
Loss from PellePharm	—	(275)	—	(4,599)
LEO call option expense	—	(3,009)	—	(1,514)
Other expense	—	(1,291)	(586)	(7)
Total other income (expense), net	26	14,209	(592)	(5,684)
Net loss and comprehensive loss	(43,832)	(169,451)	(42,430)	(69,436)
Net loss attributable to redeemable convertible noncontrolling interests and noncontrolling interests	13,267	38,702	8,274	8,251
Net loss and comprehensive loss attributable to BridgeBio	\$(30,565)	\$(130,749)	\$(34,156)	\$(61,185)
Cumulative returns on redeemable convertible preferred units (Series A, Series B and Series C)				
	(5,672)	(13,287)	(3,121)	—
Net loss attributable to redeemable founder units and redeemable common units	\$(36,237)	\$(144,036)	\$(37,277)	\$(61,185)

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	Year Ended December 31,		Three Months Ended March 31,	
	2017	2018	2018	2019
	(in thousands, except share, unit, per share and per unit data)			
Net loss per unit attributable to redeemable founder unitholders and redeemable common unitholders, basic and diluted	\$ (2.18)	\$ (8.01)	\$ (2.13)	\$ (3.25)
Total weighted-average redeemable founder units and redeemable common units used in computing net loss per unit, basic and diluted	16,650,073	17,991,781	17,485,526	18,827,234
Pro forma net loss per share, basic and diluted <sup>(1)</sup>		\$ (2.32)		\$ (0.66)
Pro forma weighted average common shares outstanding, basic and diluted <sup>(1)</sup>		62,087,997		92,741,876

(1) Immediately prior to the completion of this offering, we will complete a series of transactions pursuant to which BridgeBio Pharma LLC will become a wholly owned subsidiary of BridgeBio Pharma, Inc., a newly formed Delaware corporation. As part of the transactions, unitholders of BridgeBio Pharma LLC will exchange their units of BridgeBio Pharma LLC for shares of BridgeBio Pharma, Inc. These transactions are collectively referred to as the Reorganization. See the section of this prospectus titled "Reorganization."

	As of December 31,		As of March 31,
	2017	2018	2019
	(in thousands)		
<b>Combined and Consolidated Balance Sheet Data:</b>			
Cash and cash equivalents	\$ 91,995	\$ 436,086	\$ 373,027
Working capital <sup>(1)</sup>	88,581	412,646	347,001
Total assets	98,044	464,941	401,374
Total long-term debt obligations	—	54,507	54,861
Redeemable convertible preferred units	143,867	478,865	479,044
Redeemable founder units	1,754	1,754	1,754
Redeemable common units	1,431	1,619	1,645
Accumulated deficit	(61,427)	(170,580)	(234,733)
Noncontrolling interests	2,498	62,361	58,318
Total members' deficit	(58,929)	(108,219)	(176,415)

(1) We define working capital as current assets less current liabilities.

## MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

*You should read the following discussion and analysis of our financial condition and results of operations together with the section of this prospectus entitled "Selected Consolidated Financial Data" and our combined and consolidated financial statements and related notes included elsewhere in this prospectus. This discussion and other parts of this prospectus contain forward-looking statements that involve risks and uncertainties, such as statements of our plans, objectives, expectations, intentions and beliefs. Our actual results could differ materially from those discussed in these forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those identified below and those discussed in the section entitled "Risk Factors" included elsewhere in this prospectus.*

### Overview

We are a team of experienced drug discoverers, developers, and innovators working to create life-altering medicines that target well-characterized genetic diseases at their source. We founded BridgeBio in 2015 to identify and advance transformative medicines to treat patients who suffer from Mendelian diseases, which are diseases that arise from defects in a single gene, and cancers with clear genetic drivers. Our pipeline of over 15 development programs includes product candidates ranging from early discovery to late-stage development. Several of our programs target indications that we believe present the potential for our product candidate, if approved, to target portions of market opportunities of at least \$1.0 billion in annual sales. We have four product candidates in clinical trials that, if positive, we believe could support the filing of an application for marketing authorization. Two of these product candidates are in Phase 3 clinical trials, one is in a Phase 2/3 clinical trial, and one is in a Phase 2 clinical trial.

We focus on genetic diseases because they exist at the intersection of high unmet patient need and tractable biology. Our approach is to translate research pioneered at academic laboratories and leading medical institutions into products that we hope will ultimately reach patients. We are able to realize this opportunity through a confluence of scientific advances: (i) identification of the genetic underpinnings of disease as more cost-efficient genome and exome sequencing becomes available; (ii) progress in molecular biology; and (iii) the development and maturation of longitudinal data and retrospective studies that enable the linkage of genes to diseases. We believe that this early-stage innovation represents one of the greatest practical sources for new drug creation.

Since our inception in 2015, we have focused substantially all of our efforts and financial resources on acquiring and developing product and technology rights, building our intellectual property portfolio and conducting research and development activities for our product candidates within our wholly-owned subsidiaries and controlled entities, including partially-owned subsidiaries and subsidiaries we consolidate based on our deemed majority control of such entities as determined using either the variable interest entity, or VIE model, or the voting interest entity, or VOE model. To support these activities, we and our wholly-owned subsidiary, BridgeBio Services, Inc., (i) identify and secure new programs, (ii) set up new wholly-owned subsidiaries and controlled entities, (iii) recruit key management team members, (iv) raise and allocate capital across the portfolio and (v) provide certain shared services, including accounting and human resources, as well as workspaces. We do not have any products approved for sale and have not generated any revenue from product sales. To date, we have funded our operations with proceeds from the sale of our equity securities and, to a lesser extent, debt borrowings.

As of March 31, 2019, we had cash and cash equivalents of \$373.0 million. Since our inception, we have incurred significant operating losses. For the years ended December 31, 2017 and 2018, we incurred net losses of \$43.8 million and \$169.5 million, respectively. For the three months ended March 31, 2018 and 2019, we incurred net losses of \$42.4 million and \$69.4 million, respectively. We had an accumulated deficit as of March 31, 2019 of \$234.7 million. Our ability to generate product revenue sufficient to achieve profitability will depend heavily on the successful development and eventual commercialization of our product candidates at our

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wholly-owned subsidiaries and controlled entities. We expect to continue to incur significant expenses and increasing operating losses for at least the next several years.

### **Factors Affecting Comparability**

Our historical financial condition and results of operations for the periods presented may not be comparable, either between periods or going forward due to the factors described below.

#### *Eidos Therapeutics, Inc. 2018 Financing Transactions and IPO:*

In February 2018, we entered into a note and warrant purchase agreement with Eidos pursuant to which Eidos issued a convertible promissory note, or the Eidos Note, with the principal amount of \$10.0 million and a warrant to purchase a number of shares of preferred stock equal to \$4.0 million at the price paid by investors in the next equity financing, or the Eidos Warrant. In March 2018, we transferred 10% or \$1.0 million of our interest in the Eidos Note and the Eidos Warrant to a minority stockholder of Eidos. In March 2018, the Eidos Note was redeemed into shares of Series B redeemable convertible preferred stock of Eidos at a 30% discount to the price paid by other investors. In conjunction with these transactions, Eidos recognized a preferred stock warrant liability, tranche liability and an embedded derivative, which were recorded at fair value at inception and remeasured to fair value at each subsequent reporting date until the instruments were settled. In 2018, we recorded \$1.3 million in other income (expense) in the combined and consolidated statements of operations related to these 2018 Eidos financing transactions. All of these Eidos financial instruments were settled during 2018.

In June 2018, Eidos completed its initial public offering, or the Eidos IPO. All redeemable convertible preferred stock of Eidos was converted into common stock at the closing of the Eidos IPO. As part of the Eidos IPO, we purchased common stock of \$17.0 million. The Eidos Warrant was also not exercised upon the completion of the Eidos IPO. We previously determined that Eidos was a controlled VIE as of December 31, 2017 and through its initial public offering in June 2018, at which time we determined that Eidos is no longer a VIE. Subsequent to the Eidos IPO and through March 31, 2019, we held a majority voting interest in Eidos and consolidate Eidos under the VOE model.

#### *PellePharm, Inc. Transactions:*

PellePharm entered into a series of agreements, or the LEO Agreement, with LEO Pharma A/S, or LEO, in November 2018. As part of the LEO Agreement, we granted LEO an exclusive, irrevocable option, or the LEO Call Option, to acquire all of PellePharm's shares held by us. The LEO Call Option is exercisable by LEO on or before the occurrence of certain events relating to PellePharm's clinical development programs and no later than July 30, 2021. We account for the LEO Call Option as a current liability in our combined and consolidated financial statements because we are obligated to sell our shares in PellePharm to LEO at a pre-determined price, if the option is exercised. The fair value of the LEO Call Option on issuance was \$1.9 million. We will remeasure the LEO Call Option to fair value at each subsequent combined and consolidated balance sheet date until the LEO Call Option is either exercised or expires. We previously determined that we were the primary beneficiary of PellePharm, as of December 31, 2017 and through the date of execution of the LEO Agreement in November 2018. At the time of execution, we concluded that we are no longer the primary beneficiary of, and thus deconsolidated, PellePharm. Subsequent to the LEO Agreement, we account for our retained investment in PellePharm under the equity method and cost method.

### **Basis of Presentation and Consolidation**

Prior to June 2017, we consisted of two separate legal entities, BridgeBio LLC and BridgeBio Pharma LLC. Historically, our members have provided funding to both entities. In June 2017, to consolidate the investments made in both entities, BridgeBio LLC and BridgeBio Pharma LLC merged through a transaction under common



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control, or the Merger. As part of the Merger, BridgeBio LLC's redeemable convertible Series A preferred units, redeemable convertible Series B preferred units, redeemable founder units and redeemable common units were cancelled and holders of such units were issued the same number of BridgeBio Pharma LLC's redeemable convertible Series A preferred units, redeemable convertible Series B preferred units, redeemable founder units and redeemable common units. As a result of the Merger, we recorded a capital transaction of \$4.5 million to accumulated deficit in June 2017, which represents the difference between the carrying amounts of the cancelled and newly issued units. As a result of the Merger, there was no gain or loss recognized at BridgeBio LLC for tax purposes.

Except as otherwise indicated or the context otherwise requires, all information included herein is presented giving effect to the Merger.

Since our inception, we have created wholly-owned subsidiaries or made investments in certain controlled entities, including partially-owned subsidiaries for which we have majority voting interest under the VOE model or for which we are the primary beneficiary under the VIE model, which we refer to collectively as our consolidated entities. Ownership interests in entities over which we have significant influence, but not a controlling financial interest, are accounted for as cost and equity method investments. Ownership interests in consolidated entities that are held by entities other than us are reported as redeemable convertible noncontrolling interests and noncontrolling interests in our combined and consolidated balance sheets. Losses attributed to redeemable convertible noncontrolling interests and noncontrolling interests are reported separately in our combined and consolidated statements of operations and comprehensive loss.

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We have either created or made investments in the following entities, which are consolidated in our combined and consolidated financial statements, with the exception of PellePharm as discussed above:

Consolidated Entities	Relationship as of March 31, 2019	Date control first acquired	Ownership % as of January 1, 2017	Ownership % as of December 31, 2017	Ownership % as of December 31, 2018	Ownership % as of March 31, 2019
TheRas, Inc.	Wholly-owned subsidiary	August 2016	100%	100%	100%	100%
BridgeBio Services, Inc.	Wholly-owned subsidiary	April 2017	—	100%	100%	100%
Origin Biosciences, Inc.	Wholly-owned subsidiary	April 2018	—	—	100%	100%
Fortify Therapeutics, Inc.	Wholly-owned subsidiary	June 2018	—	—	100%	100%
Sub20, Inc.	Wholly-owned subsidiary	June 2018	—	—	100%	100%
Unnamed Entity	Wholly-owned subsidiary	December 2018	—	—	100%	100%
Eidos Therapeutics, Inc.(1)	Partially-owned subsidiary	April 2016	53.8%	79.9%	62.5%	62.3%
Molecular Skin Therapeutics, Inc.	Controlled VIE	July 2016	80%	56.5%	61.7%	59.9%
Quartz Therapeutics, Inc.	Controlled VIE	October 2016	100%	89.0%	89.0%	89.0%
PellePharm, Inc.(2)	VIE	December 2016	52.6%	54.7%	43.3%	43.3%
Navire Pharma, Inc.	Controlled VIE	February 2017	—	80.0%	78.8%	79.0%
CoA Therapeutics, Inc.	Controlled VIE	February 2017	—	100%	99.5%	99.6%
Dermecular Therapeutics, Inc.	Controlled VIE	April 2017	—	86.0%	87.6%	87.6%
Phoenix Tissue Repair, Inc.	Controlled VIE	July 2017	—	23.0%	56.7%	56.6%
QED Therapeutics, Inc.	Controlled VIE	January 2018	—	—	94.4%	95.7%
Adrenas Therapeutics, Inc.	Controlled VIE	January 2018	—	—	90.1%	89.5%
Orfan Biotech, Inc.	Controlled VIE	January 2018	—	—	85.1%	87.5%
Ferro Therapeutics, Inc.	Controlled VIE	March 2018	—	—	89.4%	88.6%
Venthera, Inc.	Controlled VIE	April 2018	—	—	82.0%	82.0%
Aspa Therapeutics, Inc.	Controlled VIE	June 2018	—	—	92.5%	93.8%

(1) We previously determined that Eidos was a controlled VIE as of December 31, 2017 and through its initial public offering in June 2018, at which time we determined that Eidos was no longer a VIE. Subsequent to the Eidos IPO and through March 31, 2019, we have held a majority voting interest in Eidos and consolidate Eidos under the VOE model.

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- (2) We previously determined that we were the primary beneficiary of PellePharm through the date of execution of the Leo Agreement in November 2018, at which time we determined that we are no longer the primary beneficiary and deconsolidated PellePharm. The ownership percentage as of December 31, 2018 of 43.3% in the above table has been revised to correct the previously reported percentage of 52.1%.

### **Financial Operations Overview**

#### ***Revenue***

To date, we have not generated any revenue from product sales and do not expect to generate any revenue from the sale of products in the foreseeable future. If our development efforts for our product candidates are successful and result in regulatory approval, we may generate revenue in the future from product sales. We cannot predict if, when, or to what extent we will generate revenue from the commercialization and sale of our product candidates. We may never succeed in obtaining regulatory approval for any of our product candidates.

#### ***Operating Expenses***

##### ***Research and Development Expenses***

Research and development expenses consist primarily of costs incurred for our research activities, including our drug discovery efforts, and the development of our product candidates, which include:

- employee-related expenses, including salaries, related benefits, equity-based compensation and travel expenses for employees engaged in research and development functions;
- expenses incurred in connection with the preclinical and clinical development of our product candidates, including under agreements with contract research organizations, or CROs;
- the cost of consultants and contract manufacturing organizations, or CMOs, that manufacture drug products for use in our preclinical studies and clinical trials;
- facilities, depreciation and amortization, insurance and other direct and allocated expenses incurred as a result of research and development activities; and
- payments made under third-party licensing and asset acquisition agreements.

We expense research and development costs as incurred. Nonrefundable advance payments that we make for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses. The prepaid amounts are expensed as the related goods are delivered or the services are performed.

Our direct research and development costs are tracked on a program-by-program basis and consist primarily of external costs, such as fees paid to consultants, contractors, CMOs and CROs in connection with our preclinical and clinical development activities. License fees and other costs incurred after a product candidate has been designated and that are directly related to the product candidate are included in direct research and development expenses for that program. License fees and other costs incurred prior to designating a product candidate are included in early stage research programs.

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The following table summarizes our research and development expenses by program incurred for the following periods:

	Year ended December 31,		Three months ended March 31,	
	2017	2018	2018	2019
	(in thousands)		(in thousands)	
BBP-265 (Eidos)	\$ 9,286	\$ 28,539	\$ 5,651	\$ 8,588
BBP-831 (QED)	444	42,726	18,364	14,727
BBP-631 (Adrenas)	446	8,848	467	3,691
BBP-454 (TheRas)	997	3,663	570	995
BBP-009 (PellePharm)(1)	10,995	17,975	3,649	—
Other Programs	8,388	38,322	5,130	16,852
Total	<u>\$30,556</u>	<u>\$140,073</u>	<u>\$33,831</u>	<u>\$44,853</u>

(1) Results for PellePharm for 2018 are through the deconsolidation date in November 2018.

We have separately provided additional detail for the research and development expenses incurred in connection with the research and development activities conducted for the product candidates being developed by Eidos, QED, Adrenas, and TheRas, certain of our consolidated entities, as we believe they represent key portfolio value drivers. We have provided additional detail for BBP-009 (PellePharm) as it is the first of our product candidates for which a third party has provided research and development funding and secured an option to acquire. Subsequent to the LEO Agreement through which LEO has obtained the irrevocable option to acquire PellePharm, PellePharm is accounted for as an equity method and cost method investment and we record our percentage of the net income/loss associated with our percentage of PellePharm ownership under the equity method. Expenses for other programs in the table above represent the research and development expenses incurred by us in connection with research on our programs conducted by all of our other consolidated entities.

We are heavily dependent on the success of our product candidates, many of which are in preclinical or the early stages of clinical development, which is a lengthy and expensive process with uncertain outcomes and the potential for substantial delays. We cannot give any assurance that any of our product candidates will receive regulatory approval, which is necessary before they can be commercialized.

Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We expect that our research and development expenses will increase substantially in connection with our planned clinical and preclinical development activities in the near term and in the future. In particular, we expect to incur significant near-term research and development expenses in connection with our ongoing Phase 3 clinical trial of BBP-265 in ATTR-CM, our planned Phase 3 clinical trial of BBP-265 in ATTR-PN and our planned Phase 3 clinical trials for BBP-831 in adjuvant urothelial carcinoma.

### **General and Administrative Expenses**

Our general and administrative costs consist primarily of employee-related costs, travel expenses, expenses for outside professional services, including legal, human resource, audit, accounting and tax services, and allocated facilities-related costs. Employee-related costs include salaries, related benefits and equity-based compensation expense. We expect to incur additional expenses as a result of this offering and operating as a public company, including expenses related to compliance with the rules and regulations of the SEC and listing standards applicable to companies listed on a national securities exchange, additional insurance expenses, investor relations activities and other administrative and professional services. We also expect to increase the size of our administrative, finance and legal functions to support the anticipated growth of our business.

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### ***Other Income (Expense), Net***

#### *Interest Income*

Interest income consists of interest income earned on our cash and cash equivalents.

#### *Interest Expense*

Interest expense consists primarily of interest expense incurred under our term loans with Hercules Capital, Inc., or Hercules.

#### *Gain on Deconsolidation of PellePharm*

After execution of the LEO Agreement, we concluded that PellePharm should be deconsolidated and we recorded our retained interest in PellePharm at fair value, resulting in a gain being recognized during the year ended December 31, 2018.

#### *Loss from PellePharm*

We recognize our share of losses from the PellePharm equity method investment as incurred. After our equity method investment was reduced to zero during the period ended March 31, 2019, we recognize our percentage of net losses consistent with our preferred stock ownership percentage.

#### *LEO Call Option Expense*

We account for the LEO Call Option as a current liability as we have the obligation to sell our PellePharm shares to LEO at a pre-determined price if the LEO Call Option is exercised. The LEO Call Option can be exercised at any time through the maturity date. The LEO Call Option was recorded at fair value on the date the option agreement was entered into with LEO in November 2018. The LEO Call Option is subject to remeasurement to fair value at each combined and consolidated balance sheet date until the LEO Call Option is either exercised or expires.

#### *Other Expense*

Other expense consists primarily of the change in fair value of the Eidos financial instruments issued and settled in 2018 and other miscellaneous expenses unrelated to our core operations.

### ***Income Taxes***

BridgeBio Pharma LLC is a “pass-through” entity under the Internal Revenue Code of 1986, as amended, or the Code, and the members are taxed directly on their respective ownership interests in the combined and consolidated income. Therefore, no provision or liability for federal income tax has been included in our combined and consolidated financial statements. For our consolidated entities, income taxes are accounted for under the asset and liability method. Under this method, deferred income tax assets and liabilities are determined based upon the difference between the consolidated financial statement carrying amounts and the tax basis of assets and liabilities and are measured using the enacted tax rate expected to apply to taxable income in the years in which the differences are expected to be reversed.

As of December 31, 2018, we had net operating losses of approximately \$157.5 million and \$101.8 million for federal and state income tax purposes, respectively, available to reduce future taxable income, if any. The net operating losses will begin to expire in 2033.

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As of December 31, 2018, we had federal research and development credit carryforwards of \$4.2 million, which will expire beginning in 2033 if not utilized. As of December 31, 2018, we had California research and development credit carryforwards of \$0.8 million. The California research and development tax credits have no expiration date.

A valuation allowance is provided for deferred tax assets where the recoverability of the assets is uncertain. The determination to provide a valuation allowance is dependent upon the assessment of whether it is more likely than not that sufficient future taxable income will be generated to utilize the deferred tax assets. Based on the weight of the available evidence, which includes our consolidated entities' historical operating losses and forecast of future losses, we have provided a full valuation allowance against the deferred tax assets resulting from the tax loss and credits carried forward.

Utilization of the net operating loss and credit carryforwards may be subject to a substantial annual limitation due to an ownership change limitation as provided by section 382 of the Code, and similar state provisions. The annual limitation may result in the expiration of net operating losses and credits before utilization. In the event that we have a change of ownership, utilization of the net operating loss and tax credit carryforwards may be restricted.

### ***Net Loss Attributable to Redeemable Convertible Noncontrolling Interests and Noncontrolling Interests***

Net loss attributable to noncontrolling interests in our combined and consolidated statements of operations is a result of our investments in our consolidated entities, which include PellePharm, Inc. (through November 2018), Eidos Therapeutics, Inc., QED Therapeutics, Inc., Adrenas Therapeutics, Inc., Orfan Biotech, Inc., Venthera, Inc., Aspa Therapeutics, Inc., Phoenix Tissue Repair, Inc., Quartz Therapeutics, Inc., Navire Pharma, Inc., Ferro Therapeutics, Inc., Dermecular Therapeutics, Inc., Molecular Skin Therapeutics, Inc., CoA Therapeutics, Inc. and consists of the portion of the net loss of those consolidated entities that is not allocated to us. Changes in the amount of net loss attributable to noncontrolling interests are directly impacted by changes in the net loss of our consolidated entities and are the result of ownership percentage changes.

### **Critical Accounting Policies and Estimates**

Our management's discussion and analysis of our financial condition and results of operations is based on our combined and consolidated financial statements, which have been prepared in accordance with United States generally accepted accounting principles. The preparation of these combined and consolidated financial statements requires us to make estimates and assumptions that affect the reported amounts of assets, liabilities, and the disclosure of contingent assets and liabilities at the date of the combined and consolidated financial statements, as well as revenue and expenses incurred during the reporting periods. Our estimates are based on our historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in Note 2 to our combined and consolidated financial statements included elsewhere in this prospectus, we believe that the following accounting policies are those most critical to the judgments and estimates used in the preparation of our combined and consolidated financial statements.

### ***Variable Interest Entities ("VIE") and Voting Interest Entities ("VOE")***

We consolidate those entities in which we have a direct or indirect controlling financial interest based on either the VIE model or the VOE model.

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VIEs are entities that, by design, either: (i) lack sufficient equity to permit the entity to finance its activities without additional support from other parties; or (ii) have equity holders that do not have the ability to make significant decisions relating to the entity's operations through voting rights, or do not have the obligation to absorb the expected losses, or do not have the right to receive the residual returns of the entity.

The primary beneficiary of a VIE is required to consolidate the assets and liabilities of the VIE. The primary beneficiary is the party that has both the power to direct the activities of the VIE that most significantly impact the VIE's economic performance and the obligation to absorb losses or the right to receive benefits from the VIE that could potentially be significant to the VIE through its interest in the VIE.

To assess whether we have the power to direct the activities of a VIE that most significantly impact the VIE's economic performance, we consider all of the facts and circumstances, including our role in establishing the VIE and our ongoing rights and responsibilities. Our assessment includes identifying the activities that most significantly impact the VIE's economic performance and identifying which party, if any, has power over those activities. In general, the parties that make the most significant decisions affecting the VIE (management and representation on the board of directors) and have the right to unilaterally remove those decision-makers are deemed to have the power to direct the activities of a VIE.

To assess whether we have the obligation to absorb losses of the VIE or the right to receive benefits from the VIE that could potentially be significant to the VIE, we consider all of our economic interests, which primarily include equity investments in preferred and common stock and issuance of notes that are convertible into preferred stock, that are deemed to be variable interests in the VIE. This assessment requires us to apply judgment in determining whether these interests, in the aggregate, are considered potentially significant to the VIE. Factors considered in assessing the significance include: the design of the VIE, including its capitalization structure; subordination of interests; payment priority; relative share of interests held across various classes within the VIE's capital structure; and the reasons why the interests are held by us.

At the VIE inception, we determine whether we are the primary beneficiary and if the VIE should be consolidated based on the facts and circumstances. We then perform ongoing reassessments at each reporting period on whether changes in the facts and circumstances regarding our involvement with the VIE results in a change to our consolidation conclusion.

Entities that do not qualify as a VIE are assessed for consolidation under the VOE model. Under the VOE model, we consolidate an entity if we determine that we, directly or indirectly, have greater than 50% of the voting shares and that other equity holders in such entities do not have substantive voting, participating or liquidation rights.

### ***Research and Development Expenses***

Research and development costs are expensed as incurred. Research and development costs consist of salaries, benefits, and other personnel related costs, including equity-based compensation expense, laboratory supplies, preclinical studies, clinical trials and related clinical manufacturing costs, costs related to manufacturing preparations, fees paid to other entities to conduct certain research and development activities on our behalf, and allocated facility and other related costs. Non-refundable advance payments for goods or services that will be used or rendered for future research and development activities are deferred and capitalized as prepaid expenses until the related goods are delivered or services are performed.

### ***Accrued Research and Development Liabilities***

We record accrued liabilities for estimated costs of research and development activities conducted by third-party service providers, which include the conduct of preclinical studies and clinical trials, and contract manufacturing activities. We record the estimated costs of research and development activities based upon the estimated amount

of services provided but not yet invoiced, and include these costs in accrued research and development liabilities in the combined and consolidated balance sheet and within research and development expense in the combined and consolidated statements of operations. These costs are a significant component of our research and development expenses.

We accrue for these costs based on factors such as estimates of the amount of work completed through discussions with internal personnel and external service providers as to the progress or stage of completion of the services and in accordance with agreements established with our third-party service providers for such services. We make significant judgments and estimates in determining the accrued research and development liabilities balance in each reporting period. As actual costs become known, we adjust our accrued estimates. Although we do not expect our estimates to be materially different from amounts actually incurred, the status and timing of services performed, the number of patients enrolled in clinical trials and the rate of patient enrollment may vary from our estimates and could result in us reporting amounts that are too high or too low in any particular period. Our accrued expenses are dependent, in part, upon the receipt of timely and accurate reporting from clinical research organizations and other third-party service providers. We record advance payments to service providers as prepaid assets, which are expensed as the contracted services are performed. To date, there have been no material differences from our accrued costs to actual costs.

### ***Equity-Based Compensation***

Because there is no public market for our units as we are a private company, our board of managers has determined the fair value of management incentive units and common units by considering a number of objective and subjective factors, including having contemporaneous and retrospective valuations of our equity performed by a third-party valuation specialist, valuations of comparable peer public companies, sales of our redeemable convertible preferred units, operating and financial performance, the lack of liquidity of our units, and general and industry-specific economic outlook. The fair value of our management incentive unit and common unit will be determined by our board of managers until such time as our common units are listed on an established stock exchange. Equity-based compensation is measured at the grant date for all equity-based awards made to employees and non-employees based on the fair value of the awards and is recognized as an expense on a straight-line basis over the requisite service period, which is generally the vesting period. We have elected to recognize the actual forfeitures by reducing the equity-based compensation in the same period as the forfeitures occur. Equity-based compensation for awards made to non-employees was measured as per ASC 505-50 until we early adopted Accounting Standards Update, or ASU, *2018-07 Compensation-Stock Compensation (Topic 718)* on January 1, 2017. We remeasured our equity-classified non-employee awards for which a measurement date had not been established at their adoption-based fair-value based measurement (January 1, 2017), and determined there was no cumulative-effect adjustment to our opening accumulated deficit. Subsequent to the adoption of ASU 2018-07, we account for non-employee awards similar to employee awards.

We have granted management incentive units and common units to employees and non-employees. These awards generally have only a service condition and vest over a period of up to five years. The awards have accelerated vesting upon a fundamental transaction, which is defined as (i) a merger, recapitalization or other business combination, (ii) a sale, transfer, exclusive license or disposition of BridgeBio Pharma LLC or (iii) a final liquidation, dissolution, winding-up or termination of BridgeBio Pharma LLC. Our consolidated entities have granted stock options that are exercisable in the underlying entity's equity and have issued restricted stock awards in the underlying entity's equity to employees and non-employees. None of the awards issued by the consolidated entities are issued for BridgeBio Pharma LLC members' capital. These awards generally have only a service condition and generally vest over a period of up to four years.

We classify equity-based compensation in our combined and consolidated statements of operations and comprehensive loss in the same manner in which the award recipients' payroll costs are classified or in which the award recipients' service payments are classified.



### ***Income Taxes***

Since BridgeBio Pharma LLC is a “pass-through” entity under the Code, our members are taxed directly on their respective ownership interests in consolidated income, and, therefore, no provision or liability for federal income tax has been included in the accompanying combined and consolidated financial statements.

For our consolidated entities, income taxes are accounted for under the asset and liability method. Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax base and net operating loss and tax credit carryforwards. Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled. Deferred tax assets are reduced by a valuation allowance if it is more likely than not that some portion of all of the deferred tax asset will not be realized.

Our consolidated entities recognize uncertain income tax positions at the largest amount that is more likely than not to be sustained upon audit by the relevant taxing authority. Changes in recognition or measurement are reflected in the period in which judgment occurs. Our consolidated entities’ policy is to recognize interest and penalties related to the underpayment of income taxes as a component of the provision for income taxes. To date, there have been no interest or penalties recorded in relation to unrecognized tax benefits.

### **JOBS Act and Emerging Growth Company Status**

We are an emerging growth company, as defined in the JOBS Act. Under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards issued subsequent to the enactment of the JOBS Act until such time as those standards apply to private companies. Other exemptions and reduced reporting requirements under the JOBS Act for emerging growth companies include presentation of only two years audited financial statements in a registration statement for an initial public offering, an exemption from the requirement to provide an auditor’s report on internal controls over financial reporting pursuant to the Sarbanes-Oxley Act of 2002, an exemption from any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation, and less extensive disclosure about our executive compensation arrangements. We have elected to use the extended transition period for complying with new or revised accounting standards that have different effective dates for public and private companies until the earlier of the date that (i) we are no longer an emerging growth company or (ii) we affirmatively and irrevocably opt out of the extended transition period provided in the JOBS Act. As a result, our combined and consolidated financial statements may not be comparable to companies that comply with the new or revised accounting pronouncements as of public company effective dates.

As described in Note 2 to our combined and consolidated financial statements, we early adopted multiple accounting standards, as the JOBS Act does not preclude an emerging growth company from adopting a new or revised accounting standard earlier than the time that such standard applies to private companies. We expect to use the extended transition period for any other new or revised accounting standards during the period in which we remain an emerging growth company.

We will remain an emerging growth company until the earliest of (i) the last day of our first fiscal year in which we have total annual gross revenues of \$1.07 billion or more, (ii) the last day of the fiscal year following the fifth anniversary of completion of this offering, (iii) the date on which we have issued more than \$1.0 billion of non-convertible debt instruments during the previous three fiscal years, or (iv) the date on which we are deemed a “large accelerated filer” under the rules of the SEC with at least \$700.0 million of outstanding equity securities held by non-affiliates.

## Recent Accounting Pronouncements

See Note 2, “Summary of Significant Accounting Policies—Recently Adopted Accounting Pronouncements” to our combined and consolidated financial statements and to our interim condensed consolidated financial statements included elsewhere in this prospectus for more information.

## Results of Operations

### Three Months Ended March 31, 2018 and 2019

	Three months ended		Increase/ (Decrease)	% Change
	2018	March 31, 2019		
	(in thousands)			
Operating expenses:				
Research and development	\$ 33,831	\$ 44,853	\$ 11,022	33%
General and administrative	8,007	18,899	10,892	136%
Total operating expenses	<u>41,838</u>	<u>63,752</u>	<u>21,914</u>	<u>52%</u>
Loss from operations	(41,838)	(63,752)	(21,914)	52%
Other income (expense), net:				
Interest income	1	2,107	2,106	*
Interest expense	(7)	(1,671)	(1,664)	23,771%
Loss from PellePharm	—	(4,599)	(4,599)	*
LEO call option expense	—	(1,514)	(1,514)	*
Other expense	(586)	(7)	579	(99)%
Total other income (expense), net	<u>(592)</u>	<u>(5,684)</u>	<u>(5,092)</u>	<u>860%</u>
Net loss and comprehensive loss	(42,430)	(69,436)	(27,006)	64%
Net loss attributable to redeemable convertible noncontrolling interests and noncontrolling interests	8,274	8,251	(23)	*
Net loss and comprehensive loss attributable to us	<u>\$ (34,156)</u>	<u>\$ (61,185)</u>	<u>\$ (27,029)</u>	<u>79%</u>

\* Not meaningful

### Research and Development Expenses

Research and development expenses increased by \$11.0 million to \$44.9 million for the three months ended March 31, 2019, from \$33.8 million for the same period in 2018, largely attributable to the number of product candidates under development increasing and the initiation of more clinical trials as compared to the prior period. The increase was primarily comprised of a \$7.1 million increase in professional and consulting services to advance our product candidates, a \$5.7 million increase in clinical development costs for our product candidates, a \$5.3 million increase in salaries and employee-related benefits, a \$4.0 million increase in allocated facility and other expenses, a \$3.6 million increase in development and drug discovery efforts for our research programs, and a \$0.3 million increase in equity-based compensation expense resulting from equity-based awards granted to employees of consolidated entities. This increase was partially offset by a \$14.7 million decrease in license fees to acquire various technologies as a result of the QED Therapeutics, Inc. asset acquisition in January 2018.

### General and Administrative Expenses

General and administrative expenses increased by \$10.9 million to \$18.9 million for the three months ended March 31, 2019, from \$8.0 million for the same period in 2018, largely due to our operations expanding as we

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added more controlled entities and development programs and as we prepared to become a public company. The increase was primarily comprised of a \$5.3 million increase in professional and consulting services such as administrative, accounting, finance, human resources and information technology services, a \$3.4 million increase in salaries and employee-related benefits, a \$1.2 million increase in equity-based compensation expense resulting from management incentive units and common units granted by us and from equity-based awards granted to employees of consolidated entities, a \$0.7 million increase in allocated facility and other expenses, and a \$0.3 million increase in legal fees.

### ***Interest Income***

Interest income for the three months ended March 31, 2019, primarily consisted of the interest earned on our investments in cash equivalents during the period. Interest income was not material for the same period in 2018.

### ***Interest Expense***

Interest expense for the three months ended March 31, 2019, primarily consisted of the interest accrued on the \$55.0 million Hercules term loans drawn in June and December 2018. Interest expense was not material for the same period in 2018.

### ***LEO Call Option Expense***

The LEO Call Option liability was recorded at fair value upon execution of the LEO Agreement in November 2018 and remeasured to fair value as of December 31, 2018 and March 31, 2019, resulting in our expense being recognized. There was no such liability subject to be recorded and remeasured as of March 31, 2018.

### ***Loss from PellePharm***

Due to the deconsolidation of PellePharm, we accounted for part of our remaining investment in PellePharm under the equity method and we recognized our share of PellePharm earnings or losses through March 31, 2019. PellePharm was a consolidated entity as of March 31, 2018 and as such no such gains or losses were recognized during the period.

### ***Other Expense***

Other expense for the three months ended March 31, 2018, consisted primarily of the change in fair value of the Eidos financial instruments issued and settled in 2018. Other expense was not material for the same period in 2019.

**Years Ended December 31, 2017 and 2018**

	<u>Year ended December 31,</u>		<u>Increase/ (Decrease)</u>	<u>% Change</u>
	<u>2017</u>	<u>2018</u>		
	(in thousands)			
<b>Operating expenses:</b>				
Research and development	\$ 30,556	\$ 140,073	\$ 109,517	358%
General and administrative	13,302	43,587	30,285	228%
Total operating expenses	<u>43,858</u>	<u>183,660</u>	<u>139,802</u>	<u>319%</u>
Loss from operations	(43,858)	(183,660)	(139,802)	319%
<b>Other income (expense), net:</b>				
Interest income	39	2,004	1,965	5,038%
Interest expense	(13)	(2,547)	(2,534)	19,492%
Gain on deconsolidation of PellePharm	—	19,327	19,327	*
Loss from PellePharm	—	(275)	(275)	*
LEO call option expense	—	(3,009)	(3,009)	*
Other expense	—	(1,291)	(1,291)	*
Total other income (expense), net	<u>26</u>	<u>14,209</u>	<u>14,183</u>	<u>54,550%</u>
Net loss and comprehensive loss	(43,832)	(169,451)	(125,619)	287%
Net loss attributable to redeemable convertible noncontrolling interests and noncontrolling interests	<u>13,267</u>	<u>38,702</u>	<u>25,435</u>	<u>192%</u>
Net loss and comprehensive loss attributable to us	<u><u>\$(30,565)</u></u>	<u><u>\$(130,749)</u></u>	<u><u>\$(100,184)</u></u>	<u><u>328%</u></u>

\* Not meaningful

**Research and Development Expenses**

Research and development expenses increased by \$109.5 million to \$140.1 million for the year ended December 31, 2018, from \$30.6 million for the same period in 2017, largely attributable to the number of product candidates under development increasing substantially from 2017 to 2018 and the initiation of more clinical trials as compared to 2017. The increase was primarily comprised of a \$25.0 million increase in professional and consulting services to advance our product candidates, a \$20.3 million increase in license fees to acquire various technologies, a \$19.2 million increase in clinical development costs for our product candidates, a \$18.4 million increase in development and drug discovery efforts for our research programs, a \$13.8 million increase in salaries and employee-related benefits, a \$11.6 million increase in allocated facility and other expenses and a \$1.0 million increase in equity-based compensation expense resulting from equity-based awards granted to employees of consolidated entities.

**General and Administrative Expenses**

General and administrative expenses increased by \$30.3 million to \$43.6 million for the year ended December 31, 2018, from \$13.3 million for the same period in 2017, largely due to our operations expanding as we added more controlled entities and development programs. The increase was primarily comprised of a \$11.8 million increase in professional and consulting services such as administrative, accounting, finance, human resources and information technology services, a \$6.1 million increase in allocated facility and other expenses, a \$5.7 million increase in salaries and employee-related benefits, a \$3.5 million increase in legal fees and a \$3.2 million increase in equity-based compensation expense resulting from management incentive units and common units granted by us and from equity-based awards granted to employees of consolidated entities.

**Interest Income**

Interest income for the year ended December 31, 2018, primarily consisted of the interest earned on our investments in cash equivalents in 2018. Interest income was not material in 2017.

### ***Interest Expense***

Interest expense for the year ended December 31, 2018, primarily consisted of the interest accrued on the \$55.0 million Hercules term loans drawn in June and December 2018. Interest expense was not material in 2017.

### ***Gain on Deconsolidation of PellePharm***

In November 2018, we deconsolidated PellePharm after execution of the LEO Agreement and recognized a gain of \$19.3 million as the difference between the fair value of our retained investment in PellePharm and our percentage of the PellePharm net deficit as of the deconsolidation date. There was no such gain recognized in 2017.

### ***LEO Call Option Expense***

The LEO Call Option liability was recorded at fair value upon execution of the LEO Agreement in November 2018. The LEO Call Option liability was remeasured to fair value as of December 31, 2018. There was no such liability subject to be recorded and remeasured in 2017.

### ***Loss from PellePharm***

Due to the deconsolidation of PellePharm, we accounted for part of our remaining investment in PellePharm under the equity method and we recognized our share of PellePharm earnings or losses from the deconsolidation date through December 31, 2018. PellePharm was a consolidated entity in 2017.

### ***Other Expense***

Other expense for the year ended December 31, 2018, consisted primarily of the change in fair value of the Eidos financial instruments issued and settled in 2018.

## **Liquidity and Capital Resources**

### ***Sources of Liquidity***

Since our inception, we have incurred significant operating losses. For the years ended December 31, 2017 and 2018, we incurred net losses of \$43.8 million and \$169.5 million, respectively. For the three months ended March 31, 2018 and 2019, we incurred net losses of \$42.4 million and \$69.4 million, respectively. We had an accumulated deficit as of March 31, 2019 of \$234.7 million. As of March 31, 2019, our outstanding debt was \$54.9 million, which is net of \$0.1 million of debt issuance costs and debt accretion. Our ability to generate product revenue sufficient to achieve profitability will depend heavily on the successful development and eventual commercialization of our product candidates at our consolidated entities. We expect to continue to incur significant expenses and increasing operating losses for at least the next several years.

In June 2018, our controlled subsidiary, Eidos, completed its U.S. initial public offering of its common stock of which net proceeds received were \$95.5 million. As of March 31, 2019, we held 22,589,300 shares of common stock of Eidos. All cash and cash equivalents held by Eidos are restricted and can be applied solely to fund the operations of Eidos.

We had cash and cash equivalents of \$373.0 million as of March 31, 2019, of which \$172.8 million was held at BridgeBio Pharma LLC. The remaining cash and cash equivalents were held by our wholly-owned subsidiaries and controlled entities, with these funds available for specific entity usage, except in limited circumstances.

### ***Secured Loans***

In June 2018, we executed a Loan and Security Agreement with Hercules Capital, Inc., or Hercules, under which we borrowed \$35.0 million, or Tranche I. The term of the loan was approximately 42 months, with a maturity

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date of January 1, 2022, or the Maturity Date. No principal payments were due during an interest-only period, commencing on the initial borrowing date and continuing through July 1, 2020, or the Amortization Date. The outstanding balance of the loan was to be repaid monthly beginning on the Amortization Date and extending through the Maturity Date. The term loan bears interest at a floating rate equal to the greater of: (i) the prime rate as reported in the Wall Street Journal plus 4.35% and (ii) 9.35%, payable monthly.

In December 2018, we executed the First Amendment to the Loan and Security Agreement, whereby we borrowed an additional \$20.0 million, or Tranche II, to increase the total principal balance outstanding to \$55.0 million. Upon draw of Tranche II, the interest-only period on the entire facility was extended until January 1, 2021, or the Amended Amortization Date. The outstanding balance of the original \$35.0 million Tranche I and the additional borrowing of \$20.0 million Tranche II is to be repaid monthly beginning on the Amended Amortization Date and extending through July 1, 2022, or the Amended Maturity Date. The additional \$20.0 million loan bears interest at a floating rate equal to the greater of: (i) the prime rate as reported in the Wall Street Journal plus 3.35% and (ii) 9.10%, payable monthly.

In May 2019, we executed the Second Amendment to the Loan and Security Agreement, whereby we borrowed an additional \$20.0 million, or Tranche III, to increase the total principal balance outstanding to \$75.0 million. The outstanding balance of Tranche I, Tranche II and Tranche III is to be repaid monthly beginning on the Amended Amortization Date and extending through the Amended Maturity Date. Tranche III bears interest at a floating rate equal to the greater of: (i) the prime rate as reported in the Wall Street Journal plus 3.10% and (ii) 9.10%, payable monthly.

On the earliest to occur of (i) the Amended Maturity Date, (ii) the date we prepay the outstanding principal amount of the Amended Hercules Term Loan, or (iii) the date the outstanding principal amount of the Amended Hercules Term Loan otherwise becomes due, we will owe Hercules an end of term charge equal to 6.35% of the \$35.0 million principal amount of the Tranche I term loan, or \$2.2 million, 5.75% of the \$20.0 million principal amount of the Tranche II term loan, or \$1.2 million, and 5.75% of the \$20.0 million principal amount of the Tranche III term loan, or \$1.2 million.

Effective the first fiscal quarter following the completion by us of an initial public offering, or IPO, of our shares on a United States-based national exchange with total net proceeds of no less than \$175.0 million, we shall receive: (i) a further six month interest-only extension to July 1, 2021, (ii) a further six month maturity extension to January 1, 2023, (iii) a reduction of 0.5% on the then effective interest rate on Tranches I and II, and (iv) the option to pay up to 1.5% of scheduled cash pay interest on the entire facility as payment in kind, or PIK Interest, with such cash pay interest paid as PIK Interest at a 1:1.2 ratio. All PIK Interest shall be capitalized and added to the outstanding principal balance under the Amended Hercules Term Loan, which shall then accrue further cash interest and fees pursuant to the terms of the Amended Hercules Term Loan.

The Amended Hercules Term Loan contains customary representations and warranties, events of default, and affirmative and negative covenants for a term loan facility of this size and type. However, Hercules has no covenants that limit or restrict the ability of a wholly-owned subsidiary or controlled entity that is predominantly involved in advancing our development programs to incur indebtedness. At the time of the close of the Amended Hercules Term Loan, there is no liquidity covenant on us. However, beginning on September 15, 2019, we may be required to maintain \$20.0 million in unrestricted cash, or the Minimum Cash Requirement, under certain circumstances. If we have not completed an IPO by September 15, 2019, we will be required to maintain the Minimum Cash Requirement with Hercules. In the event that an IPO occurs before September 15, 2019, we will be required to maintain the Minimum Cash Requirement, unless either of these conditions are achieved: i) our market capitalization is in excess of \$750 million, ii) Part A of Eidos' Phase III ATTRIBUTE trial produces positive data that would be supportive of an NDA filing, or iii) the acceptance of an NDA by the FDA related to any product filed by any of our wholly-owned subsidiaries or controlled entities. Hercules cannot limit or restrict our ability to dispose of assets, make investments, or make acquisitions. As pledged collateral for our obligations under the Amended Hercules Term Loan, we granted Hercules a security interest in all of our assets or personal property, including all equity interests owned or hereafter acquired by us. Further, at Hercules' sole discretion we

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must make a mandatory prepayment equal to 75% of net cash proceeds received from the sale or licensing of any pledged or collateral assets, including intellectual property, of a consolidated entity owned by us, or the repurchase or redemption of any pledged collateral by certain specified operating companies. None of our consolidated entities are a party to, nor provide any credit support or other security in connection with the Amended Hercules Term Loan.

### ***Liquidity Risks***

As of March 31, 2019, we had cash and cash equivalents of \$373.0 million. We believe that our currently available resources will enable us to fund our projected operating expenses and capital expenditures through at least the next 12 months.

We will not generate revenue from product sales unless and until we successfully complete clinical development and obtain regulatory approval for our product candidates. If we obtain regulatory approval for any of our product candidates and do not enter into a commercialization partnership, we expect to incur significant expenses related to developing our internal commercialization capability to support product sales, marketing and distribution. Further, upon the completion of this offering, we expect to incur additional costs associated with operating as a public company. As a result, we will need substantial additional funding to support our continuing operations and pursue our growth strategy. Until such time as we can generate significant revenue from product sales, if ever, we expect to finance our operations through a combination of equity offerings, debt financings, collaborations, strategic alliances and marketing, distribution or licensing arrangements. If we do raise additional capital through public or private equity offerings, the ownership interest of our existing stockholders, including investors in this offering, will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect our stockholders' rights. If we raise additional capital through debt financing, we may be subject to covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. Further, we may be unable to raise additional funds or enter into such other agreements or arrangements when needed on favorable terms, or at all. If we fail to raise capital or enter into such agreements as, and when, needed, we may have to significantly delay, scale back or discontinue the development and commercialization of one or more of our product candidates.

Because of the numerous risks and uncertainties associated with research, development and commercialization of pharmaceutical products, we are unable to estimate the exact amount of our operating capital requirements. Our future funding requirements will depend on many factors, including, but not limited to:

- the successful achievement of preclinical and clinical milestones;
- continuing our platform research and drug discovery and development efforts;
- conducting preclinical and clinical trials for our current product candidates and additional product candidates;
- establishing a sales, marketing and distribution infrastructure to commercialize any product candidates for which we may obtain regulatory approval;
- establishing and maintaining manufacturing and supply chain capacity sufficient to provide adequate supplies of our product candidates to support our ongoing and planned clinical trials and commercial quantities of any product candidates for which we may obtain marketing approval;
- maintaining, expanding and protecting our intellectual property portfolio;
- acquiring or in-licensing other product candidates and technologies;
- continuing to discover and develop additional product candidates; and
- hiring additional personnel to support our program development efforts to obtain regulatory approval and securing additional facilities for operations; operating as a public company upon completion of this offering.

Because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to accurately predict the timing or amount of increased expenses or when or if we will be able to achieve

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or maintain profitability. Even if we are able to generate product sales, we may not become profitable. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce or terminate our operations.

### **Cash Flows**

The following table summarizes our cash flows during the periods indicated:

	<b>Year ended December 31,</b>		<b>Three months ended</b>	
	<b>2017</b>	<b>2018</b>	<b>March 31,</b>	
	<b>(in thousands)</b>		<b>(in thousands)</b>	
Net cash used in operating activities	\$ (40,488)	\$ (136,643)	\$ (17,951)	\$ (59,140)
Net cash used in investing activities	(464)	(21,036)	(15,177)	(2,427)
Net cash provided by (used in) financing activities	112,983	501,548	15,693	(1,651)
Net increase (decrease) in cash and cash equivalents and restricted cash	<u>\$ 72,031</u>	<u>\$ 343,869</u>	<u>\$ (17,435)</u>	<u>\$ (63,218)</u>

#### *Net Cash Flows from Operating Activities*

Net cash used in operating activities for the three months ended March 31, 2019 was \$59.1 million and primarily consisted of our net loss of \$69.4 million and changes in net operating assets and liabilities of \$0.7 million, which were partially offset by non-cash charges of \$11.0 million. The net change in operating assets and liabilities were primarily due to a decrease of \$2.0 million in accounts payable due to timing of payments to vendors, an increase of \$1.9 million in other assets due to deferred offering costs, a decrease of \$0.4 million in accrued compensation and benefits and an increase of \$0.3 million in prepaid expenses and other current assets, which were partially offset by an increase of \$2.2 million in other accrued liabilities due to increased consulting accruals and increase of \$1.7 million in accrued research and development liabilities due to increased activities at CROs and CMOs. Our non-cash charges primarily consisted of a \$4.6 million loss from PellePharm, \$2.2 million for equity-based compensation expense, \$2.0 million for acquired in-process research and development assets and \$1.5 million of expense related to the revaluation of the LEO Call Option liability.

Net cash used in operating activities for the three months ended March 31, 2018 was \$18.0 million and primarily consisted of our net loss of \$42.4 million, which was partially offset by non-cash charges of \$18.3 million and a change in net operating assets and liabilities of \$6.1 million. Our non-cash charges primarily consisted of \$16.9 million for acquired in-process research and development assets due to asset acquisition by QED in January 2018, \$0.8 million in equity-based compensation expense and \$0.4 million from change in fair value of Eidos financial instruments. The net change in operating assets and liabilities was primarily due to an increase of \$4.3 million in accounts payable and increase of \$3.1 million in accrued research and development liabilities, both due to an increase in the level of research and development expenses and timing of receipt of invoices from and payments to vendors. These amounts were partially offset by an increase of \$1.7 million in prepaid expenses and other current assets primarily due to increase in prepaid research and development costs at QED and an increase of \$1.0 million in other assets.

Net cash used in operating activities for the year ended December 31, 2018 was \$136.6 million and primarily consisted of our net loss of \$169.5 million, which was partially offset by non-cash charges of \$10.3 million and a change in net operating assets and liabilities of \$22.5 million. Our non-cash charges primarily consisted of \$17.9 million for acquired in-process research and development assets, \$6.1 million for equity-based compensation expense, and \$3.0 million of expense related to the LEO Call Option. These changes were partially offset by a \$19.3 million gain on the deconsolidation of PellePharm. The net change in operating assets and liabilities was primarily due to an increase of \$16.7 million in accounts payable due to an increase in the level of research and development expenses and timing of payments to vendors, an increase of \$5.8 million in accrued research and development liabilities, an increase of \$3.4 million in accrued compensation and benefits due to



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higher headcount, an increase of \$3.4 million in other accrued liabilities and an increase of \$0.2 million in other liabilities. These changes were partially offset by an increase of \$6.1 million in prepaid expenses due to the timing of prepaid clinical and research related expenses.

Net cash used in operating activities for the year ended December 31, 2017 was \$40.5 million and primarily consisted of our net loss of \$43.8 million, which was partially offset by non-cash charges of \$2.1 million and a change in net operating assets and liabilities of \$1.2 million. Our net non-cash charges primarily consisted of \$1.8 million for equity-based compensation expense and \$0.3 million for depreciation and amortization. The net change in operating assets and liabilities was primarily due to an increase of \$1.6 million in accounts payable, an increase of \$2.6 million in accrued research and development liabilities, an increase of \$1.1 million in accrued compensation and benefits, and an increase of \$0.3 million in other liabilities. These changes were partially offset by an increase of \$4.3 million in prepaid expenses and other current assets and an increase of \$0.1 million in other assets.

### *Net Cash Flows from Investing Activities*

Net cash used in investing activities for the three months ended March 31, 2019 was \$2.4 million, which consisted of \$2.0 million paid for in-progress research and development assets acquired in connection with Phoenix Tissue Repair, Inc. asset acquisition and \$0.4 million related to purchase of property and equipment.

Net cash used in investing activities for the three months ended March 31, 2018 was \$15.2 million, which consisted of \$15.0 million paid for in-progress research and development assets acquired through asset acquisitions and \$0.2 million related to purchase of property and equipment.

Net cash used in investing activities for the year ended December 31, 2018 was \$21.0 million, which primarily consisted of \$16.0 million paid for in-progress research and development assets acquired through asset acquisitions, \$2.2 million related to purchase of property and equipment and a \$2.9 million decrease in cash and cash equivalents resulting from the deconsolidation of PellePharm.

Net cash used in investing activities for the year ended December 31, 2017 was \$0.5 million, which consisted of our purchase of property and equipment for the purchase of furniture and office equipment, laboratory equipment, and leasehold improvements.

### *Net Cash Flows from Financing Activities*

Net cash used in financing activities of \$1.7 million for the three months ended March 31, 2019 was primarily related to \$1.0 million in cash distributions to our members from the sale of our investment in MyoKardia, Inc. and \$0.9 million payment of deferred offering costs, partially offset by \$0.2 million in proceeds from repayment of nonrecourse notes.

Net cash provided by financing activities of \$15.7 million for the three months ended March 31, 2018 was primarily related to \$12.2 million in proceeds from third-party investors in redeemable convertible noncontrolling interests, \$2.5 million in proceeds from term loans, net of issuance costs, and \$1.0 million in proceeds from issuance of promissory notes.

Net cash provided by financing activities of \$501.5 million for the year ended December 31, 2018 was primarily related to the net proceeds of \$298.7 million from the issuance of our redeemable convertible Series D preferred units, net proceeds of \$95.5 million from the issuance of common stock in connection with the Eidos IPO, proceeds of \$58.4 million from third-party investors in redeemable noncontrolling interests, net proceeds of \$56.4 million received from term loans, net proceeds of \$36.3 million from the issuance of our redeemable convertible Series C preferred units and proceeds from the issuance of promissory notes for \$1.0 million. These amounts were partially offset by the repurchase of a noncontrolling interest in Eidos for \$44.2 million and repayment of term loans for \$1.1 million. The net cash proceeds from the Eidos initial public offering cannot be used by us or our other subsidiaries and may only be used by Eidos or its subsidiaries, if any.

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Net cash provided by financing activities of \$113.0 million for the year ended December 31, 2017 was primarily related to the net proceeds of \$11.8 million from the issuance of our redeemable convertible Series B preferred units, net proceeds of \$95.2 million from the issuance of our redeemable convertible Series C preferred units, proceeds from the issuance of promissory notes for \$4.0 million, proceeds from the repayment of nonrecourse notes for \$0.1 million, proceeds from third-party investors in redeemable noncontrolling interests for \$2.8 million, and proceeds from the common stock issuance and stock option exercise by wholly-owned subsidiaries and VIEs for \$0.1 million. These amounts were offset by \$1.2 million in cash distributions to our members from the sale of our investment in MyoKardia, Inc.

### **Contractual Obligations**

The following table summarizes our contractual obligations as of December 31, 2018:

	Payments Due by Period				Total
	Less than 1 year	1 to 3 years	3 to 5 years	More than 5 years	
Operating lease obligations	\$ 1,924	\$ 3,092	(in thousands) \$ 695	\$ —	\$ 5,711
Hercules term loans debt	\$ —	\$33,764	\$21,236	\$ —	\$55,000
Interest on term loans debt and final end of term payment	\$ 5,200	\$ 9,166	\$ 4,056	\$ —	\$18,422
Total contractual obligations	\$ 7,124	\$46,022	\$25,987	\$ —	\$79,133

In addition to the amount set forth in the table above, we executed the Second Amendment to the Loan and Security Agreement in May 2019 and borrowed an additional \$20.0 million to increase the total principal balance outstanding to \$75.0 million, see “—Liquidity and Capital Resources” above.

We have certain payment obligations under various license and collaboration agreements. Under these agreements we are required to make milestone payments upon successful completion and achievement of certain intellectual property, clinical, regulatory and sales milestones. The payment obligations under the license and collaboration agreements are contingent upon future events such as our achievement of specified development, clinical, regulatory and commercial milestones, and we will be required to make development milestone payments and royalty payments in connection with the sale of products developed under these agreements. As the achievement and timing of these future milestone payments are not probable or estimable, such amounts have not been included in our combined and consolidated balance sheet as of December 31, 2018, our condensed consolidated balance sheet as of March 31, 2019, or in the contractual obligations table above. For additional information regarding certain of our license and collaboration agreements, see “—License and Collaboration Agreements” below.

In addition, we enter into agreements in the normal course of business with contract research organizations for clinical trials and with vendors for preclinical studies and other services and products for operating purposes, which are generally cancelable upon written notice. These payments are not included in the contractual obligations table above.

### **License and Collaboration Agreements**

Our commitments primarily consist of obligations under our agreements entered into with third-party licensors.

### **Off-Balance Sheet Arrangements**

As of December 31, 2018 and March 31, 2019, we did not have any off-balance sheet arrangements, as defined in Item 303(a)(4)(ii) of SEC Regulation S-K. While we have investments classified as VIEs, their purpose is not to provide off-balance sheet financing.

**Quantitative and Qualitative Disclosures about Market Risk**

Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates. We held cash and cash equivalents of \$436.1 million and \$373.0 million as of December 31, 2018 and March 31, 2019, respectively. We generally hold our cash in interest-bearing demand deposit accounts. Cash equivalents consist of amounts invested in money market accounts. Due to the nature of our cash and cash equivalents, a hypothetical 100 basis point change in interest rates would not have a material effect on the fair value of our cash and cash equivalents.

As of December 31, 2018 and March 31, 2019, we had \$55.0 million in variable rate debt outstanding. The Amended Hercules Term Loan matures in July 2022, with interest-only monthly payments until January 2021. The original loan of \$35.0 million bears interest at a floating rate equal to the greater of: (i) the prime rate as reported in the Wall Street Journal plus 4.35% and (ii) 9.35% (9.85% as of March 31, 2019) and the additional borrowing of \$20.0 million bears interest at a floating rate of equal to the greater of: (i) the prime rate as reported in the Wall Street Journal plus 3.35% and (ii) 9.10% (9.10% as of March 31, 2019).

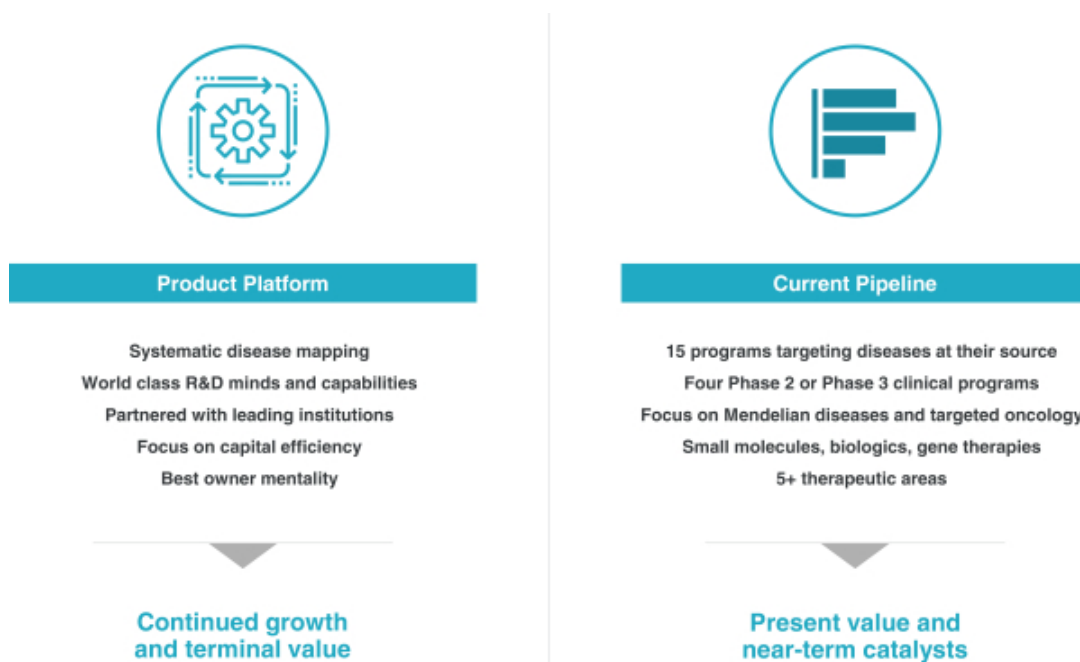
## BUSINESS

### Overview

We are a team of experienced drug discoverers, developers and innovators working to create life-altering medicines that target well-characterized genetic diseases at their source. We founded BridgeBio in 2015 to identify and advance transformative medicines to treat patients who suffer from Mendelian diseases, which are diseases that arise from defects in a single gene, and cancers with clear genetic drivers. Our pipeline of over 15 development programs includes product candidates ranging from early discovery to late-stage development. Several of our programs target indications that we believe present the potential for our product candidates, if approved, to target portions of market opportunities of at least \$1.0 billion in annual sales. We have four product candidates in clinical trials that, if positive, we believe could support the filing of an application for marketing authorization. Two of these product candidates are in Phase 3 clinical trials, one is in a Phase 2/3 clinical trial, and one is in a Phase 2 clinical trial.

We focus on genetic diseases because they exist at the intersection of high unmet patient need and tractable biology. Our approach is to translate research pioneered at academic laboratories and leading medical institutions into products that we hope will ultimately reach patients. We are able to realize this opportunity through a confluence of scientific advances, including: (i) identification of the genetic underpinnings of disease as more cost-efficient genome and exome sequencing becomes available; (ii) progress in molecular biology; and (iii) the development and maturation of longitudinal data and retrospective studies that enable the linkage of genes to diseases. We believe that this early-stage innovation represents one of the greatest practical sources for new drug creation.

We believe we have developed a sustainable and scalable product platform that supports the continued growth of our company and the advancement of our pipeline. Leveraging our product platform, we have a goal of adding two to three programs to our pipeline each year going forward.



### ***Our Platform***

Our platform is distinguished by several key elements:

- **World class discovery and development talent:** Our team has previously submitted over 30 INDs and 15 NDAs, in aggregate. Our operations are overseen by a Management Committee that is comprised of renowned leaders in cancer and rare disease drug development.
- **Disciplined approach to target identification and prioritization:** We pair a systematic mapping of the genetic disease landscape with a proprietary set of over 10 criteria to narrow our focus on diseases with attractive attributes for drug development. We look for diseases with high unmet need and well-characterized mechanisms that present opportunities to address the root cause of disease.
- **Opportunistic approach to drug candidate selection:** We seek the best science and drug mechanisms of action, wherever they can be found. We accept programs that meet our standards at any stage of development, and we are agnostic to therapeutic area. However, we only pursue programs with treatment modalities that we believe are biologically suited to address the target disease.
- **Focus at the level of each program:** We maintain a decentralized structure wherein each program is housed in its own subsidiary. This allows us to build a team of experts and specialists tailored to the needs of each program, and who are economically incentivized at the program level. We enable our subsidiary leaders to make certain operational decisions outside of a centralized management hierarchy, as we fundamentally believe that those operators who have the most intimate program knowledge are best positioned to make key operational decisions.
- **Operational efficiency:** We aim to rapidly and decisively advance our product candidates to objective critical decision points. At each stage of research, discovery, or development, we direct resources toward the opportunities that we believe are the most promising, and we discontinue programs that do not meet performance thresholds. We field a minimum viable team for each asset, with the goal of ensuring that each program has sufficient personnel to fit its purpose while reducing excess overhead costs. We accomplish this by hiring the best talent, centralizing and sharing certain support functions across various programs, and leveraging external providers where appropriate. This enables us to minimize traditionally fixed costs at the program level.
- **Portfolio breadth and diversification:** We have built a broad and diversified portfolio with 15 programs that vary across stage of development, therapeutic category and modality. We believe that our programs are biologically uncorrelated, covering different diseases, different targets, and different modalities, such that the results of one program will not impact the development of others. Further, the breadth of our portfolio mitigates the impact of failure of any single program. As a result, we can be objective about each of our programs and allocate capital efficiently, delivering staged funding across our portfolio based on each program's scientific merits.
- **Optimized ownership for each program:** When we believe that we are best suited to continue a program's development, we will continue to fund it internally. If we believe a strategic partner is better suited to progress a program, we will consider externalizing development at economically attractive terms.

### ***Our Pipeline***

Our product platform supports the advancement of our current pipeline, which includes over 15 development programs that can be divided into three key categories:

- **Mendelian:** Ten small molecule and protein replacement product candidates, of which two are currently in Phase 3 clinical trials, one is in a Phase 2/3 clinical trial, one is in a Phase 1/2 clinical trial, three are currently in IND-enabling development and three are in lead optimization. Over the next 24 months, we expect to have at least six of these product candidates in clinical development. Several of our product

candidates in this category target some of the most prevalent Mendelian diseases, including ATTR and achondroplasia. Two of our programs in this category have received breakthrough therapy designation from the FDA.




- **Oncology:** Four targeted oncology programs, including one in Phase 2 clinical development, of which one is in IND-enabling development and two are in lead optimization, that address key oncogenic pathways including FGFR, KRAS and SHP2. These programs have potentially broad applicability across a number of solid tumor types with high unmet patient need.
- **Gene therapy:** Focused on developing treatments for Mendelian diseases that are particularly suited to gene therapy. Our two programs are both in IND-enabling development and we expect both of these programs to be in the clinic by 2020. Our gene therapy programs are led by executives who have substantial domain expertise and are recognized leaders in this field, and we are actively building our gene therapy capabilities.

Of our development programs, we believe the following, which we refer to as our key value drivers, have the greatest potential to drive significant value for our company due to a combination of factors, including their stage of development, potential availability of expedited development pathways, degree of unmet medical need and potential market size in the applicable target indication:

- BBP-265 (also known as AG10, under development at our subsidiary, Eidos Therapeutics, Inc.), a small molecule stabilizer of TTR that is in an ongoing Phase 3 clinical trial for the treatment of ATTR-CM.
- BBP-831 (under development at our subsidiary, QED Therapeutics, Inc.), a small molecule selective FGFR1-3 inhibitor being developed for the treatment of FGFR-driven cancers and achondroplasia, for which we intend to submit an NDA in 2020 for the treatment of CCA as a second-line or later therapy.
- BBP-631 (under development at our subsidiary, Adrenas Therapeutics, Inc.), an AAV5 gene transfer product candidate in preclinical development for the treatment of CAH, driven by 21OHD.
- BBP-454 (under development at our subsidiary TheRas, Inc.), a preclinical development program for small molecule inhibitors of KRAS for the treatment of pan-mutant KRAS-driven cancers, which act via two novel binding pockets.

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The following table summarizes our material development programs, their estimated patient populations, their therapeutic modalities and their development status:

Program / Subsidiary <sup>1</sup>	Diseases	Patient population (US+EU) <sup>2</sup>	Modality	Pre-Clinical		Clinical			
				Lead Finding / Optimization	IND Enabling	Phase 1	Phase 2	Phase 3	
 Mendelian	BBP-265 / Eidos ☆	ATTR	>400,000	☆	[Progress bar: Phase 1 to Phase 3]				
	BBP-831 / QED ☆	Achondroplasia	55,000	☆	[Progress bar: Lead Finding / Optimization to Phase 1]				
	BBP-870 / Origin	MacD Type A	100	☆	[Progress bar: Phase 1 to Phase 3]				
	BBP-009 / PellePharm <sup>4</sup>	Gorlin, frequent BCC	120,000	☆	[Progress bar: Phase 1 to Phase 3]				
	BBP-589 / PTR	RDEB	1,500	☆	[Progress bar: Lead Finding / Optimization to Phase 1]				
	BBP-681 / Venthera	Venous Malformations	117,000	☆	[Progress bar: Lead Finding / Optimization to Phase 1]				
	BBP-671 / CoA	PKAN, OAs	7,000	☆	[Progress bar: Lead Finding / Optimization to Phase 1]				
	BBP-711 / Orfan	PH1/ FSF	5,000/ 1.5M	☆	[Progress bar: Lead Finding / Optimization to Phase 1]				
	BBP-561 / MoST	Netherton	11,000	☆	[Progress bar: Lead Finding / Optimization to Phase 1]				
BBP-761 / Fortify	LHON	20,000	☆	[Progress bar: Lead Finding / Optimization to Phase 1]					
 Oncology	BBP-831 / QED ☆	FGFR+ cancers	37,000*	☆	[Progress bar: Phase 1 to Phase 3]				
	BBP-454 / TheRas ☆	KRAS+ cancer	>500,000*	☆	[Progress bar: Lead Finding / Optimization to Phase 1]				
	BBP-398 / Navire	Multiple tumors	>500,000*	☆	[Progress bar: Lead Finding / Optimization to Phase 1]				
 Gene Therapy	BBP-954 / Ferro	Multiple Tumors	>500,000*	☆	[Progress bar: Lead Finding / Optimization to Phase 1]				
	BBP-631 / Adrenas ☆	CAH	>75,000	☆	[Progress bar: Lead Finding / Optimization to Phase 1]				
BBP-812 / Aspa	Canavan	1,000	☆	[Progress bar: Lead Finding / Optimization to Phase 1]					

<sup>1</sup> Each of our programs is housed in a separate subsidiary. <sup>2</sup> Patient population: Prevalence except for asterisked figures which represent incidence; <sup>3</sup> Ongoing clinical trial we believe could support filing an application for marketing authorization, although the FDA and other regulatory authorities have not indicated their agreement or that additional trials will not be required. <sup>4</sup> We are party to an option agreement pursuant to which LEO Pharma A/S has been granted an exclusive, irrevocable option to acquire PellePharm, including the BBP-009 program. If the option is exercised by LEO Pharma A/S, we will no longer have rights to develop and commercialize BBP-009. See "Business—Our Material Agreements—BBP-009 (Patislogib): Option Agreement with LEO Pharma A/S." <sup>5</sup> Planned NDA submission for the treatment of cholangiocarcinoma as a second-line or later therapy

## Our Focus

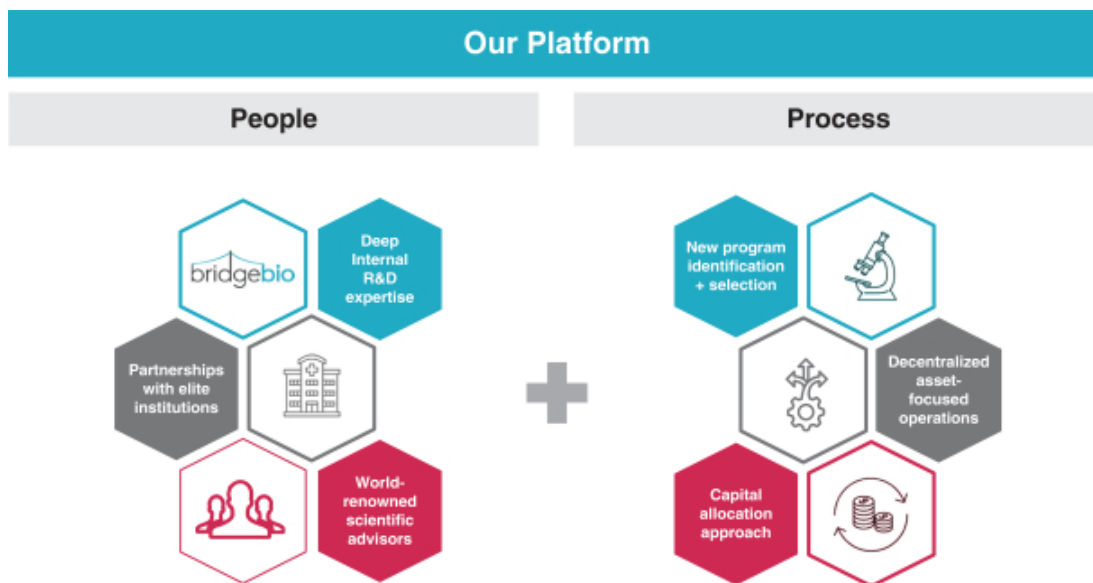
We focus on developing medicines for genetic diseases that arise from mutations in patients' DNA. These mutations can be either inherited or arise spontaneously. The genetic diseases we target include both Mendelian diseases and certain cancers with clear genetic drivers.

We are focused on genetic diseases due to several inherent advantages:

- **Clear targets for drug development:** Genetic diseases often provide direct links from the mutation and pathway of disease to symptoms. This mechanistic clarity allows us to identify tractable targets and develop product candidates whose mechanism of action is designed to treat the source of the disease.
- **Historically higher than average probability of success:** We are pursuing drug development for indications generally classified as orphan diseases. Between 2006 and 2015, drug programs in orphan designated indications had, on average, a 2.5 times higher likelihood of successful development from Phase 1 to approval than drugs across all indications.
- **High unmet need and meaningful commercial opportunity:** Thousands of genetic diseases exist and affect millions of individuals worldwide. Over 7,400 rare Mendelian phenotypes have been identified. However, since 1996, the FDA has approved fewer than 70 treatments for genetically driven conditions. This presents a vast unmet patient need which we believe we can help address.

## Our Platform

Our product platform consists of our people and our process.



### Our People

We are led by a Management Committee that includes respected leaders in the field of drug development who have worked together previously. This team includes our founders Charles Homcy, Frank McCormick, Phil Reilly and Neil Kumar, and is supplemented by our Chairman of Research & Development, Richard Scheller, and our senior advisor, Len Post. Our research and developments efforts are spearheaded by Chief Scientific Officer, Uma Sinha, and leading medicinal chemist Robert Zamboni.

Our team has contributed to the development of more than 30 molecules from preclinical development through IND submission and more than 15 approved products. Our team has a rich set of experiences at the intersection of genetic disease and company leadership, including founding and leadership roles at companies including bluebird bio, Inc., Genentech, Inc., Global Blood Therapeutics, Inc., MyoKardia, Inc., Onyx Pharmaceuticals, Inc., Portola Pharmaceuticals, Inc. and Voyager Therapeutics, Inc.

Our subsidiaries' management teams consist of individuals with diverse backgrounds, including entrepreneurs, experienced scientists and business and science-oriented professionals. The complementary skills of these groups facilitate the creation of lean teams with overlapping strengths well-suited to our empowered, decentralized management model.

Due to our leaders' relationships and experience, we have built a deep network of relationships across the genetic disease space, with leading institutions, academics and industry.

### Our Process

Our process is comprised of three components: new program identification and selection, operations and capital allocation.



### ***New Program Identification and Selection***

A disciplined and proactive approach to identifying and selecting new programs is central to our platform. We see the following as critical elements of our new program identification and selection process:

- **Comprehensive mapping of the genetic disease space:** To prospectively inform our search for new development candidates, we pair a systematic mapping of the genetic disease landscape with a proprietary set of over 10 criteria to narrow our focus on diseases with attractive attributes for drug development. We maintain a science-driven, constantly evolving perspective on the universe of potential development targets driving the diseases we prioritize.
- **Targeting well-characterized mechanisms of disease at the source:** We seek to treat genetic diseases by correcting disease pathobiology at its source. By targeting the source, our goal is to mitigate the risks that compensatory pathways may overcome the interventions that we seek to develop. This requires a robust understanding of genotype and phenotype relationships and of the biological pathways involved in each disease. Based on our deep understanding of disease mechanisms, we employ therapeutic modalities that we believe are biologically suited to address the target disease.
- **Leveraging deep relationships within the genetic disease area:** Our contacts and partnerships provide us with access to a wide range of ongoing scientific work in genetic disease areas. We also remain proactive, constantly surveying the landscape for potentially actionable opportunities. To date, we have executed partnerships with leading academic institutions such as Boston Children's Hospital, Cincinnati Children's Hospital Medical Center, MD Anderson Cancer Center, the University of California San Francisco, St. Jude Children's Research Hospital, and others. We plan to continue to grow and expand these types of relationships.
- **Thorough vetting combined with early and decisive action:** We follow a disciplined and methodical approach to review new opportunities before committing capital to a development program. Our Management Committee reviews all potential development programs utilizing a number of parameters to vet the attractiveness of investments, with a clear focus on the underlying science. These parameters include mechanistic rationale, preclinical and clinical data generated, potential commercial viability, intellectual property position, prospects for favorable pricing and reimbursement and the potential impact of competition. In addition, our Management Committee is focused on portfolio fit and capital allocation. Once we deem an opportunity appropriate, we move rapidly to acquire or license the asset.

### ***Decentralized, Asset-Focused Operations***

We employ a decentralized operating model designed to enable robust scientific discovery and drug development in a scalable and capital efficient manner. As a feature of this model, we establish subsidiary organizations organized around individual product candidates which are directly responsible for the advancement of each asset. Our approach empowers operational decision making at the level of the program, which we believe increases productivity and speed. Key elements of our operating model include:

- **Subsidiary teams incentivized on individual asset outcomes:** We build teams of experts and specialists tailored to the needs of each program who are economically incentivized at the program level. This creates an intense focus on advancing drug candidates to patients. Our structure leaves certain operational decision-making in the hands of those closest to the programs. We believe that this results in faster and better decision making.
- **Lean cost structure at the program level:** We build our teams with a view towards fielding the minimum viable team for each program. This requires hiring the best talent and using best-in-class external providers wherever possible. Our approach allows us to maintain what we believe is among the lowest ratios of headcount to development candidate programs in the industry. As of March 31, 2019, we had fewer than 155 full time employees, including 19 at the parent company and 133 at subsidiaries. We often assemble teams around early stage assets comprised initially of fewer than five people, growing to approximately ten people at the IND-enabling stage. Once a program is in the clinic, we will size and structure a team commensurate with need, subject to our minimum viable team approach.

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- **Minimization of overhead and fixed costs:** Our central team provides operating leverage to our program teams by providing certain shared services and workspaces. We believe this enables us to minimize overhead costs that may otherwise be incurred by individual companies that are required to build out full non-R&D support functions and fixed cost infrastructure, despite relatively small pipelines.
- **Small central team incentivized on portfolio outcomes:** At the parent company level, we maintain a small central team focused on (i) identifying and securing new programs, (ii) setting up new subsidiaries and (iii) recruiting key management team members. The central team is also responsible for raising and allocating capital across the portfolio in collaboration with the Management Committee. Central team members are compensated with equity at the parent level, and are thereby incentivized to maximize value of the portfolio.

### ***Capital Allocation Approach***

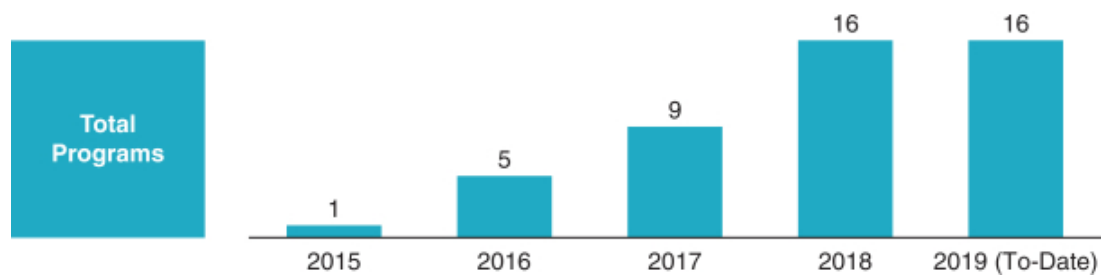
Our approach to capital allocation is designed to help mitigate the inherent risks of drug development, while, at the same time, maximizing opportunities to create value for shareholders and patients. This capital allocation approach is guided by the following core tenets:

- **Staged funding based on scientific results:** We seek to ensure objective and science-driven decision making when investing in each of our programs. We therefore fund programs with capital sufficient to reach key decision points in their development. Upon reaching these decision points, our Management Committee reviews the data generated by the program and re-evaluates whether the program warrants continued investment. If a program passes such a decision point, we continue to fund its development. If not, we may discontinue its development. Given the risks inherent in early-stage development, we spend judiciously on early stage opportunities.
- **Optimized program ownership:** Our goal is to ensure that each of our programs has the resources and expertise needed to provide the best possible chance to reach patients broadly. While we generally intend to develop our programs through approval and ultimately self-commercialize, we may consider entering into strategic partnerships for our programs when we believe such partnerships will further our goals and are economically attractive.
- **Broadly diversified portfolio:** While we are focused on developing assets in a category with what we believe are meaningfully increased odds of successful development, we recognize that we will likely experience development failures. Given this expectation, we have built a broad and diversified portfolio to mitigate the impact of this risk. Our portfolio of programs in development spans a range of therapeutic areas, modalities and stages of development. We believe that our programs are uncorrelated, with the success or failure of any individual program being decoupled from the outcome of others in the portfolio, and that this approach provides a fundamentally reduced corporate risk profile.

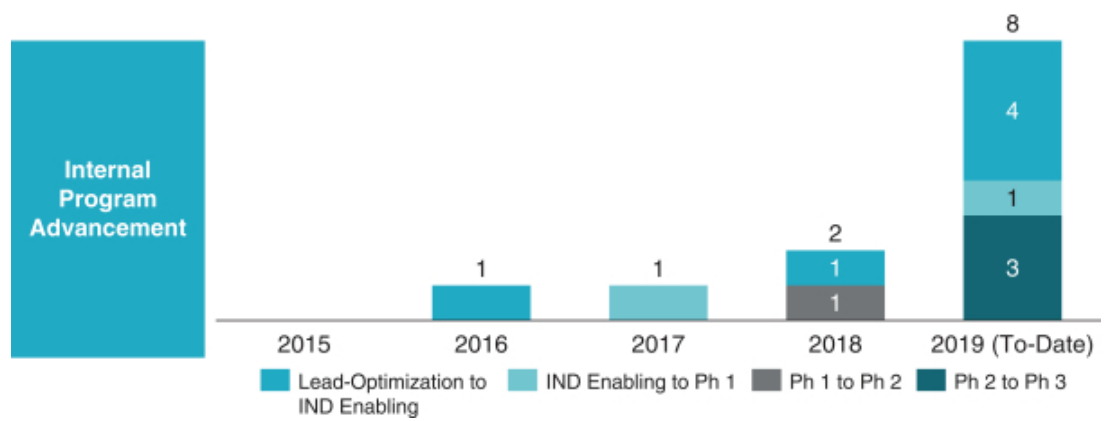
## Our Pipeline

### Pipeline Growth

Since our inception, we have actively built our pipeline through business development efforts, including the acquisition and in-licensing of assets, and advancing programs through internal stage-gates. The following chart shows the growth of assets in our pipeline:






We have advanced our product candidates through key stage-gates as shown below:



**Summary and Key Catalysts**

The following table summarizes our programs, their current status and upcoming catalysts:

Program (Subcategory)	Disease	Patient population (L10-CU) <sup>1</sup>	Modality	Current status	Anticipated upcoming catalysts	
 Mendelian	BBP-265 (Eidos) ☆	ATTR	>400,000	☆	Phase 3 <sup>2</sup>	<ul style="list-style-type: none"> <li>Phase 3 OLE data (2019)</li> <li>Initiation of Phase 3 in ATTR-PN (2019)</li> <li>Anticipated reporting of 12-month 6MWD data from Phase 3 ATTR data study in ATTR-CM (2021)</li> <li>Potential NDA submission in ATTR-CM (2021)</li> </ul>
	BBP-451 (G4C) ☆	Acknowledgments	55,000	☆	Pre-Clinical	<ul style="list-style-type: none"> <li>IND filing and initiate Phase 1/2 (2020)</li> <li>Anticipated Phase 1/2 clinical proof-of-concept data (2021)</li> </ul>
	BBP-470 (Origis) ☆	MeD Type A	100	☆	Phase 2 <sup>2</sup>	<ul style="list-style-type: none"> <li>NDA submission (2020)</li> </ul>
	BBP-009 (PellePhase) <sup>3</sup>	Gorlin, HF-BCC	120,000	☆	Phase 3 <sup>2</sup>	<ul style="list-style-type: none"> <li>Initiation of Phase 3b trial in HF-BCC (2019)</li> <li>Conclude Phase 3 trial in Gorlin Syndrome (2020)</li> </ul>
	BBP-569 (FTR) ☆	ICCB	1,300	☆	Phase 1	<ul style="list-style-type: none"> <li>Data from Phase 1/2 trial (2020)</li> </ul>
	BBP-461 (Festres) ☆	Vascular malformations	117,000	☆	Pre-Clinical	<ul style="list-style-type: none"> <li>IND filing (2020)</li> </ul>
	BBP-471 (CoA) ☆	PKAN, CA10R	7,000	☆	Pre-Clinical	<ul style="list-style-type: none"> <li>IND filing (2020)</li> </ul>
	BBP-711 (Orbis) ☆	PIH/FSF	2,000 <sup>4</sup> 1.5M	☆	Pre-Clinical	<ul style="list-style-type: none"> <li>Development candidate nomination (2019)</li> </ul>
	BBP-561 (BioST) ☆	Netherton	11,000	☆	Pre-Clinical	<ul style="list-style-type: none"> <li>Development candidate nomination (2019)</li> </ul>
	BBP-761 (Fortis) ☆	USHN	20,000	☆	Pre-Clinical	<ul style="list-style-type: none"> <li>Development candidate nomination (2020)</li> </ul>
 Oncology	BBP-451 (G4C) ☆	FGFR+ Cancers	37,000 <sup>5</sup>	☆	Phase 2 <sup>2</sup>	<ul style="list-style-type: none"> <li>Initiate Phase 3 clinical trial for advanced CCA in the 1L setting (2019)</li> <li>NDA filing for advanced CCA in 2L setting or later (2020)</li> <li>Initiate Phase 3 clinical trial in adjacent unmet oncology (2020)</li> </ul>
	BBP-451 (TheRise) ☆	KRAS+ cancer	>500,000 <sup>6</sup>	☆	Pre-Clinical	<ul style="list-style-type: none"> <li>Development candidate nomination (2020)</li> </ul>
	BBP-598 (Tevira) ☆	Multiple cancers	>500,000 <sup>6</sup>	☆	Pre-Clinical	<ul style="list-style-type: none"> <li>IND filing (2020)</li> </ul>
	BBP-594 (Ferra) ☆	Multiple Cancers	>500,000 <sup>6</sup>	☆	Pre-Clinical	<ul style="list-style-type: none"> <li>Development candidate nomination (2020)</li> </ul>
 Gene Therapy	BBP-451 (Adriana) ☆	CAH	>75,000	☆	Pre-Clinical	<ul style="list-style-type: none"> <li>IND filing (2020)</li> <li>Anticipated reporting of clinical proof-of-concept data (2021)</li> </ul>
	BBP-012 (Kupa) ☆	Cerevis	1,000	☆	Pre-Clinical	<ul style="list-style-type: none"> <li>IND filing (2020)</li> </ul>

☆ Key Value Drivers
☆ Small molecule
☆ Topical small molecule
☆ Biologic
☆ Gene therapy

\* Each of our programs is housed in a separate subsidiary  
<sup>1</sup> Patient population: Prevalence figures presented except for asterisked which represent incidence; midpoint of range presented where data estimates are available  
<sup>2</sup> Ongoing clinical trial we believe could support filing an application for marketing authorization, although the FDA and other regulatory authorities have not indicated their agreement or that additional studies will not be required  
<sup>3</sup> We are party to an option agreement pursuant to which LEO Pharma A/S has been granted an exclusive, irrevocable option to acquire PellePhase, including the BBP-009 program. If the option is exercised by LEO Pharma A/S, we will no longer have rights to develop and commercialize BBP-009.

**KEY VALUE DRIVERS**

**BBP-265/AG10 (Eidos): TTR Amyloidosis**

**Summary**

- We are developing BBP-265, an oral small molecule TTR, stabilizer, for the treatment of TTR amyloidosis, or ATTR, including both cardiomyopathy and polyneuropathy manifestations, or ATTR-CM and ATTR-PN, respectively

**Development Status and Catalysts**

- Ongoing – Phase 3 clinical trial in ATTR-CM
- 2019 – Planned initiation of Phase 3 clinical trial in ATTR-PN
- 2019 – Anticipated reporting of data from ATTR-CM Phase 2 open-label extension on safety and key cardiac biomarkers
- 2021 – Anticipated reporting of 12-month 6MWD data from Phase 3 ATTRibute study in patients with ATTR-CM
- 2021 – Potential NDA submission in ATTR-CM

**Disease Overview**

- Amyloid deposition can cause ATTR-CM (wild-type and mutant) and ATTR-PN. Both manifestations of disease are progressive, have a significant negative impact on quality of life, and are eventually fatal

- 
- ATTR is caused by dissociation of TTR tetramers into monomers and subsequent aggregation as amyloid proteins (amyloid deposition) leading to organ damage
  - Prevalence greater than 400,000 worldwide for ATTR-CM, and greater than 10,000 worldwide for ATTR-PN
  - Cardiomyopathy diagnosis by non-invasive nuclear imaging and/or endomyocardial biopsy, polyneuropathy diagnosis by physical exam and/or neurophysiological tests
  - No established standard of care exists globally for ATTR-CM, though tafamidis has recently been approved for this indication in the United States and Japan. Tafamidis, patisiran and inotersen are currently marketed for ATTR-PN in various geographies

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**Our Product Concept**

- Oral small molecule TTR stabilizer designed to bind TTR and mimic the conformation of the naturally occurring T119M rescue mutation, which “super-stabilizes” TTR tetramers
- Potential to halt or reverse cardiomyopathy and neuropathy progression
- In a Phase 2 clinical trial completed in ATTR-CM patients, BBP-265 was well-tolerated, and findings showed near complete TTR stabilization and restoration of normal serum TTR levels
- There is a strong correlation between level of TTR stabilization, TTR serum levels, and disease severity. We believe that clinical outcomes may be correlated to the level of TTR stabilization
- TTR is an important biological molecule. We believe that therapeutic interventions that increase serum TTR are likely to result in better clinical outcomes than therapies that decrease serum TTR levels, assuming similar levels of monomer concentration

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**Key Competitors**

- Tafamidis, a TTR tetramer stabilizer
  - Patisiran, a RNAi TTR knockdown agent
  - Inotersen, an antisense oligonucleotide TTR knockdown agent
- 

***Disease Overview***

ATTR is a disease caused by destabilization of TTR tetramers resulting in amyloid deposition. TTR is a protein that occurs naturally in the form of a tetramer, which is a molecular structure consisting of four identical subunits, or monomers, and performs multiple physiologic roles, including the transport of essential hormones and vitamins. In ATTR, TTR tetramers become destabilized due to a mutation in the TTR gene or as part of the natural aging process. Destabilized TTR dissociates into monomers, self-aggregates and assembles into fibrils which are deposited, predominantly in the heart and nervous system, driving disease pathophysiology.

ATTR is commonly categorized by its genotypic cause and primary clinical manifestation: wild-type ATTR cardiomyopathy, or ATTRwt-CM, which results from an age-related process; mutant ATTR cardiomyopathy, or ATTRm-CM; and ATTR polyneuropathy, or ATTR-PN, which is only associated with TTR mutants. All three forms of the disease are progressive and fatal. ATTRwt-CM and ATTRm-CM patients generally present with symptoms later in life (older than 50) and have median life expectancies of three to five years from diagnosis. ATTR-PN presents either in a patient’s early 30s or later (older than 50), and results in a median life expectancy of five to ten years from diagnosis. Progression of all forms of the disease causes significant disability, impacts productivity and quality of life, and creates a significant economic burden due to the costs associated with patient need for supportive care. As the disease progresses, ATTRwt-CM and ATTRm-CM patients may require frequent hospitalizations and repeated interventions. ATTR-PN patients experience gradual loss of the ability to walk without assistance, and autonomic nervous system function affecting digestion and blood pressure.

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The worldwide estimated prevalence of ATTRwt-CM, ATTRm-CM, and ATTR-PN is approximately 400,000, 40,000, and 10,000, respectively. However, we believe that the cardiomyopathic forms of the disease are significantly underdiagnosed. For example, recent literature has suggested that between 12% to 19% of patients diagnosed with heart failure with preserved ejection fraction may, in fact, have undiagnosed ATTR-CM. This single segment represents approximately half of the 6.0 million to 7.0 million estimated people with heart failure in the United States alone. With the increasing availability of disease modifying therapeutics, disease awareness is heightened.

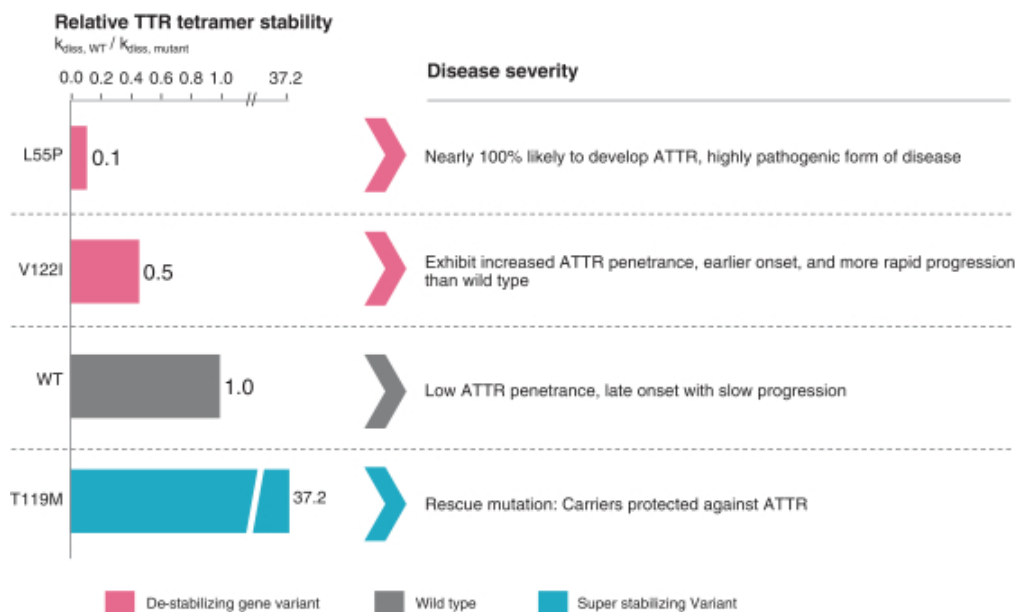
We believe the population of diagnosed ATTR-CM patients is also growing rapidly due to the shift to an accurate and reliable non-invasive diagnostic imaging technique. Historically, a heart biopsy was required to make a diagnosis of ATTR-CM. Recently, however, it has been shown that scintigraphy with technetium-labelled radiotracers is a highly accurate, non-invasive and cost effective method for ATTR-CM diagnosis. We believe that both increased disease awareness and availability of this non-invasive diagnostic imaging technique are allowing for earlier diagnosis of ATTR-CM patients and the identification of previously misdiagnosed patients.

### ***Our Product Concept***

BBP-265 is a clinical-stage orally-administered, small molecule TTR stabilizer being developed to treat ATTR at its source by reducing the level of amyloid formation through TTR stabilization. This has been shown in preclinical studies and clinical trials to prevent the dissociation of tetrameric TTR into monomers, and in preclinical studies, to reduce the rate of amyloid fibril formation. In addition, BBP-265 has been shown to lead to increased circulating levels of tetrameric TTR. BBP-265 has been designed to bind TTR in a way that causes TTR's conformational structure to mimic that of the well-characterized T119M variant, a naturally occurring rescue mutation which super stabilizes the TTR tetramer. T119M has been observed to prevent the dissociation of TTR tetramers into monomers; T119M tetramers dissociate 40-fold more slowly than wild-type tetramers in biochemical assays. Known as a trans-allelic trans-suppressor, individuals who coinherit the T119M rescue mutation along with a TTR-destabilizing mutation, are protected against the development of ATTR.

In third party clinical trials of tafamidis and diflunisal, interventional approaches that increased TTR stabilization led to improved outcomes in this disease and were correlated with increases in serum TTR. Further, based on genetic data, there is a correlation between the level of TTR stabilization, serum TTR levels, and disease severity. As a result, we believe that serum TTR is a predictive biomarker for disease prognosis and that there may be a relationship between more effective TTR stabilization, serum TTR levels, and improved clinical outcomes. Based on head-to-head preclinical data, we believe that BBP-265 has the potential to stabilize TTR to a greater extent than other TTR stabilizers.

**Human genetics suggest TTR stability is associated with disease severity**



The above chart shows the correlation between TTR stability, as assessed using recombinant protein in vitro, and disease severity in ATTR patients. Patients with TTR variants that result in highly destabilized TTR are nearly 100% likely to develop the disease, like those with the L55P gene variant. Patients who have super-stabilized TTR, as in the case of individuals with the T119M gene variant, are protected against ATTR and cerebrovascular diseases.

We believe that TTR is an important plasma protein as evidenced by the fact that it is highly evolutionarily conserved, existing in high concentrations in all vertebrates. We believe that therapies that increase serum TTR are likely to result in better clinical outcomes than therapies that decrease TTR serum levels, assuming similar levels of monomer reduction. This hypothesis is further supported by two prospective studies of 68,602 participants in Denmark over an average 32 years of clinical follow-up, which showed that individuals who inherited the T119M mutation in the absence of a TTR pathogenic gene had higher circulating TTR concentrations, had a lower range of cerebrovascular events, especially fatal or debilitating stroke, and had a five-to-ten year increase in life expectancy relative to the general population. Additionally, data from a retrospective study at Boston University, suggests a correlation between serum TTR changes and mortality in ATTRwt-CM patients.

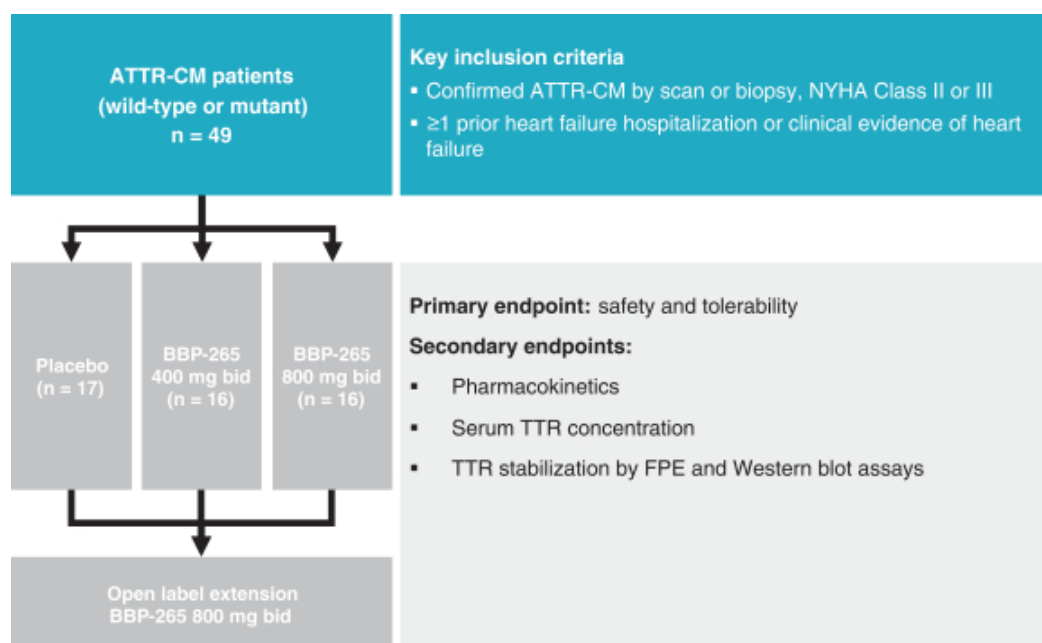
**Clinical Data**

*Phase 2 Data*

In April 2018, we initiated our Phase 2 randomized, placebo-controlled, dose-ranging clinical trial of BBP-265 in 49 patients with symptomatic ATTR-CM, of which 14 had ATTRm-CM. Eligible patients were randomized in a 1:1:1 ratio to placebo or 400 mg or 800 mg of BBP-265 twice daily. The primary objective of the trial was to evaluate the safety and tolerability of BBP-265 administered to adult subjects with symptomatic ATTR-CM. The secondary objectives were to characterize the pharmacokinetics, or PK, of BBP-265 in symptomatic ATTR-CM subjects and to describe the pharmacodynamics, or PD, properties of BBP-265, as well as the PK-PD relationship

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of BBP-265. The PD assessments of TTR stabilization were measured by fluorescent probe exclusion, Western blot and serum prealbumin (TTR). The trial design is depicted below:



Enrolled symptomatic ATTR-CM subjects ranged in age from 60 to 86 years of age, with a mean age of 74.1, and 92% of subjects were male. In this trial, we enrolled subjects exclusively with advanced disease, with 29% of subjects presenting with New York Heart Association (NYHA) Class III heart failure symptoms and a high baseline NT-proBNP with a mean of 3,368 pg/mL. Additionally, on average, subjects had relatively low TTR at baseline with a mean of 22.0 mg/dL. The laboratory reference range for serum TTR is 20mg/dL to 40 mg/dL in healthy individuals. Both high NT-proBNP and low TTR are biomarkers of disease severity. The subject disposition and baseline characteristics are shown below.

Characteristic	Placebo (n = 17)	BBP-265 400 mg (n = 16)	BBP-265 800 mg (n = 16)	Total (n = 49)
Age, mean (range)	73.2 (60-83)	73.8 (60-83)	75.4 (67-86)	74.1 (60-86)
Male, n (%)	17 (100%)	14 (88%)	14 (88%)	45 (92%)
ATTRm-CM, n (%)	3 (18%)	6 (38%)	5 (31%)	14 (29%)
NYHA Class III, n (%)	5 (29%)	6 (38%)	3 (19%)	14 (29%)
Race, n (%)				
White	13 (76%)	10 (62%)	12 (75%)	35 (72%)
Black	3 (18%)	4 (25%)	3 (19%)	10 (20%)
Other	1 (6%)	2 (13%)	1 (6%)	4 (8%)
NT-proBNP (pg/mL) <sup>1</sup>	3151 ± 2705	3589 ± 3020	3377 ± 2806	3368 ± 2789
Troponin I (ng/mL) <sup>2</sup>	0.17 ± 0.30	0.22 ± 0.24	0.10 ± 0.06	0.16 ± 0.22
TTR (mg/dL) <sup>3</sup>	23.4 ± 5.5	23.2 ± 5.7	19.5 ± 4.2	22.0 ± 5.4

1 NT-proBNP normal range = 0 – 449 pg/mL; NT-proBNP = N-Terminal pro B-type Natriuretic Peptide

2 Troponin I normal range = 0 – 0.02 ng/mL

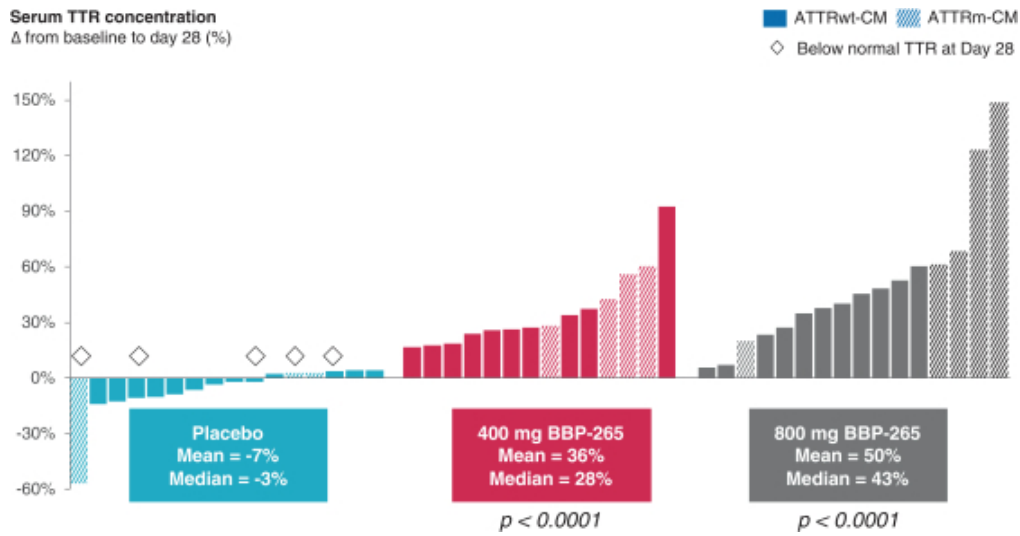
3 TTR normal range = 20 – 40 mg/dL



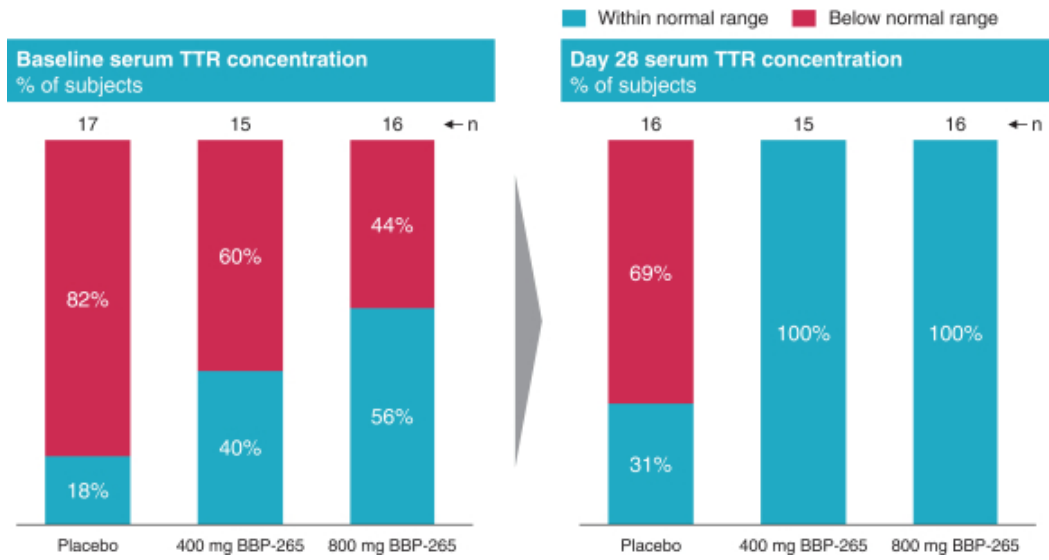
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Overall, BBP-265 was well-tolerated in symptomatic ATTR-CM subjects with no lab safety signals of potential clinical concern attributed to study drug. In this trial, 88% of subjects administered placebo experienced AEs and 63% and 69% of subjects administered 400 mg and 800 mg BBP-265 experienced AEs, respectively. In both the placebo and active treatment groups, most of the AEs were mild to moderate in severity. The most commonly observed AEs, occurring in four or more subjects across the treatment and placebo groups, were atrial fibrillation, constipation, diarrhea and muscle spasms. Three subjects reported SAEs during this study. One placebo-treated subject experienced two SAEs of atrial fibrillation and congestive heart failure and another placebo-treated subject experienced cellulitis in their lower extremity. One BBP-265 treated subject experienced an SAE of shortness of breath on study, which was considered unlikely to be related to study drug.

As shown in the chart below, subjects in the placebo group experienced a mean 7% reduction in the circulating tetrameric TTR concentrations compared to baseline. Conversely, subjects administered either 400 mg or 800 mg BBP-265 showed a dose-dependent statistically significant mean increase in circulating TTR of 36% and 50%, respectively, compared to baseline. Compared to placebo, both the 400 mg and 800 mg BBP-265 arms demonstrated statistically significant increases in mean circulating TTR ( $p < 0.0001$  for both arms). p-value is a statistical calculation that relates to the probability that the difference between groups happened by chance, with a p-value of less than 0.05 (i.e., less than 5% probability that the difference happened by chance) generally being used as the threshold to indicate statistical significance. There was a greater observed treatment effect in subjects with mutant ATTR-CM as compared to subjects with wild-type ATTR-CM, which we believe can be explained, in part, by the lower absolute serum TTR of mutant ATTR-CM subjects at baseline.

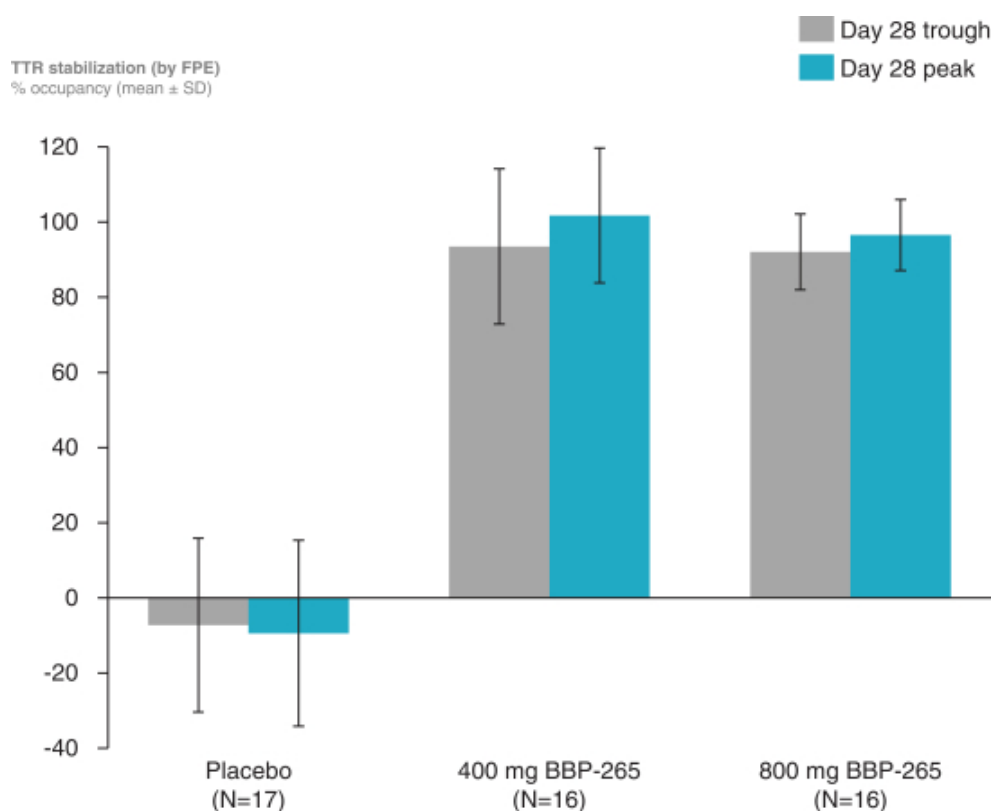


The following chart shows that treatment with BBP-265 restored serum TTR concentrations to within the normal range in all subjects at Day 28.



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*Ex vivo* stabilization assays demonstrated near-complete TTR stabilization by BBP-265, with greater than 90% average tetramer stabilization across subjects treated with 400 mg and 800 mg BBP-265 as shown in the chart below. The stabilization response was consistent across mutant and wild-type TTR carriers and replicates previously reported clinical and preclinical TTR stabilization data.



An open label extension study of the Phase 2 clinical trial remains ongoing. We expect to report results from this study in 2019, which we expect will include data on safety, key cardiac biomarkers and key echocardiogram parameters.

### Phase 1 Data

In our Phase 1 clinical trial, 56 healthy volunteers were administered BBP-265 and at the highest tested dose we observed greater than 95% stabilization of TTR, on average, across the entire dosing interval and 100% stabilization at peak blood levels. In contrast, the peak blood levels achieved by tafamidis at 20 mg and 80 mg provided approximately 45% and 60% stabilization at peak blood levels, respectively, in our preclinical studies. We believe these observations of BBP-265's comparatively higher stabilization were attributable to BBP-265's binding mode and specificity for binding to TTR and not other plasma proteins.

We observed no clinically important AEs or laboratory-based signals of potential clinical concern associated with BBP-265 in healthy adult volunteers participating in our Phase 1 clinical trial of BBP-265. Most AEs were reported by single subjects, and all were mild to moderate in intensity. The only AEs that occurred in more than one subject were dry mouth, generalized headache, upper respiratory infection, and dizziness, all of which occurred in two separate subjects. In our preclinical studies, BBP-265 exhibited a 40- to 80-fold therapeutic window between its target therapeutic blood level and those concentrations associated with observed, dose-limiting animal toxicity in

28-day studies. We achieved or exceeded that targeted therapeutic blood level in healthy volunteers and ATTR-CM patients at doses that were well-tolerated in the Phase 1 and Phase 2 clinical trials.

***Preclinical Data***

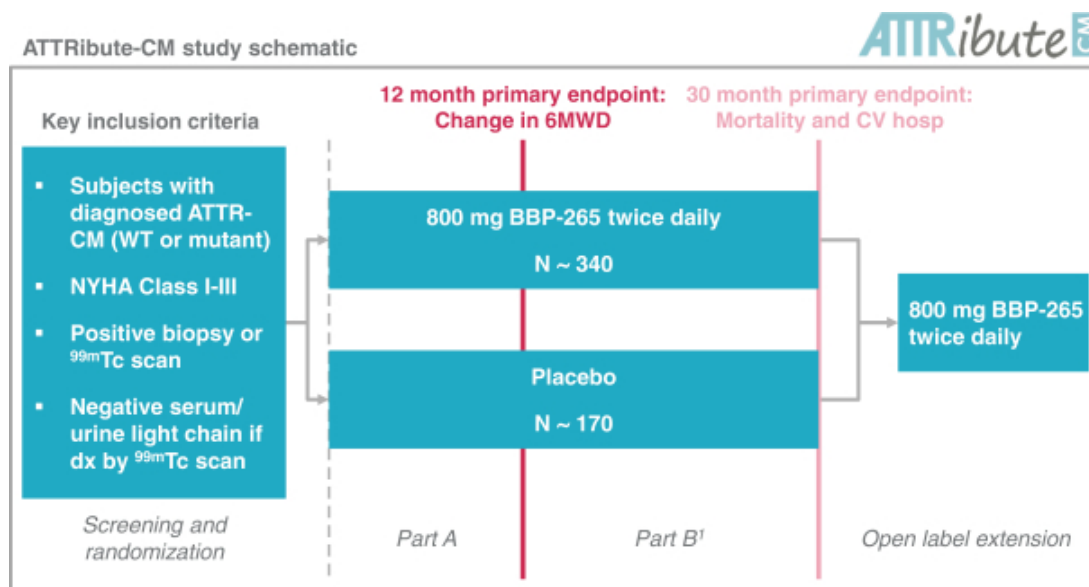
X-ray crystallography indicated that BBP-265 enables hydrogen bonding at the bottom of the thyroxine binding pocket of the TTR molecule, mimicking the structure of the naturally-occurring T119M rescue mutation. To our knowledge, BBP-265 is the only TTR stabilizer in clinical development or clinical use that has been observed in models to mimic this super-stabilizing mechanism of the naturally-occurring T119M rescue mutation.

Further, our preclinical studies in models support that BBP-265's binding to TTR may be highly specific and not significantly affected by the presence of additional plasma proteins. In contrast, published regulatory documents support that tafamidis also binds to the highly abundant plasma protein albumin, which competes with tafamidis' ability to bind and stabilize TTR. This is reflected in the free fraction observed for tafamidis (less than 0.5%) from the reported literature versus BBP-265 (3.6%) in our preclinical studies, suggesting that the percentage of total drug available for TTR binding may be greater for BBP-265 than for tafamidis at therapeutic concentrations.

In our preclinical studies using blood samples from ATTR patients, 10  $\mu$ M BBP-265 also resulted in greater than 85% TTR stabilization across a range of mutations that led to ATTRm-CM or ATTR-PN, which represent over 70% of all patients with mutation-driven ATTR.

**Clinical Development Plan**

In November and December of 2018, we met with the FDA to discuss a potential regulatory path for BBP-265 in ATTR-CM. Following these discussions, we initiated a randomized, global Phase 3 study of BBP-265 in ATTR-CM patients (ATTRibute-CM). ATTRibute-CM will enroll approximately 510 subjects with symptomatic ATTR-CM, including both wild-type and mutant TTR carriers with New York Heart Association Class I-III symptoms. Subjects will be randomized 2:1 between treatment (AG10 800 mg twice daily) and placebo. In Part A, change in six-minute walk distance (6MWD) at 12 months will be compared between treatment and placebo groups as a potential registrational endpoint. We anticipate reporting 12-month 6MWD data from Phase 3 ATTRibute study in patients with ATTR-CM in 2021. If the change in 6MWD is highly statistically significant, we anticipate submitting an NDA for ATTR-CM in 2021. In Part B, the study will continue for a total duration of 30 months, at which point all-cause mortality and cardiovascular hospitalizations will be compared between treatment and control groups. A schematic of the trial is shown below:



Secondary endpoints include: Kansas City Cardiomyopathy Questionnaire, serum TTR, TTR stabilization  
 As local standard of care evolves, concomitant use of approved, indicated therapies may be allowed

6MWD = Six minute walk distance; NYHA = New York Heart Association;  
<sup>99m</sup>Tc = Technetium labeled pyrophosphate (PYP) or bisphosphonate (e.g., DPD); dx = diagnosis;  
 CV hosp = cardiovascular-related hospitalizations

We believe the safety and tolerability data of BBP-265 in healthy volunteers and in ATTR-CM patients will also be relevant for the ATTR-PN patient population. Subject to authorization from applicable regulatory authorities, we plan to initiate a Phase 3 clinical development program for BBP-265 in ATTR-PN in 2019. We do not intend to file an IND with the FDA for this indication as we plan to conduct this study outside of the United States.

**Market Opportunity**

We believe that the total market for ATTR therapeutic interventions will continue to grow for the foreseeable future as the population of diagnosed patients increases as a result of heightened disease awareness and the adoption of non-invasive diagnostic techniques. As such, if BBP-265 is approved, we believe that there could be a significant population of newly diagnosed patients who can be treated with BBP-265 who have not previously been treated with a disease-modifying therapy.

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If approved, we believe that BBP-265 could have meaningful commercial potential. Further, we believe that BBP-265, if approved, may present advantages over other therapeutics based on the tolerability of BBP-265 observed in our clinical trials to date and its intended oral route of administration.

### **Key Competitors**

There are three primary therapeutic approaches being studied as treatments for ATTR: TTR knockdown, TTR stabilization and TTR clearance, each of which is expected to compete with BBP-265, if approved.

Among therapeutics that are designed to stabilize TTR, potential competitors include: Vyndamax (tafamidis) and Vyndaqel (tafamidis meglumine), which are approved in the United States and Japan for the treatment of ATTR-CM and which are approved in certain countries outside the United States for the treatment of ATTR-PN; SOM0226 (tolcapone, CRX-1008), being developed by Corino Therapeutics Inc.; and diflunisal, a generic, non-steroidal anti-inflammatory drug, or NSAID, that is approved for symptomatic treatment of mild to moderate pain, osteoarthritis and rheumatoid arthritis with a boxed warning for cardiovascular and gastrointestinal risk.

Among therapeutics that are designed to knock down TTR, potential competitors include: ONPATTRO (patisiran), currently marketed by Alnylam Pharmaceuticals, Inc., or Alnylam, for the treatment of ATTR-PN; vutrisiran, currently being developed by Alnylam; TEGSEDI (inotersen), which was developed by Ionis Pharmaceuticals, Inc. and is currently being marketed by Akcea Therapeutics, Inc. for the treatment of ATTR-PN; and preclinical compounds being developed by Intellia Therapeutics, Inc. and Arcturus Therapeutics, Ltd.

Among therapeutics targeting TTR clearance, potential competitors include PRX004, being developed by Prothena Therapeutics plc; and a recombinant human antibody for ATTR that is in preclinical development by Neurimmune Holding AG.

### **BBP-831/Infigratinib (QED): FGFR-Driven Cancers**

<b>Summary</b>	<ul style="list-style-type: none"><li>• We are developing infigratinib, an oral FGFR1-3 selective tyrosine kinase inhibitor, or TKI, for the treatment of FGFR-driven cancers</li></ul>
<b>Development Status and Catalysts</b>	<ul style="list-style-type: none"><li>• Ongoing – Preparation for NDA submission in advanced CCA, as a second-line or later therapy</li><li>• Ongoing – Investigator-initiated trial in certain cancers involving FGFR fusions or translocations</li><li>• 2019 – Anticipated first patient enrollment in Phase 3 clinical trial in advanced CCA as a first-line therapy</li><li>• 2020 – Planned initiation of Phase 3 clinical trial in adjuvant UC</li><li>• 2020 – Anticipated NDA filing for treatment of advanced CCA as a second-line or later therapy</li></ul>
<b>Disease Overview</b>	<ul style="list-style-type: none"><li>• CCA:<ul style="list-style-type: none"><li>– Rare, aggressive cancer of the bile ducts of the liver where the majority of newly diagnosed cases are non-resectable</li><li>– Incidence of approximately 37,000 in the United States and European Union. Approximately 15% to 20% of patients have FGFR2 fusions or translocations</li><li>– Standard of care is single-agent or combination chemotherapy</li></ul></li></ul>

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- UC:
    - Cancer of the lining of the urinary tract. Most patients with MIBC, and invasive UTUC, will have their tumors excised as a first line of treatment and are most likely to be candidates for adjuvant therapy. Upon resection, however, approximately 50% of cases recur within two years
    - Incidence of approximately 200,000 in the United States and European Union. In these territories, approximately 45,000 are MIBC and approximately 15,000 are invasive UTUC, which comprise our initial targeted indications. Approximately 15% to 20% of all patients with MIBC and approximately 50% to 60% of patients with invasive UTUC have FGFR3 genomic alterations
    - There is no standard of care for adjuvant treatment. Some patients may receive platinum-based regimens, but many are ineligible due to impaired renal function
  - FGFR genomic alteration status determined through standard genomic screening panels
  - Other FGFR-driven cancers:
    - Approximately 0.6% of all solid tumor cancers have fusions or translocations in the FGFR gene
    - FGFR fusions or translocations are particularly prevalent in gastric adenocarcinoma, glioma, carcinoma of unknown primary and endometrial carcinoma
  - Other FGFR-related syndromes
    - Tumor-induced ostomalacia, or TIO, is a rare paraneoplastic characterized by bone pain, fractures and muscle weakness. It is caused by tumoral overproduction of fibroblast growth factor 23 (FGF23), which in many cases can be caused by an FGFR1 fusion

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**Our Product Concept**

- Oral small molecule FGFR1-3 specific inhibitor
- Designed to abrogate signaling via the FGFR1-3 pathways and inhibit cancer growth in FGFR-driven cancers, including CCA and UC
- In Phase 1 and Phase 2 clinical trials, infigratinib has shown activity that we believe to be meaningful in clinical measures such as overall response rate, in advanced CCA with FGFR2 fusions or translocations and in UC with FGFR3 genomic alterations
- In advanced CCA, we believe infigratinib could be the first FGFR inhibitor approved for this indication
- In UC with FGFR3 genomic alterations, we believe infigratinib, if approved, could play an important role due to the lack of options for adjuvant treatment for patients who are ineligible or unfit to receive platinum-based therapy

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**Key Competitors**

- Pemigatinib, a small molecule FGFR inhibitor
- TAS-120, a small molecule FGFR inhibitor
- Derazantinib, a small molecule FGFR inhibitor

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**Disease Overview**

FGFRs are a family of genes that regulate multiple biological processes including cell proliferation, angiogenesis, and tissue repair. Amplifications, mutations, and fusions/translocations in FGFR genes are present

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in multiple cancers, and it is believed that they are key drivers in certain cancer types. FGFR genomic alterations have been shown to be present in approximately 7% of cancers. FGFR fusions or translocations, more specifically, have been shown to be present in approximately 0.6% of solid tumors.

Below is a table showing the frequency of certain FGFR genomic alterations in different tumor types:

Tumor type	Incidence (U.S. and EU)	Estimated Occurrence of FGFR genomic alterations*	Most common alteration(s)
<b>Cholangiocarcinoma</b>	20,000	15-20%	FGFR2 fusions or translocations
<b>Urothelial carcinoma</b>	200,000		
Non-muscle invasive bladder cancer	130,000	35-60%	FGFR3 mutations
Muscle invasive bladder cancer	41,000	15-20%	FGFR3 mutations
Non-invasive upper tract urothelial carcinoma	13,000	80-90%	FGFR3 mutations
Invasive upper tract urothelial carcinoma	19,500	50-60%	FGFR3 mutations
<b>Gastric adenocarcinoma</b>	40,000	10%	FGFR2 amplifications, FGFR2 fusions or translocations
<b>Glioma</b>	10,000	5%	FGFR3 fusions or translocations, FGFR1 amplifications
<b>Head and neck squamous cell carcinoma</b>	90,000	15%	FGFR1 genomic alterations
<b>Carcinoma of unknown primary</b>	20,000	5-10%	FGFR2/3 fusions or translocations
<b>Endometrial adenocarcinoma</b>	125,000	10-15%	FGFR2 fusions or translocations

\* Approximate percentages

### *Cholangiocarcinoma*

CCA is a rare and aggressive epithelial malignancy of the bile ducts of the liver. Approximately 20,000 new cases are diagnosed each year in the United States and European Union. The majority of newly diagnosed cases are non-resectable, meaning the malignancy cannot be removed completely through surgery. CCA, including resectable and non-resectable cases, has a median overall survival, or OS, between 20 and 28 months from diagnosis, and a five-year survival rate of approximately 25%.

Currently, no product has been specifically approved for the treatment of non-resectable CCA. Standard of care in locally advanced (i.e., non-resectable) and/or metastatic disease for first-line treatment is platinum-based chemotherapy, which has median with a progression-free survival, or PFS, of approximately eight months and an OS of approximately 12 months. Approximately 85% of these patients will move on to receive a second-line of treatment.

Second-line treatment for advanced and/or metastatic CCA is alternative single or combination agent chemotherapy; however, second-line chemotherapy has shown only single-digit response rates on average. In a comprehensive review of 25 studies, median PFS was 3.2 months and overall response rate, or ORR, was 7.7% for patients receiving second-line treatment. As a result, the National Comprehensive Cancer Network, or NCCN, guidelines for the treatment of CCA currently do not recommend any specific regimen for second-line treatments. Further, there are currently no targeted therapies approved for the treatment of CCA.



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### *Urothelial Carcinoma*

UC is a cancer of the lining of the urinary tract with approximately 200,000 new cases diagnosed each year in the United States and European Union.

UC can be categorized as bladder cancer, or BC, and upper tract urothelial carcinoma, or UTUC. In BC, patients are typically segmented into muscle-invasive, or MIBC, and non-muscle invasive, or NMIBC disease. UTUC, in turn, can be classified as invasive or non-invasive disease. We are initially focused on developing infigratinib for the 45,000 MIBC and 15,000 invasive UTUC cases that occur annually in the United States and European Union.

Patients that present with MIBC or invasive UTUC are typically candidates for surgical resection, specifically radical cystectomy or radical nephroureterectomy, respectively, as an initial treatment. However, upon resection, approximately 50% of cases will recur within two years of surgery. Following surgical resection there is no standard of care for adjuvant treatment, especially for cisplatin ineligible patients. There are limited clinical data suggesting that cisplatin-based adjuvant regimens may increase disease-free survival. And, as renal function is impaired in many patients due to age and surgical removal of the bladder, ureter and/or kidney, many patients are not candidates for cisplatin-based therapy. Data suggests that approximately 40% to 50% of MIBC patients and 70% to 80% of invasive UTUC patients are cisplatin ineligible after radical cystectomy and radical nephroureterectomy, respectively.

### ***Our Product Concept***

Signaling via FGFR genes is thought to be a key driver of certain cancers, including CCA and UC. As an FGFR1-3 specific inhibitor, infigratinib abrogates signaling via the FGFR1-3 pathways, interfering with oncogenic signaling and cancer growth.

Infigratinib has shown clinical activity in advanced and/or metastatic CCA with FGFR2 fusions or translocations, with an ORR of 26.9%, in a Phase 2 clinical trial as described below. In advanced and/or metastatic CCA, limited treatment options make FGFR-directed therapies particularly attractive potential treatment options.

Infigratinib has also shown clinical activity in advanced and/or metastatic UC with FGFR3 genomic alterations in a Phase 1 expansion cohort, with an ORR of 25.4%, as described below. The majority of patients with invasive UC undergo surgical resection; however, there is no standard of care for adjuvant treatment post-surgery, especially for cisplatin-ineligible patients. While there is limited evidence for the use of cisplatin-based chemotherapy as an adjuvant treatment, many patients are cisplatin ineligible due to poor renal function. We believe that infigratinib could play a meaningful role as an adjuvant treatment for patients with UC driven by FGFR3 genomic alterations.

### ***Clinical Data***

Infigratinib has been studied in ten clinical trials that include four Phase 1 clinical trials in healthy volunteers, three Phase 1 clinical trials in cancer patients, and three Phase 2 clinical trials in certain cancer patients, and has demonstrated clinical proof of concept in CCA and UC. To date, infigratinib has been tested in over 600 subjects, including healthy volunteers and cancer patients, and has demonstrated acceptable tolerability.

In the studies described below, the following revised RECIST guideline version 1.1, or RECIST 1.1, criteria, which are the accepted criteria by the scientific community for the tumor type discussed, were used to define responses:

- ORR, which is defined as the proportion of patients with a best overall response of partial response, or PR, plus those with complete response, or CR.
- Response duration, which is measured from the time of initial response until documented tumor progression.

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- Disease control rate, or DCR, which is defined as the proportion of patients who have achieved a best overall response of CR, PR, or stable disease, or SD.
- Progression free survival, or PFS, which is defined as the time from randomization/start of treatment until objective tumor progression or death, whichever occurs first.
- Overall survival, or OS, which is defined as the time from randomization until death from any cause.

### CCA Phase 2 Clinical Trial

Infigratinib is being studied in an open-label, single-arm, Phase 2 clinical trial in patients with advanced and/or metastatic CCA, referred to as the '2204 trial. The study initially enrolled patients with any FGFR genomic alterations and was later amended to enroll only patients with FGFR2 fusions or translocations, who represented patients showing the strongest response. To date, we have reported interim data in 71 CCA patients with FGFR2 fusions or translocations, who had previously received a cisplatin-and gemcitabine-containing regimen, or a gemcitabine-containing regimen (for those who are considered intolerant to cisplatin) and are continuing to enroll patients in the trial. Patients received infigratinib 125 mg once daily for 21 days followed by seven days off in 28-day cycles until disease progression. The primary endpoint of the study is ORR. Secondary endpoints include PFS, best overall response, or BOR, DCR, OS, safety and PK. The median age of enrolled subjects is 53 years, 62.0% are female, 100.0% are FGFR2 fusion or translocation positive, and 7.0% have co-existing FGFR2 mutations. A significant majority of patients enrolled in the trial were pretreated, with 65.1% having received at least two prior lines of antineoplastic therapy.

At an interim analysis based on a data cut-off date of August 8, 2018, we observed the following results for FGFR2 fusion or translocation positive patients. The data presented in the table below are based on patients with potential for confirmation (n=67; patients who had completed or discontinued prior to six cycles). All responses were investigator-assessed.

ORR, % (95% CI)	26.9 (16.8-39.1)
ORR in patients receiving prior lines of treatment, %	
<sup>‡</sup> 1 (n=28)*	39.3
<sup>‡</sup> 2 (n=39)	17.9
BOR <sup>‡</sup> (confirmed and unconfirmed PRs)*, %	32.8
DCR, % (95% CI)	83.6 (72.5-91.5)
Median duration of response, months (95% CI)	5.4 (3.7-7.4)
Median PFS, months (95% CI)	6.8 (5.3-7.6)
Median OS, months (95% CI)	12.5 (9.9-16.6)

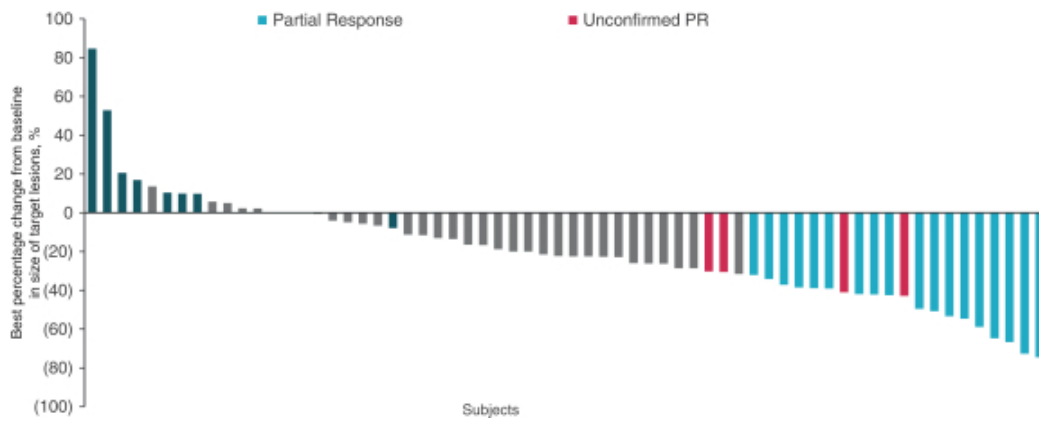
\* Three patients received no prior systemic therapy for advanced or metastatic CCA

<sup>‡</sup> BOR defined per RECIST1.1: patients with one scan with greater than 30% change from baseline in target lesions, without confirmation from a second scan

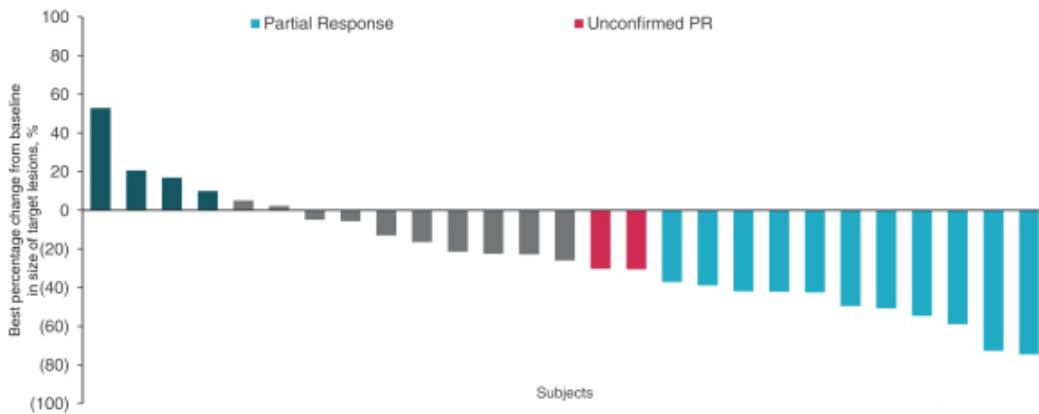
An intention-to-treat analysis was also performed. In the intention-to-treat population (n=71), ORR was 25.4% and BOR was 31.0%.

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A waterfall chart showing the change in target lesion size for patients in this trial as of the interim analysis date follows (n=64; patients with potential for confirmation and who had both baseline and one post-baseline assessment available at time of analysis). This chart shows both the change in tumor size, as well as best response per RECIST 1.1 guidelines, providing a visual representation of patient responses to infigratinib treatment:



The ORR as of the interim analysis was observed to be higher in the subsegment of patients who had received only one prior line of therapy (39.3% versus 26.9% for all patients). A waterfall chart showing the change in target lesion size for patients in this subsegment follows (n=27; patients who had received one or fewer prior lines of systemic therapy for treatment of advanced or metastatic CCA and with potential for confirmation and who had both baseline and one post-baseline assessment available at time of analysis). This chart shows both the change in tumor size, as well as response per RECIST 1.1 guidelines, providing a visual representation of patient responses to infigratinib treatment:



*Urothelial Carcinoma Phase 1 Clinical Trial Expansion Cohort*

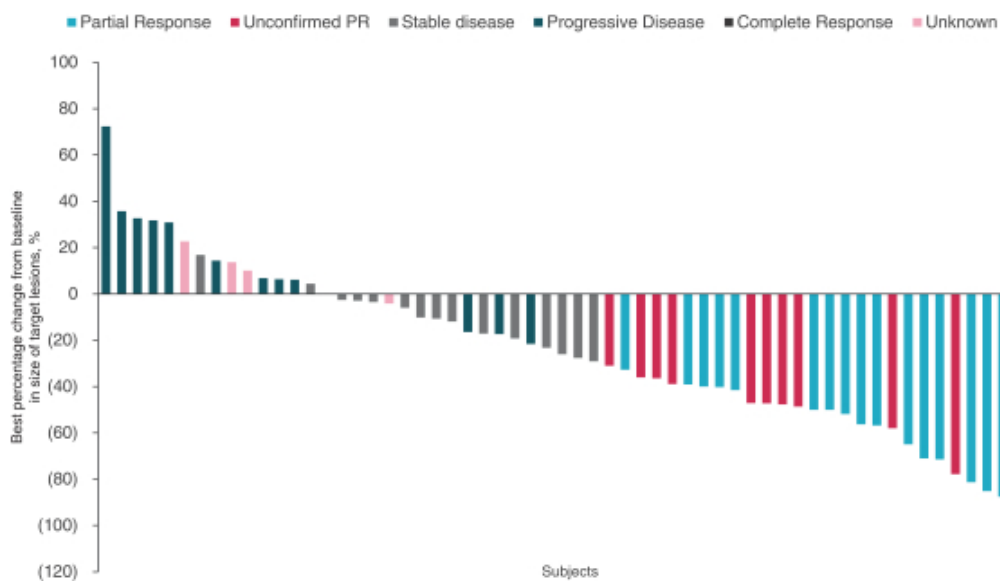
In a Phase 1 open-label, single arm expansion cohort of the '2101 study, patients with UC harboring FGFR3 genomic alterations (n=67) received infigratinib 125 mg once daily for 21 days followed by seven days off in 28-day cycles until progression. Patients enrolled in the trial had a median age of 67 years, and 68.7% were male. 92.5% of patients had FGFR3 mutations and 7.5% had FGFR3 fusions or translocations. The primary objective of the '2101 study was to determine the maximum tolerated dose, or MTD, and thus the recommended Phase 2

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clinical trial dose and schedule of single agent oral BGJ398 in patients with advanced solid tumors. The key secondary objective of the expansion cohort in this study was to assess preliminary anti-tumor activity in patients treated with infogratinib. Other secondary objectives included safety, tolerability and PK analyses. Patients enrolled in the trial were heavily pre-treated, 70% of enrolled patients having received two or more prior lines of therapy. The endpoint assessment of patients from this trial follows:

ORR, %	25.4 (15.5-37.5)
BOR (confirmed and unconfirmed PRs), %	41.8
DCR, %	64.2 (51.5-75.5)
Median duration of response, months (95% CI)	5.6 (2.3-11.0)
Median PFS, months (95% CI)	3.8 (3.1-5.4)
Median OS, months (95% CI)	7.8 (5.7-11.9)

A waterfall chart highlighting the best percentage change from baseline in size of target lesions in this trial follows (n=60; patients with potential for confirmation and who had both baseline and one post-baseline assessment available at time of analysis). This chart shows both the change in tumor size, as well as response per RECIST 1.1 guidelines, providing a visual representation of patient responses to infogratinib treatment:



Of the 67 patients treated in this expansion cohort, eight were diagnosed as having UTUC. In these eight patients, ORR was 50% and DCR was 100%.

*Safety Data*

Infigratinib has been studied in over 600 patients to date, including 134 healthy volunteers, 421 oncology patients treated with infigratinib monotherapy, and 62 oncology patients treated with infigratinib in combination with BYL719, a phosphoinositide 3-kinase, or PI3K inhibitor. To date, at the dose of 125mg daily (three weeks on, one week off), the dose being used in our ongoing and planned Phase 2 and Phase 3 clinical trials, infigratinib has shown acceptable tolerability with expected on-target class effects. The table below show safety data for all oncology patients (n=421) exposed to infigratinib monotherapy across all studies, dosing levels, and dosing schedules, and provides a summary of the most frequently observed AEs in 25.0% of oncology patients:

	<u>Hyperphosphatemia</u>	<u>Fatigue</u>	<u>Constipation</u>	<u>Stomatitis</u>	<u>Decreased appetite</u>	<u>Nausea</u>	<u>Diarrhea</u>	<u>Alopecia</u>
All Grades	61.5%	40.9%	37.1%	33.7%	30.2%	29.0%	27.3%	26.8%
Grade 3+	6.9%	5.4%	1.0%	4.0%	2.9%	2.9%	2.1%	0.0%

The most commonly reported treatment emergent adverse event of any grade was hyperphosphatemia, occurring in 61.5% of patients. Hyperphosphatemia is an on-target AE based on FGFR1 inhibition. Other frequently reported AEs included fatigue (40.9%), constipation (37.1%), stomatitis (33.7%), decreased appetite (30.2%), nausea (29.0%), diarrhea (27.3%), and alopecia (26.8%).

In the ‘2101 trial, a Phase 1 open-label dose-escalation study of 208 patients with advanced solid malignancies across multiple tumor types at multiple doses, eight grade 3 or 4 AEs (30.8% of grade 3 or 4 AEs observed in this study) were suspected to be related to study treatment, with the most common events being hypophosphatemia (7.7%) and lipase increase (7.7%). This is generally consistent with the number of grade 3 or 4 AEs related to study treatment across all studies to date. The following SAEs in the ‘2101 trial were adjudicated as being related to infigratinib: cataract, constipation, abdominal pain, hyperphosphatemia, hypophosphatemia (each reported in two patients, or 1.0% of patients); and hypercalcemia (reported in three patients, or 1.5% of patients).

In a Phase 1 expansion cohort of UC patients in the ‘2101 trial, 70.1% of patients discontinued due to progressive disease, 14.9% due to AEs, 3.0% due to withdrawal of consent, 1.5% due to loss to follow-up, and 1.5% due to death. 35.8% of patients experienced SAEs and 6.0% experienced SAEs suspected to be related to study treatment.

In an ongoing Phase 2 open-label, single arm study for patients with advanced or metastatic CCA and FGFR2 gene fusions or translocations, or the ‘2204 trial, as of August 8, 2018, 61.4% of patients had discontinued due to progressive disease, 9.6% due to AEs, 8.4% due to physician decision, 4.8% due to withdrawal of consent, 1.2% due to loss to follow-up, 2.4% due to death, and 1.2% for unknown reasons. As of August 8, 2018, 10.8% of patients had ongoing treatment. 39.4% of patients with FGFR2 fusions or translocations experienced SAEs and 11.3% experienced SAEs suspected to be related to study treatment.

In the dose-escalation cohort of the Phase 1 clinical trial, the maximum tolerated dose, or MTD, for infigratinib was found to be 125 mg/ day, which is approximately 2 mg/kg. Per further investigation of tolerability, safety, PD and PK, an alternative dosing schedule, i.e. three weeks on/one week off, at 125 mg was explored. The three weeks on/one week off at 125mg was declared as the recommended Phase 2 dose, or RP2D, by Novartis.

In total, 37.2% (n=157/422) of oncology patients across all trials with monotherapy treatment experienced SAEs. SAEs occurring in greater than 1.0% of patients included dyspnea (2.4%), general physical health deterioration (2.4%), pyrexia (2.1%), sepsis (2.1%), vomiting (2.1%), abdominal pain (1.9%), pneumonia (1.9%), anemia (1.7%), hypercalcemia (1.7%), acute kidney injury (1.4%), constipation (1.4%), dehydration (1.4%), nausea (1.4%), and urinary tract infection (1.4%).

In total, 41.9% (n=26/62) of oncology patients treated with combination treatment experienced SAEs. SAEs occurring in greater than 1.0% of patients included pyrexia (6.5%), general physical health deterioration (3.2%),

pleural effusions (3.2%), stomatitis (3.2%), diarrhea (3.2%), deep vein thrombosis (3.2%), dyspnea (1.6%), anemia (1.6%), constipation (1.6%), nausea (1.6%), dysphasia (1.6%), hyponatremia (1.6%), muscular weakness (1.6%), pain in extremity (1.6%), septic shock (1.6%), small intestinal obstruction (1.6%), lower abdominal pain (1.6%), colostridium difficile colitis (1.6%), headache (1.6%), peripheral edema (1.6%), tumor pain (1.6%), hematoma (1.6%), hyperglycemia (1.6%), hypersensitivity (1.6%), iliac vein occlusion (1.6%), malignant neoplasm progression (1.6%), peripheral swelling (1.6%), peroneal nerve palsy (1.6%), maculopapular rash (1.6%), rectal hemorrhage (1.6%), renal failure (1.6%), skin infection (1.6%), ureteric obstruction (1.6%), and wound decomposition (1.6%).

In healthy volunteers (n=134), no SAEs were reported.

### *Potential Additional Indications*

As demonstrated in early clinical data, treatment with infigratinib monotherapy has shown promising activity across multiple tumor types with FGFR fusions or translocations, including glioblastoma, gallbladder cancer, and carcinoma of unknown primary. Further, preclinical data suggest that infigratinib is likely to be active in gastric cancer with FGFR activating mutations. Many of Novartis' trials of infigratinib monotherapy did not differentially enroll patients by the type of genomic alteration present in tumors. Based on available data, we believe that tumors harboring specific genomic alterations, including activating mutations, fusions, or translocations are the most likely to be drivers of cancer, and are thus the most likely to be responsive to infigratinib monotherapy. As we consider development of infigratinib monotherapy in indications outside of CCA and UC, we are focusing our development in indications with these specific genomic alterations. We believe that some of the negative results obtained in the Novartis trials, as discussed below, were driven by selection of patients with FGFR amplifications and non-activating mutations, which are genomic alterations that, we believe, may not be key drivers of cancer.

Preclinical data also provide proof-of-concept that infigratinib may provide synergistic efficacy in combination with other anti-cancer agents in indications including: breast cancer in combination with VEGFR inhibitors, and non-small cell lung cancer, or NSCLC, in combination with EGFR inhibitors or MEK inhibitors. In an FGFR1 amplified breast cancer mouse model, *in vivo* synergy with ZD6474, a VEGFR inhibitor, was shown in relation to tumor suppression. In PC9 xenograft mouse models, infigratinib combination with gefitinib (an EGFR inhibitor) prevented development of *in vivo* resistance. Further, dual inhibition with infigratinib and gefitinib suppressed *in vitro* outgrowth of EGFRi-tolerant persister clones. Finally, upfront combination with trametinib forestalled resistance driven by FGFR1 in NCI-H520 (FGFR1 amplified squamous cell carcinoma), DMS114 (FGFR1 amplified small cell lung cancer), and AN3 CA (FGFR2 mutated endometrial carcinoma) cell lines. We believe these preclinical data provide a rationale for potential combination trials with infigratinib that are currently being explored.

### *Other FGFR-related syndromes: tumor-induced ostomalacia*

Tumor-induced ostomalacia, or TIO, is a rare paraneoplastic disease characterized by bone pain, fractures and muscle weakness. It is caused by tumoral overproduction of fibroblast growth factor 23, or FGF23, which acts on the proximal renal tubule, and decrease phosphate reabsorption and 1 $\alpha$ -hydroxylation of 25 hydroxyvitamin D, leading to hypophosphatemia and osteomalacia. In up to 60% of cases, a fibronectin-1(FN1)-FGFR1 fusion has been identified that may serve as the primary driver of the syndrome. As a result, we believe an FGFR inhibitor, such as infigratinib, may have benefit in TIO.

Infigratinib is being studied in an ongoing Phase 2, open-label, non-randomized, single-arm, single-site, investigator initiated trial. The primary objective of the study is to induce a complete metabolic response in patients with TIO, as demonstrated by normalization of FGF23 and phosphate homeostasis. Patients will be dosed, initially, with 75mg per day, which will be escalated up to 125mg daily dependent upon on patient response. To-date two patients with TIO have been enrolled in the study and were treated with infigratinib, and

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we expect to enroll up to 10 patients in this clinical trial. Both patients demonstrated normalization or near normalization of FGF23 and normalization of phosphate levels after treatment.

### *Other Clinical Trials*

The trials of infigratinib conducted with the most advanced clinical data are the '2101 and '2204 trials discussed above, and two other Phase 2 clinical trials, the '2201 and 'US04 trials. In certain cohorts in the '2101, '2201 and 'US04 trials, low rates of response were observed, which led to a decision to cease developing infigratinib in these programs. It should be noted that '2101, '2201, and 'US04 trials enrolled patients with all FGFR genomic alterations. While the '2204 trial initially enrolled patients with all forms of FGFR genomic alterations, it was subsequently amended to enroll patients only with FGFR2 fusions or translocations.

- In the '2101 trial, the indications with the largest number of patients were squamous non-small cell lung cancer, or sqNSCLC, (n=36), breast cancer (n=32), and UC (n=75, including 67 patients in a Phase 1 expansion cohort). sqNSCLC and breast cancer patients predominantly displayed FGFR1 amplifications, whereas UC patients exclusively displayed FGFR3 mutations. In this trial, ORR was observed to be 11.1% in sqNSCLC, 0.0% in breast cancer, 37.5% in UC patients in the first part of the Phase 1 study (n=8), and 25.4% in UC patients in the Phase 1 expansion cohort (n=67). ORR across all indications for patients at the MTD or RP2D (n=173) was 13.9%, and DCR was 49.1%. Median PFS for this group was 3.1 months (95% CI: 2.1-3.7 months). OS was not collected for the entire cohort of patients and is only available for the UC expansion cohort, which was 7.8 months (95% CI: 5.7-11.6 months), as reported above.
- '2201 was a Phase 2 open-label, single arm study for patients with recurrent resectable or unresectable glioblastoma performed by Novartis. Twenty-six patients with FGFR genomic alterations, predominantly amplifications and mutations, were enrolled in the study and responses were seen in two patients. ORR was 7.6% and DCR was 30.8%. Data is still to be analyzed and median PFS and OS are not available.
- 'US04 was a Phase 2 open-label, single arm study in patients with pre-identified FGFR-genomic alterations and solid tumors and/or hematologic malignancies performed by Novartis. Eighty-five patients, the majority of whom had FGFR amplifications or mutations, were enrolled with multiple tumors. At the last interim analysis, ORR was 7.5% and clinical benefit rate, or CBR, was 15.0% across all indications. Median PFS was 1.8 months (95% CI: 1.8-2.0 months) and median OS was 6.2 months (95% CI: 4.4-9.8 months).
- The ongoing '2204 trial was initiated by Novartis and is now being conducted by us in patients with CCA with FGFR2 fusions or translocations. At an interim analysis with a cut-off date of August 8, 2018, ORR was 26.9% and DCR was 83.6% in those patients with potential for confirmation. Other efficacy measures are reported above.

### *Clinical Development Plans*

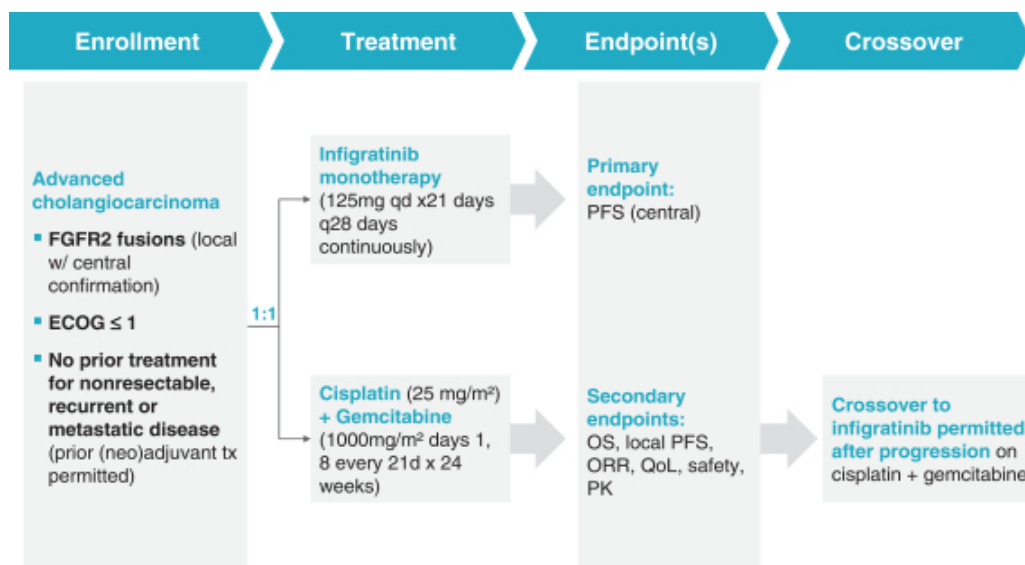
#### *Advanced CCA*

We intend to file an NDA with the FDA for second line and later advanced CCA with FGFR2 fusions or translocations in 2020 with the data that have been generated to date from clinical trials of infigratinib, following meetings with the FDA in 2019, including a planned pre-submission meeting with the Center for Drug Evaluation and Research and the Center for Devices and Radiological Health in mid-2019 to discuss analytical validation and clinical bridging to support the marketing authorization pathway for the companion diagnostic we are developing with FMI. We believe that approval of this companion diagnostic via the premarket approval, or PMA, pathway will be required for approval of an NDA for infigratinib; however, based on our interactions with the FDA to date, we do not expect to have to perform any additional clinical work specifically related to the companion diagnostic we are developing with FMI. We expect to continue to enroll approximately 20 additional subjects in the ongoing CCA Phase 2 clinical trial ('2204). Subject to additional communications with the FDA, we do not expect that data from these patients will be necessary to file our NDA.

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*First-line CCA*

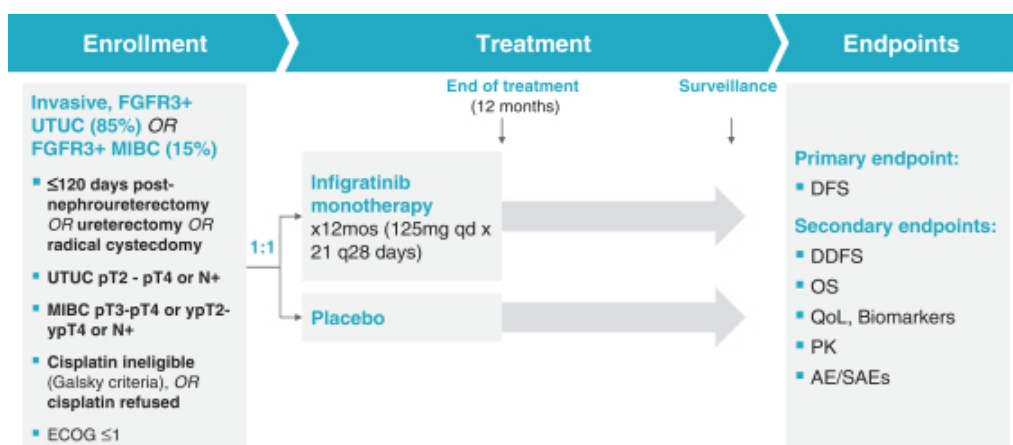
We anticipate enrolling the first patient in a Phase 3 randomized, open-label clinical trial of infigratinib as a first-line therapy for CCA compared to gemcitabine and cisplatin in advanced and/or metastatic CCA with FGFR2 fusions or translocations in 2019. The trial design for this study follows, with a target enrollment of approximately 350 patients globally:



ECOG=Eastern Cooperative Oncology Group, a simple measure of functional status; DDFS=distant disease-free survival; QoL=quality of life

*Adjuvant Urothelial Carcinoma*

We expect to initiate a Phase 3 randomized, double-blind, placebo controlled clinical trial in cisplatin-ineligible adjuvant UC with FGFR3 genomic alterations in 2020. The trial design for this study follows with a target enrollment of approximately 250 patients globally:





### *Other Cancers with FGFR Fusions or Translocations*

We are exploring potential clinical development paths for infigratinib in additional FGFR fusion or translocation-driven cancers, as we believe that fusions or translocations are the most likely FGFR genomic alterations to be sensitive to infigratinib monotherapy, based on available data. To date, infigratinib has shown responses in FGFR fusion or translocation-driven CCA and UC, as well as gallbladder cancer, carcinoma of unknown primary, and glioblastoma. An investigator initiated trial has been initiated at the Ohio State University to study infigratinib in patients with multiple tumor types exhibiting FGFR fusions or translocations to further explore the activity of infigratinib in FGFR fusion or translocation-driven solid tumors. Expected enrollment in the trial is approximately 50 patients. Based on data generated from this investigator-initiated trial, as well as ongoing translational research, we plan to implement a development strategy for a tumor agnostic approach, i.e., pursuing approval based on a biomarker rather than a specific cancer indication.

### **Key Competitors**

There are eight other FGFR targeted assets currently known to be in clinical development. These product candidates have not been compared with infigratinib in head-to-head studies. However, efficacy and tolerability data of competitive compounds appears to be in-line with the data from clinical studies of infigratinib.

Key competitors include pemigatinib, an FGFR TKI under Phase 2 and Phase 3 clinical development by Incyte Corporation, TAS-120, an FGFR TKI under Phase 2 clinical development by Taiho Oncology, Inc., derazantinib, an FGFR TKI under Phase 2 and Phase 1/2 clinical development by ArQule, Inc. in collaboration with Basilea Pharmaceutica International Limited, erdafitinib, an FGFR TKI under development by Janssen Pharmaceuticals, Inc. for which an NDA has been submitted, vofatamab, an FGFR3 monoclonal antibody under Phase 1/2 clinical development by Rainier Therapeutics, Inc. and rogaratinib, an FGFR TKI under Phase 2/3 clinical development by Bayer AG.

### **BBP-831/Infigratinib (QED): Achondroplasia**

<b>Summary</b>	<ul style="list-style-type: none"><li>We are developing infigratinib, an oral FGFR1-3 selective TKI in preclinical development for the treatment of achondroplasia at a significantly lower dose than those doses studied in our oncology programs for infigratinib</li></ul>
<b>Development Status and Catalysts</b>	<ul style="list-style-type: none"><li>2020 – Planned IND submission and initiation of Phase 1/2 clinical trial</li><li>2021 – Anticipated reporting of clinical proof-of-concept data from Phase 1/2 clinical trial</li></ul>
<b>Disease Overview</b>	<ul style="list-style-type: none"><li>Achondroplasia is the most common form of disproportionate short stature</li><li>All cases are driven by autosomal dominant FGFR3 gain of function mutations. This leads to downstream signaling through both the MAPK and STAT1 pathways, which impacts hypertrophic differentiation and chondrocyte proliferation, respectively</li><li>Prevalence of greater than 55,000 in the United States and European Union, incidence of one in 10,000 to 30,000 live births worldwide</li><li>Diagnosis: often suspected prenatally based on shortened long bones and macrocephaly on ultrasound and confirmed by molecular testing after birth. No disease modifying treatments are currently approved in the United States or European Union</li></ul>
<b>Our Product Concept</b>	<ul style="list-style-type: none"><li>Oral small molecule FGFR1-3 specific inhibitor</li><li>Has the potential to treat the disease at its source by reducing FGFR3 downstream signaling. Unlike CNP mimetics, this approach also inhibits downstream STAT1 signaling</li><li>Preclinical data have demonstrated proof of concept in a mouse model of achondroplasia at doses significantly below those doses studied in our oncology clinical trials</li></ul>

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- Key Competitors**
- Vosoritide, a CNP mimetic
  - Transcon CNP, a long-acting CNP mimetic
  - TA-46, a FGFR ligand trap
- 

### ***Disease Overview***

Achondroplasia is the most frequent cause of disproportionate short stature, and FGFR3 mutations have been shown to be the molecular source of the condition. Achondroplasia has a prevalence of greater than 55,000 in the United States and European Union, and an estimated worldwide incidence of one in 10,000 to 30,000 live births. The condition leads to a disproportionate short stature with anomalies in bone development and potential for foramen magnum stenosis, spinal stenosis, cardiovascular complications and obesity. The average height is approximately 4'4" for a male and 4'1" for a female with achondroplasia. Lifespan and intelligence are most often normal.

Achondroplasia is an autosomal dominant condition caused by a gain-of-function point mutation in the FGFR3 gene. Approximately 97% of cases are due to G380R substitution and 80% of cases are the result of de novo mutations. FGFR3 is expressed in osteoblasts and chondrocytes where it plays a critical role in regulating bone growth through the MAPK pathway, which drives hypertrophic differentiation, and through the STAT1 pathway, which drives chondrocyte proliferation. Apart from growth hormones, which are approved in Japan, we are not aware of any other medicines approved for marketing by the FDA or the EMA for the treatment of achondroplasia.

### ***Our Product Concept***

FGFR3 gain-of-function mutations are the driver behind the pathophysiology of achondroplasia. As an FGFR1-3 inhibitor, we believe that infigratinib has the potential to decrease pathologic signaling downstream of FGFR3 and treat achondroplasia at its source.

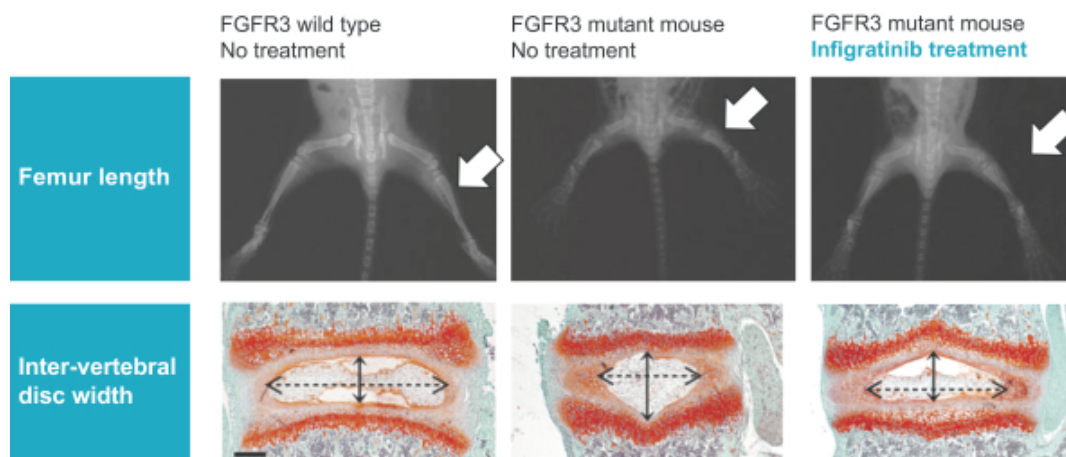
Preclinical proof of concept has been demonstrated in an achondroplasia mouse model at dose levels as low as 2% of those used in our oncology trials. In our Phase 1 dose escalation clinical trials of infigratinib, we saw acceptable tolerability, including no instances of hyperphosphatemia, at three to six times the expected dose level in our achondroplasia trials. Based on these results, we do not expect significant tolerability issues at the proposed dose level in the clinic.

### ***Preclinical Data***

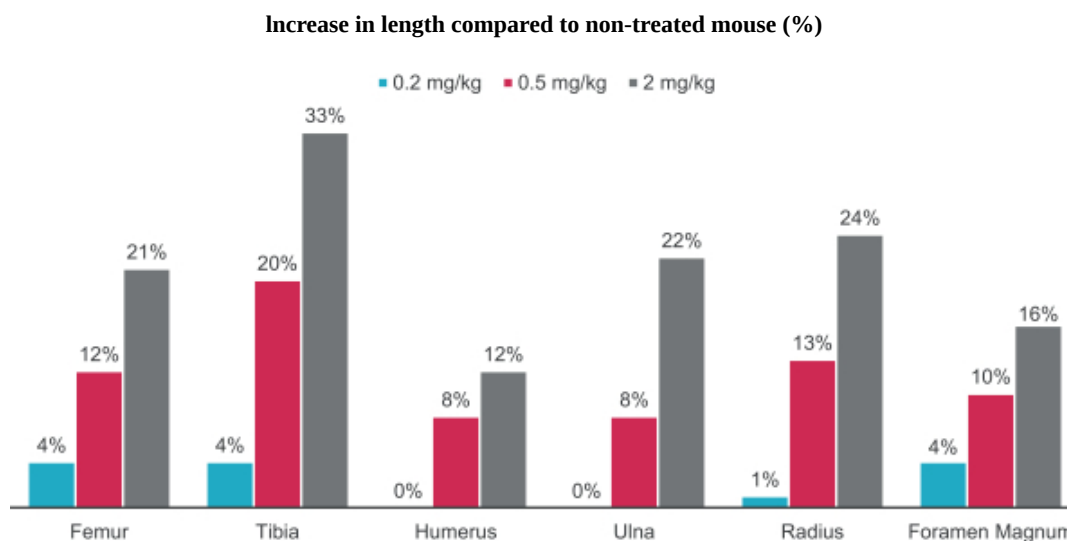
Infigratinib has been studied preclinically in a mouse model of achondroplasia that recapitulates anomalies of the growth plates, vertebrae, and intervertebral discs. Investigators observed that infigratinib rescued *ex vivo* bone growth of mutant mouse embryo femurs after six days of treatment. Further, 15 days of treatment showed *in vivo* bone growth, which mimics human achondroplasia in many respects. Effects on both appendicular and axial skeletal parameters were observed in this study.

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Below are figures demonstrating the extent of femur growth and intervertebral disc width rescue in wild-type, untreated model, and infigratinib treated (2 mg/kg) model mice:



*In vivo* bone growth was further demonstrated at lower doses (0.2 mg/kg and 0.5 mg/kg) by the same laboratory. Together, preclinical studies at all doses have demonstrated meaningful increases in skeletal growth parameters between treated and untreated mutant mice, as follows:



Notably, treatment with infigratinib did not modify the expression of FGFR1 in the hypertrophic zone of the growth plate. The effects seen were mainly due to FGFR3 inhibition, with no other gross side effects being observed in these preclinical studies.

### Clinical Development Plan

Subject to the completion of ongoing juvenile and chronic toxicity studies, we expect to file an IND for infigratinib in achondroplasia and initiate a Phase 1/2 clinical trial in 2020. The Phase 1/2 clinical trial is designed as an open-label, dose-escalation and expansion trial in children with achondroplasia prior to growth

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plate closure. The primary objective of this study will be to assess safety and tolerability in children with achondroplasia. Secondary objectives will include PK analyses, change in growth velocity, and assessment of quality of life.

### Key Competitors

Infigratinib is the only direct FGFR1-3 inhibitor that has been publicly disclosed in development for the treatment of achondroplasia. There are three other identified companies developing compounds for the treatment of achondroplasia using alternative mechanistic approaches: BioMarin Pharmaceutical Inc. (vosoritide), Ascendis Pharma A/S (TransCon CNP), and Therachon AG (TA-46) (recently acquired by Pfizer Inc.). Preclinical data for all of the compounds is shown below.

<b>Compound Company</b>	<b>Vosoritide</b>	<b>TransCon CNP</b>	<b>TA-46</b>
<b>Mechanism of Action</b>	BioMarin Pharmaceutical Inc. <ul style="list-style-type: none"><li>• CNP analogue</li><li>• Designed to reduce signaling via MAPK pathway</li></ul>	Ascendis Pharma A/S <ul style="list-style-type: none"><li>• Long-acting CNP analogue</li><li>• Designed to reduce signaling via MAPK pathway</li></ul>	Therachon AG <ul style="list-style-type: none"><li>• FGFR-ligand trap</li><li>• Designed to reduce signaling via both MAPK and STAT1 pathways</li></ul>
<b>Route of Administration</b>	Subcutaneous	Subcutaneous	Subcutaneous
<b>Status</b>	Phase 3 ongoing	Phase 1 in healthy subjects complete	Phase 1 in healthy subjects ongoing
<b>Tolerability</b>	Injection site reactions; hypotension	Well-tolerated in clinical studies	Undisclosed
<b>Reported preclinical increase in tibia length*</b>	6.6%	12.3%	8.6%
<b>Reported preclinical increase in femur length*</b>	5.2%	Undisclosed	6.2%

\* Preclinical data from vosoritide, TransCon CNP is from FGFR3<sup>Y367C/+</sup> mouse model; TA-46 is from the FGFR3<sup>ACH/+</sup> mouse model; comparisons are from different preclinical experiments, including differences in protocols and in reported efficacy, and may not be directly comparable.

### BBP-631 (Adrenas): Congenital Adrenal Hyperplasia

<b>Summary</b>	<ul style="list-style-type: none"><li>• We are developing BBP-631, a preclinical AAV, gene transfer product candidate, for the treatment of CAH, caused by 21OHD. BBP-631 was granted orphan drug designation from both FDA and EMA in 2018.</li></ul>
<b>Development Status and Catalysts</b>	<ul style="list-style-type: none"><li>• Ongoing – Nonhuman primate studies, preparation for GLP toxicity studies</li><li>• 2020 – IND submission anticipated</li><li>• 2021 – Anticipated reporting of clinical proof-of-concept data</li></ul>
<b>Disease Overview</b>	<ul style="list-style-type: none"><li>• CAH is a debilitating, life-threatening disease defined by an inability to produce the steroids cortisol and aldosterone, and an excess production of testosterone. Complications include adrenal crises, dehydration, and virilization in females. The “classic” form with the most severe phenotype can be categorized into salt-wasting (75%) and simple virilizing (25%), with the former demonstrating only 0-1% of residual enzyme activity</li><li>• Incidence of approximately 1/20,000 births worldwide for the salt-wasting form, incidence of approximately 1/60,000 births worldwide for the simple virilizing form, and a combined prevalence estimated to be greater than 75,000 in the United States and EU. Over 90% of cases are caused by inactivating mutations in 21OH, with autosomal recessive inheritance</li><li>• Diagnosis: Newborn screening for 21OHD is conducted in every U.S. state and most European countries by measuring 17<math>\alpha</math>-hydroxyprogesterone</li></ul>

- Standard of care consists of chronic supraphysiologic doses of exogenous steroids (corticosteroids and mineralocorticoids) leading to significant side effects

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<b>Our Product Concept</b>	<ul style="list-style-type: none"><li>• Intravenously-administered AAV5 gene transfer therapy intended to replace the 21OH enzyme in the adrenal cortex, potentially normalizing steroid levels (e.g., cortisol, aldosterone, and androgens)</li><li>• A study in nonhuman primates demonstrated significant transfection in the adrenals where 21OH is synthesized</li></ul>
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<b>Key Competitors</b>	<ul style="list-style-type: none"><li>• NBI-74788, a CRF receptor antagonist</li><li>• SPR001, a CRF receptor antagonist</li><li>• ATR-101, an ACAT1 inhibitor</li></ul>
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### ***Disease Overview***

CAH is a debilitating and life-threatening disease with no available cure, despite newborn screening for the disease being conducted in every U.S. state. The disease is defined by an inability to produce cortisol and aldosterone, and an excess production of testosterone. Lack of cortisol disrupts glucose metabolism and the body's normal response to stress, leading to potentially fatal adrenal crises, while lack of aldosterone disrupts sodium retention, resulting in low blood pressure, arrhythmia and dehydration. Additionally, excess testosterone causes virilization in females, often leading to ambiguous genitalia and masculinizing features at birth. Hormonal changes during puberty compound the CAH deficiencies. Females often suffer from limited fertility and require intensive treatment before, during, and after pregnancy, and up to 40% of adult males will have adrenal rest tumors which can lead to gonadal dysfunction and infertility, occasionally requiring surgery.

Over 90% of CAH cases are caused by 21OHD, a genetic defect in the CYP21A2 gene coding for the enzyme 21OH. Mutations resulting in loss of enzymatic activity of 21OH prevent conversion of progesterone into 11-deoxycorticosterone and 17-hydroxyprogesterone (17OHP) into 11-deoxycortisol, which are the precursors to aldosterone and cortisol, respectively.

CAH patients with 21OHD can be divided into two categories depending on the type of genetic mutation: classic and non-classic. We are primarily focused on treating classic patients, who have the more severe phenotype and that can be categorized into simple virilizing (approximately 25% of patients) and salt-wasting (approximately 75%) by the severity of aldosterone deficiency and level of residual 21OH enzyme activity. Patients with the salt-wasting form of disease have residual enzyme activity of 0-1% of normal and patients with the simple virilizing phenotype have 1-10% enzyme activity. All patients with the classic form require treatment at birth, as cortisol deficiency can lead to adrenal crisis as early as one to four weeks of life and can quickly lead to death. The salt-wasting form has an incidence of one in 20,000 births, while the simple virilizing form has an incidence of one in 60,000 births. Together, these translate to an estimated 600 classic patients born in the United States and Europe per year. We estimate there are more than 75,000 patients in the United States and Europe in the total addressable patient population.

Current standard of care treatments do not cure patients, but replace missing glucocorticoids, such as cortisol and mineralocorticoids, such as aldosterone, as well as reduce excessive androgen secretion. Although glucocorticoids are the mainstay of CAH therapy, individuals respond in varying ways, and chronic use of glucocorticoids in children and adults requires careful management because of the well-known side effects of these drugs, such as Cushingoid features, metabolic disease, obesity, hypertension, growth retardation, glucose intolerance, electrolyte disturbance, bone demineralization/increased risk of fracture and delayed puberty. Clinical management of classic CAH is often a very difficult balance between hyperandrogenism and hypercortisolism.

### ***Our Product Concept***

BBP-631 is a preclinical AAV5 gene transfer product candidate designed for the treatment of CAH due to 21OHD by replacing the 21OH enzyme in the adrenal cortex. Replacement of enzyme function has the potential to normalize flux through the pathway, simultaneously addressing the lack of cortisol and aldosterone, as well as the excess of testosterone and other androgens. Genotype-phenotype correlation studies in CAH suggest that non-classic patients, who are often asymptomatic and do not require treatment, have enzyme activity that is a little as 10% to 20% of normal individuals. We believe that an AAV gene therapy may be able to restore this level of enzymatic activity in CAH patients with both simple virilizing and salt-wasting forms of disease, providing substantial clinical impact and potentially eliminating the need for treatment with exogenous steroids. BBP-631 was granted both FDA and EMA orphan drug designation in 2018 for the treatment of CAH caused by 21OHD.

### ***Development Status***

Initial preclinical activity was explored in a Cyp21 knockout mouse model using AAVrh10. An IV injection of vector genomes was observed to improve multiple disease-related factors over a 15-week duration window, including an increase in body weight, a decrease in urinary progesterone (the main substrate of 21OH), and an increase in renin expression (signaling an increased capacity for salt retention).

A study in nonhuman primates (NHP) comparing evaluated AAV serotypes 1, 5, and 6 identified AAV5 as the optimum serotype. We observed significant transfection in the adrenals where 21OH is synthesized. Additionally, AAV5 has relatively low seroprevalence in the human population limiting potential immunogenicity issues.

We are currently conducting two sets of ongoing NHP studies, designed to evaluate durability of expression, dosing/transgene expression relationships, and preliminary safety. Data from these studies will be collected over a six-month period, and the results will inform dose selection for clinical trials and complete IND-enabling work.

In the first set of experiments, which evaluated a lower dose of  $3 \times 10^{12}$  vector genomes per kilogram, we have observed sustained increases in Cyp21 mRNA levels up to six months out. Rapid decreases in vector genome counts and mRNA levels due to adrenal cell turnover have not been observed between 1.5 and six months, providing preliminary support for sustained transgene expression. Toxicology studies remain ongoing. We continue to evaluate pathology from the animals as we conduct the full required safety assessments for submission of this IND.

In the second set of experiments, where we are evaluating additional doses of BBP-631, we have also observed increasing Cyp21 mRNA levels up to three months out. We have not observed rapid decreases in vector genome counts and mRNA levels due to adrenal cell turnover between one to three months, supporting our initial findings regarding sustained transgene expression. Data from six months is not yet available.

We anticipate filing an IND for BBP-631 in 2020. Subject to the acceptance of this IND and the initiation and completion of our anticipated Phase 1 clinical trial of BBP-631 in accordance with our plans, we anticipate receiving clinical proof-of-concept data in 2021.

### ***Key Competitors***

There are two alternative therapeutic mechanisms being investigated for treatment of CAH. The first are corticotropin-releasing factor type 1 (CRF1) receptor antagonists. CRF1 receptor antagonists regulate the release of adrenocorticotrophic hormone (ACTH) from the pituitary gland, which stimulates androgen and cortisol synthesis in the adrenal gland. In healthy individuals, endogenous cortisol provides negative feedback to the release of ACTH, which keeps androgen synthesis well regulated. Because this negative feedback is severely impaired in CAH patients, supraphysiologic doses of exogenous steroids are required to normalize androgen synthesis in these patients. While CRF1 receptor antagonists may regulate androgen synthesis, they do not address the lack of cortisol

or aldosterone production in these patients. Therefore, steroid supplementation is still required with CRF1 receptor antagonists. Two CRF receptor antagonists, NBI-74788 (under development by Neurocrine Biosciences, Inc.) and SPR001 (under development by Spruce Biosciences, Inc.), are currently in Phase 2 clinical trials.

The second alternative therapeutic mechanism is acetyl-coenzyme A acetyltransferase 1 (ACAT-1) inhibition. Inhibition of this metabolic enzyme induces targeted cell death in the adrenal gland, reducing steroid production and secretion. However, like CRF1 receptor antagonists, ACAT-1 inhibitors do not address the lack of cortisol or aldosterone production in these patients. ATR-101, an ACAT1 inhibitor, is currently in Phase 2 clinical development by Millendo Therapeutics, Inc.

While these alternative therapeutic mechanisms attempt to address meaningful aspects of the disease by potentially reducing the need for exogenous steroids, neither is able to address the disease at its source by targeting the complete set of features that define the disease. In particular, these mechanisms cannot obviate the need to administer steroids because they do not address the body's inability to synthesize cortisol and aldosterone. In contrast, we believe enzymatic replacement by gene therapy has the potential to simultaneously address all facets of the disease by restoring proper flux through the hormonal pathways, reducing androgen production by providing alternative pathways for the precursor molecules to be converted into cortisol or aldosterone.

**BBP-454 (TheRas): KRAS-Driven Cancers**

<b>Summary</b>	<ul style="list-style-type: none"><li>• We are advancing BBP-454, a preclinical development program for two novel approaches to inhibit KRAS activity, for the treatment of KRAS-driven cancers</li></ul>
<b>Development Status and Catalysts</b>	<ul style="list-style-type: none"><li>• Ongoing – Lead optimization</li><li>• 2020 – Development candidate nomination anticipated</li></ul>
<b>Pathway Overview</b>	<ul style="list-style-type: none"><li>• KRAS is a key driver of a number of large cancer indications with high unmet patient need including non-small cell lung cancers, pancreatic adenocarcinomas, and colorectal adenocarcinomas. Historically, KRAS has been thought to be an undruggable target, due to its lack of clear binding pockets</li><li>• KRAS normally drives cell growth and differentiation. Activating mutations, however, are understood to result in the uncontrolled development of cell proliferation and cancerous growth</li><li>• In order to signal for tumor growth, KRAS must be tethered to the cell membrane through a domain on KRAS known as the hypervariable region, or HVR. This can only occur when the HVR is in an “open” position</li><li>• Incidence of over 500,000 patients diagnosed with a KRAS-driven cancer in the United States and European Union</li></ul>
<b>Our Product Concept</b>	<ul style="list-style-type: none"><li>• We are developing small molecule, pan-mutant KRAS inhibitors, which act through binding to 2 novel sites on KRAS</li><li>• Our first approach involves compounds that bind KRAS at a novel pocket on the HVR, characterized by Frank McCormick, one of our co-founders and leader of the NCI RAS initiative, which prevents KRAS from binding to the cell membrane, thereby preventing signaling via the KRAS pathway</li><li>• The second approach involves targeting a unique residue on KRAS which promotes its degradation and thus down-regulates signaling</li></ul>
<b>Key Competitors</b>	<ul style="list-style-type: none"><li>• MRTX849, a KRAS G12C inhibitor</li><li>• AMG-510, a KRAS G12C inhibitor</li><li>• mRNA-5671, an mRNA vaccine</li><li>• Other small molecule KRAS G12C, G12D, and G13C inhibitors</li></ul>

**Pathway Overview**

KRAS is a member of the RAS family of oncogenes, which also includes HRAS and NRAS, and together comprise some of the most well-known monogenic drivers of cancer. Mutations in NRAS are frequently found in leukemia and melanoma, while HRAS is frequently mutated in bladder, thyroid, and head and neck squamous cell carcinoma. KRAS mutations are a frequent driver of a number of the largest cancer indications with high unmet medical need, including 30% of non-small cell lung cancers, 98% of pancreatic adenocarcinomas, and 45% of colorectal adenocarcinomas. The most common KRAS mutations involve a change from glycine at position 12 in the protein to aspartic acid (G12D, 36% of all KRAS mutations), valine (G12V, 24%), and cysteine (G12C, 15%) but also include mutations at glycine 13 and glutamine 61. In aggregate, over 500,000 patients in the United States and Europe are diagnosed with KRAS-driven cancers, annually.

KRAS is a G protein, meaning that it cycles between ON and OFF states when bound to GTP or GDP, respectively. When active, KRAS interacts with multiple proteins that initiate a series of reactions that

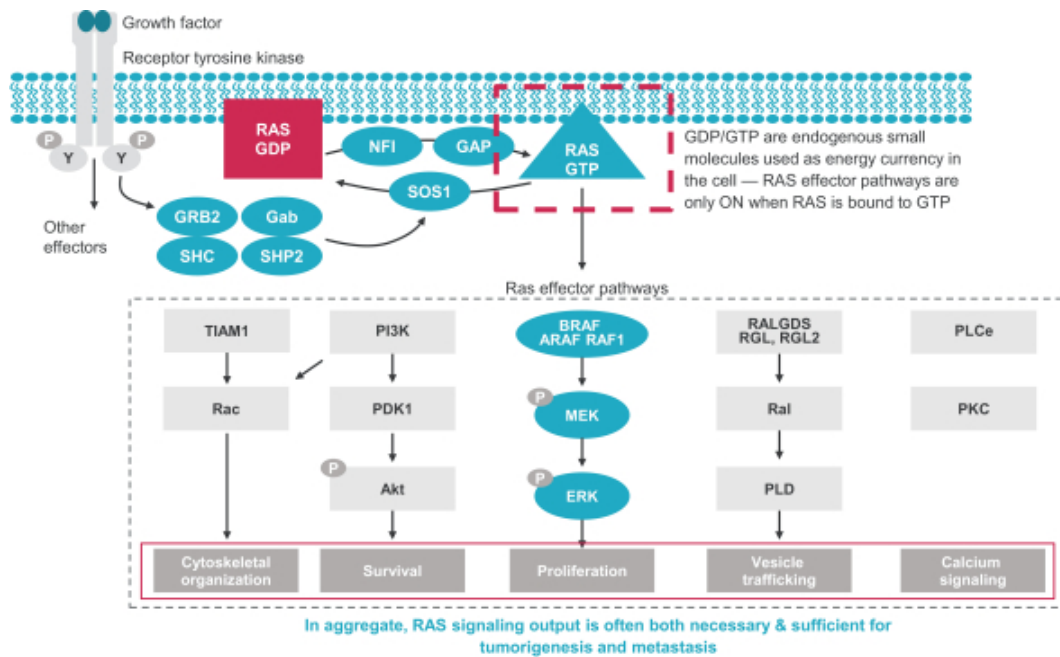


collectively cause cells to grow and divide. Because of its critical position atop multiple pathways, aberrant KRAS activation is a potent driver of unwanted cell growth, resulting in tumors. Normal KRAS is kept almost entirely in its inactive state by GTPase-activating proteins (GAPs), which cause KRAS to quickly convert GTP into GDP. All forms of mutant KRAS are insensitive to GAPs and remain bound to GTP long enough to drive oncogenic signaling. In order to initiate signaling, KRAS is required to be both localized to the cell membrane and bound to GTP. Historically, KRAS has been viewed as an undruggable target, due to the lack of a clear binding pocket to drug.

KRAS localization to the cell membrane is facilitated by modification to KRAS on the HVR. This modification consists of addition of a farnesyl or geranylgeranyl group to the cysteine residue at position 185 (C185) by farnesyl transferase or geranylgeranyl transferase. KRAS can only be modified when the hypervariable region is “open” and accessible to the transferring enzymes. When the hypervariable region is “closed,” KRAS cannot be modified, preventing its association with the cell membrane and subsequent downstream signaling.

An earlier therapeutic strategy inhibiting farnesyl transferase, which transfers farnesyl group onto HRAS to allow membrane association, has generated effective clinical responses in tumors driven by mutant HRAS. Though these molecules proved ineffective in KRAS, which has an adaptive capability to utilize an alternative modification (geranylgeranylation), these results for mutant HRAS provide a proof of concept for using a single agent disrupting localization of an oncogenic RAS mutant to treat RAS-driven tumors.

**The RAS Signaling Pathway**



**Our Product Concept**

KRAS-mutant cancers are driven by active, GTP-bound KRAS located at the cell membrane. We are developing two different strategies that target KRAS through novel, mutation-agnostic mechanisms. The first involves preventing modification of C185 in the HVR, disrupting the membrane localization process that is required for KRAS signaling. The second directly reduces concentration of active GTP-bound KRAS by targeting a novel residue to induce degradation.

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Frank McCormick, one of our co-founders and leader of the NCI RAS initiative, characterized a novel druggable binding pocket involved in positioning of the HVR on KRAS. Molecules that bind this eponymous “McCormick” pocket were confirmed to stabilize the KRAS HVR in a “closed” state, where C185 is not accessible for modification to localize KRAS to the membrane, thereby preventing oncogenic signaling. This mechanism is independent of the specific mutation causing KRAS tumors and is expected to apply to all oncogenic KRAS mutants. We have identified compounds that bind at this new pocket and covalently modify C185, thus preventing farnesylation and geranylgeranylation and thereby blocking membrane association.

The second approach is another pan-mutant KRAS drug which targets the histidine residue at position 95 (H95), an amino acid unique to KRAS located in the G-domain. Initial preclinical data have demonstrated that our initial series of compounds are able to downregulate KRAS signaling by degrading GTP-KRAS and thereby reducing the concentration of GTP-bound KRAS. We believe that degrading fully processed, active KRAS at the plasma membrane is the most direct strategy for eliminating oncogenic signaling across all mutant KRAS cancers. We believe our approach compares favorably with several other identified competitive approaches, which bind GDP-bound KRAS, which exists only transiently in mutant KRAS cancers. This mechanism is mutation agnostic, in contrast to competitive molecules, which only target a single mutation.

### ***Development Status***

Our current lead series targeting C185 show a sub-micromolar cell proliferation IC<sub>50</sub> in KRAS-expressing fibroblasts, and a greater than 30-fold IC<sub>50</sub> advantage for mutant KRAS over NRAS and HRAS. Treated cells also show dose-dependent depletion of membrane-associated KRAS, associated with decreased signaling through mutant KRAS and increased cancer cell death.

We are collaborating with the NCI RAS initiative and utilizing one of the most powerful supercomputers in the world at Lawrence Livermore National Labs to conduct molecular dynamics simulations, in order to continue to optimize our initial leads. Initial compounds targeting H95 are similarly under development; and currently studied compounds exhibit high micromolar affinity. We intend to nominate a development candidate in 2020.

### ***Key Competitors***

Due to its high prevalence in cancer, we expect to face competition from other small molecule KRAS inhibitors as well as other modalities, including mRNA vaccines, that target KRAS mutations. The majority of these product candidates focus on a single version of mutant KRAS, G12C. This form is particularly accessible for drug development due to the reactivity of the mutant cysteine residue. However, both compounds we are developing will target a broader set of cancers by pursuing mutation-agnostic mechanisms. In particular, our competitors may include:

- MRTX849, a KRAS inhibitor that only targets KRAS harboring a G12C mutation. Mirati Therapeutics, Inc., or Mirati, has dosed its first patient in a Phase 1/2 clinical trial enrolling patients with solid tumors harboring a KRAS G12C mutation. Additionally, Mirati is currently developing a KRAS G12D inhibitor through preclinical testing.
- AMG-510, a KRAS inhibitor that only targets KRAS harboring a G12C mutation, is in Phase 1 clinical development by Amgen Inc.
- AZD4785, an antisense oligonucleotide initially developed by Ionis Pharmaceuticals, Inc. and licensed to AstraZeneca plc, or AstraZeneca, is currently being tested in a Phase 1 clinical trial in NSCLC by AstraZeneca. AZD4785 targets a sequence in KRAS RNA to inhibit protein production.
- mRNA-5671, an mRNA vaccine targeting the four most common KRAS mutations, is currently under joint development by Moderna, Inc. and Merck & Co., Inc. The rationale is to induce a neoantigen response causing T-cells to attack tumors with KRAS mutations.

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- ARS-1620, a G12C-specific covalent small molecule, is currently in preclinical development by Wellspring Pharmaceutical Corporation in collaboration with Janssen Pharmaceuticals, Inc.
- Revolution Medicines, Inc. is in the hit-to-lead phase of discovery for small molecule inhibitors of KRAS mutations G12C, G12D, and G13C acquired from Warp Drive Bio, Inc.

### MENDELIAN PORTFOLIO

#### BBP-870 (Origin): MoCD Type A

<b>Summary</b>	<ul style="list-style-type: none"><li>• We are developing BBP-870, an IV formulation of synthetic cyclic pyranopterin monophosphate, or cPMP, for the treatment of molybdenum cofactor deficiency, or MoCD, Type A. BBP-870 received breakthrough therapy designation from the FDA in 2013 for MoCD, orphan drug designation from the FDA in 2009 and EMA in 2010 for the treatment of MoCD Type A, and rare pediatric disease designation for the treatment of MoCD Type A in June 2017</li></ul>
<b>Development Status and Catalysts</b>	<ul style="list-style-type: none"><li>• 2019 – Planned initiation of rolling NDA submission with application anticipated to be submitted in 2020</li></ul>
<b>Disease Overview</b>	<ul style="list-style-type: none"><li>• MoCD Type A is an autosomal recessive inborn error of metabolism that is characterized by the disruption of the molybdenum cofactor, or MoCo, biosynthesis pathway. The disease presents very early in life (median presentation at first day of life) with heterogeneous neurological symptoms such as seizures and feeding difficulties. Median survival for patients with MoCD is estimated to be approximately three years</li><li>• MOCS1 catalyzes the conversion of GTP to cPMP, a critical component in the biosynthesis of molybdenum cofactor. Patients with MoCD Type A have dysfunctional MOCS1 and are unable to produce cPMP</li><li>• Incidence estimated to be one in 100,000 to 200,000 live births worldwide, with MoCD Type A accounting for approximately two-thirds of all cases</li><li>• Diagnosis: Confirmed by genotyping. Blood and urine metabolite analyses are readily available to further initial clinical suspicion</li><li>• There are no available treatments approved for any form of MoCD. Supportive care and anti-convulsant therapy may be used to manage symptoms</li></ul>
<b>Our Product Concept</b>	<ul style="list-style-type: none"><li>• IV-administered, synthetically manufactured cPMP is designed to restore the biosynthetic production of molybdenum cofactor in patients with MoCD Type A</li><li>• To date, subjects with MoCD in clinical trials of BBP-870 have seen meaningful improvements in symptoms and symptomatic progression relative to historical controls. Patients have survived for a median of greater than eight years, well in excess of that seen in natural history studies of patients with the disease</li></ul>
<b>Key Competitors</b>	<ul style="list-style-type: none"><li>• We are not currently aware of any key competitors</li></ul>

#### *Disease Overview*

MoCD is an autosomal recessive inborn error of metabolism that is characterized by the disruption of the molybdenum cofactor, or MoCo, biosynthesis pathway. This results in reduced activity of enzymes that require

MoCo as a co-factor. In patients with MoCD, sulfite and S-sulfocysteine, or SSC, accumulate, resulting in brain injury. SSC is typically at very low or undetectable levels in healthy individuals, and this metabolite can be a diagnostic parameter for these patients. Patients diagnosed with MoCD are classified based on which gene is dysfunctional (MOCS1, MOCS2, MOCS3 or GPHN). Each gene product is necessary for a different step of the MoCo biosynthesis pathway. MOCS1 gene-related mutations are the most common cause of MoCD. Patients with MoCD Type A, or those with mutations in MOCS1, account for approximately two thirds of all patients with MoCD.

Disease presentation is complex, and symptoms often overlap with a number of different diseases. However, simple blood and urine metabolite analysis can potentially rapidly and cheaply narrow down the potential causative disease to either MoCD or isolated sulfite oxidase deficiency, or ISOD. Currently, only genotyping can confirm the diagnosis of MoCD type A.

MoCD prognosis is poor, with a median age at onset of symptoms within one day of birth and a median survival time of 36 months. Patients who survive beyond the neonatal period are likely to have severe irreversible central nervous system injury due to sulfite toxicity. A natural history study of 82 patients conducted in 2015 described key signs and symptoms in patients with MoCD including seizures (72%), feeding difficulties (26%), hypotonia (11%), motor development delay (9%), lens dislocation (2%), hemiplegia (2%), and hyperreflexia (1%).

The incidence of MoCD is estimated on average to be between 1/100,000 to 1/200,000 live births globally, with Type A accounting for two-thirds of all cases. Multiple publications state that there are over 100 known cases of MoCD to date, although MoCD is thought to be widely underdiagnosed. These estimates are likely underestimates of the true incidence due to a lack of awareness among physicians, the high infant mortality rate associated with the condition, and the potential clinical overlap with other conditions. There are no approved therapies for MoCD, and typically only supportive care such as anticonvulsants is used.

### ***Our Product Concept***

Patients with MoCD Type A are deficient in cyclic pyranopterin monophosphate, or cPMP, due to defects in MOCS1, which catalyzes the conversion of GTP to cPMP in patients with normal physiology. We are developing BBP-870 as a direct replacement of the missing substrate for the synthesis of MoCo in these patients, which, if successful, we believe may allow for restoration of normal MoCo biosynthesis.

### ***Clinical Data and Development History***

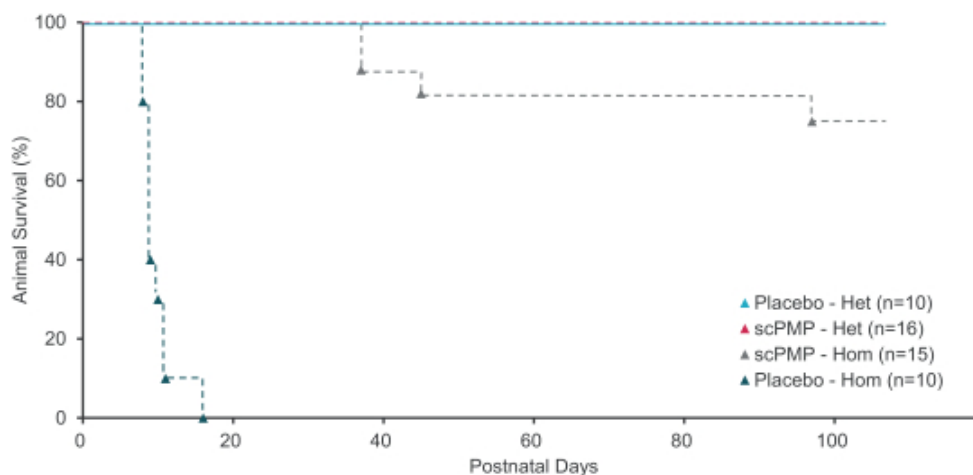
#### ***Studies with recombinant cPMP (rcPMP)***

cPMP was first tested clinically in pediatric patients with MoCD by Colbourne Pharmaceuticals (previously Orphatec Pharma) on a compassionate use basis. Colbourne utilized recombinant *E. coli*-derived cPMP (rcPMP) and administered the drug to 16 patients diagnosed with MoCD (11 Type A and 5 Type B). In patients with MoCD Type A, biomarkers returned to close to normal within two days of treatment with rcPMP. This was maintained for up to five years throughout the study. Eight patients with Type A disease rapidly improved under treatment and convulsions were either completely suppressed or substantially reduced. Three patients treated early remained seizure free and demonstrated near-normal long-term development. No biochemical or clinical response was detected in patients with Type B disease, as expected based on the molecular pathway and deficiencies of disease.

Of the 11 patients with MoCD Type A treated with rcPMP on a compassionate use basis, seven went on to receive BBP-870 in the Phase 2 clinical trial, as described below. Of the four patients who did not enroll in the Phase 2 clinical trial of BBP-870, two died, and the remainder were not receiving treatment as of the start of the BBP-870 Phase 2 clinical trial.

*Studies with BBP-870 (scPMP)*

Alexion acquired Colbourne’s rcPMP program and devised a synthetic manufacturing route to create BBP-870 (scPMP) – the monohydrobromide dihydrate salt of cPMP. This was done in order to improve the manufacturing process and reduce the costs of manufacturing. In preclinical studies, BBP-870 improved survival in MOCS1 knockout mice as shown in the following figure and reduced SSC in MOCS1 knockout mice to normal levels.



*Phase 1 Clinical Trial*

Alexion ran a randomized, blinded, placebo-controlled, single-dose, sequential-cohort, dose-escalation Phase 1 study designed to evaluate the safety, tolerability, and PK of IV BBP-870 in healthy adult subjects. This was the first time BBP-870 was administered to humans. A total of three dose cohorts were enrolled, each with six active and two placebo subjects, at the following doses: 0.10 mg/kg, 0.32 mg/kg, 0.90 mg/kg. In total, 18 subjects received BBP-870 and six received placebo.

This clinical trial demonstrated that single administered doses were well-tolerated in healthy adult subjects. One SAE was observed in the placebo arm of the trial and no SAEs were observed in patients receiving BBP-870. The maximum tolerable dose was not determined as no dose-limiting toxicities were observed.

*Phase 2 Clinical Trial*

BBP-870 is being studied in an ongoing Phase 2 clinical trial, which was initiated by Alexion and remains active, but not enrolling new patients. This study is a multicenter, multinational, open-label, dose-escalation study designed to evaluate the safety and efficacy in infants and children with a diagnosis of MoCD Type A confirmed by mutation in the MOCS1 gene, who were previously receiving treatment with rcPMP from Colbourne Pharma. There was no pre-specified primary efficacy endpoint. Secondary efficacy endpoints included change from baseline in: biomarkers, clinical neurological findings, motor and cognitive assessments, feeding patterns and other parameters. It was anticipated that as patients transitioned from rcPMP to BBP-870, they would have no change in their clinical status, thereby demonstrating comparable efficacy of BBP-870 to rcPMP.

On enrollment into the study, patients discontinued rcPMP and received daily IV infusions of BBP-870 at the same dose as their last dose of rcPMP. Dose escalation could occur after two months of treatment with BBP-870 until the patient either reached a dose that was not tolerated, or the patient’s exposure exceeded or was predicted to exceed that of the no observed adverse effects level, or NOAEL, AUC. After the six-month initial treatment period, patients entered the 60-month extension period, and continue to receive daily dosing of BBP-870 at their final tolerated dose.

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A total of seven patients with MoCD Type A have been enrolled into the study. As of March 13, 2019, no patients have dropped out of the trial, and all continue to receive BBP-870 at daily doses between 240 and 1,200 µg/kg. The duration of treatment with scPMP in the study ranges from over two years to almost five years. The prior duration of treatment with rcPMP for the patients enrolled in the study ranged from approximately eight months to more than six years. As of March 13, 2019, these seven patients have generally maintained clinical stability. As of January 31, 2019, the median age of the seven patients who remain in the trial is greater than eight years. The median survival for untreated patients is approximately three years.

BBP-870 has been well-tolerated. While there have been 31 SAEs and five AEs observed in the trial as of March 13, 2019, most were reported as recovered, and most were reported as not related or unlikely to be related to study drug. No SAEs or AEs definitely related to study drug were reported. Further, there were no deaths and no discontinuations of the clinical study or treatment as a result of an AE in subjects who received BBP-870 in this study. By System Organ Class, or SOC, the SAEs and AEs were as follows: infection and infestations (17), respiratory, thoracic and mediastinal disorders (two), general disorders and administration site conditions (10), product issues (three), surgical and medical procedures (one), metabolism and nutrition disorders (two) and vascular disorders (one).














### *Phase 2/3 Clinical Trial*

A Phase 2/3 clinical trial of BBP-870 was initiated by Alexion in neonates with newly diagnosed MoCD Type A and remains open, although it is not currently enrolling new patients. In July 2017, in advance of our acquisition of the BBP-870 program, enrollment was paused by Alexion. Two subjects enrolled in the trial. One subject was determined not to have a diagnosis of MoCD Type A on genetic analysis and was discontinued from the study after 17 days of treatment. The other subject remains on study drug. As of March 13, 2019, the patient remaining on study drug demonstrated progress on developmental milestones, along with improvement in biomarkers. There were 11 SAEs and two AEs observed (10 SAEs and two AEs in the subject remaining on study drug); however, these were adjudicated to be unrelated or unlikely to be related to study drug. By SOC for the subject remaining on study drug, the SAEs and AEs were as follows: respiratory, thoracic and mediastinal disorders (one); product issues (one); infections and infestations (five), general disorders and administration site conditions (two), gastrointestinal disorders (two) and cardiac disorders (one). One SAE of cardiac failure was observed after discontinuation in the subject not diagnosed with MoCD Type A. The SAE was reported as resolved and was considered not to be related to treatment with study drug.

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### Summary of Clinical Evidence

The totality of clinical evidence generated in the Phase 2 and Phase 2/3 clinical trials of BBP-870 has demonstrated promising activity. The figure below shows that patients treated with BBP-870 have achieved meaningful development milestones and functional improvements that are typically not seen in historical, genotype-matched controls. Notably, five of eight treated patients have achieved the development milestone of sitting upright with support and three of eight patients have achieved the development milestone of ambulation, to-date. These development milestones are typically not observed patients with MoCD Type A and were not achieved in genotype-matched natural history controls not treated with BBP-870.

Patient	Age at Tx Initiation <sup>2</sup>	Tx Duration <sup>2</sup>	Milestones Achieved <sup>1</sup>			Genotype-matched Natural History
			Rolling	Sitting <sup>4</sup>	Ambulatory	
1	1d	8.8y				Death at d10
2	1d	7.5y				Spastic quadriplegia
3	1d	2.6y		 <sup>5</sup>		Death at 1m – 4y
4	6d	8.9y	Spastic quadriplegia			Death at <1y
5	9d	9.2y				Death at <1y
6	37d	10.7y	Spastic quadriplegia			Spastic quadriplegia
7	69d	6.5y	Spastic quadriplegia			Unknown
8	<244d <sup>6</sup>	>3.6y <sup>6</sup>				Death at 5 wks / Spastic quadriplegia

**Milestones of sitting upright with support and ambulation are typically never seen in genotype-matched Natural History controls**

<sup>1</sup> Based on 2 April 2018 data cut; <sup>2</sup> Tx initiation for rcPMP for pts 1,2,4-8, scPMP for pt 3; <sup>3</sup> As of 31 January 2019, includes rcPMP tx duration for pts 1,2,4-8; <sup>4</sup> Upright with support at minimum; <sup>5</sup> With support; <sup>6</sup> Age at Tx initiation and Tx duration with rcPMP not available for pt 8

### Clinical Development Plan

Subject to planned communications with the FDA we intend to request in 2019, including communications arising from our Type B meeting with the FDA in 2019, we expect to initiate the submission of a rolling NDA seeking regulatory approval for BBP-870 for the treatment of MoCD Type A in the United States in 2019, based on existing safety and efficacy data. We expect the rolling submission of the NDA to be completed by 2020, at which time we expect to also submit a request for priority review.

While we believe the existing safety and efficacy data for BBP-870 are sufficient to support approval in the United States, we are considering additional clinical trial and expanded access program options in 2019. We believe that these could enable us to gather more data about MoCD Type A and BBP-870, identify patients who will ultimately be candidates for commercial therapy in the event that BBP-870 is approved, and most importantly, provide patients with a devastating and generally lethal disease the potential to receive treatment prior to commercial approval. Meetings with the European Medicines Agency are pending and therefore the data requirements sufficient for a marketing authorization application, or MAA, filing are unknown.

BBP-870 originally received orphan drug designation from the FDA in 2009 and EMA in 2010 for the treatment of MoCD Type A. In 2013, BBP-870 received breakthrough therapy designation from the FDA for MoCD and most recently, BBP-870 received a rare pediatric disease designation in June 2017 for the treatment of MoCD Type A.

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In the event that BBP-870 receives marketing authorization, we intend to market and distribute the drug worldwide. Given the severity of disease and lack of any available disease modifying treatments, we believe that there will be meaningful demand for our product if it is approved. While the current estimated incidence of one in 100,000 to 200,000 live births is low, we believe that the incidence of the condition may be underestimated. We believe this is due to the lack of physician awareness of the disease, high rates of infant mortality, and general CNS symptomatology that is not necessarily distinct from other diseases.

We are currently planning and executing on activities to both increase disease awareness amongst the physician community and to identify prospective patients for therapy. We are working with leading neonatologists, pediatric neurologists, and geneticists to increase awareness of MoCD Type A, and to educate the medical community that readily accessible metabolite testing can be used to screen patients. We are also speaking with providers of in-vitro diagnostic panels to include MoCD Type A in their screening assays.

As an FDA-designated rare pediatric disease, we plan to pursue a priority review voucher which would issue upon NDA approval by the FDA if applicable requirements are met. These vouchers entitle the holder of such voucher to request priority review of a subsequent drug or biologic application. Such vouchers are transferrable and we may elect to sell the voucher if and when received.

### **Key Competitors**

We are not currently aware of any key competitors.

### **BBP-009/Patidegib (PellePharm): Gorlin Syndrome and High Frequency Basal Cell Carcinoma**

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<b>Summary</b>	<ul style="list-style-type: none"><li>We are developing BBP-009, an investigational topical gel formulation of patidegib, a hedgehog inhibitor, for the treatment of Gorlin Syndrome and High-Frequency Basal Cell Carcinoma, or HF-BCC. We have received breakthrough therapy designation from the FDA, as well as orphan drug designation from both the FDA and EMA for BBP-009</li></ul>
<b>Development Status and Catalysts</b>	<ul style="list-style-type: none"><li>Ongoing – Phase 3 clinical trial in Gorlin Syndrome</li><li>2019 – Anticipated initiation of Phase 2b clinical trial in HF-BCC</li><li>2020 – Conclude Phase 3 clinical trial in Gorlin Syndrome</li></ul>
<b>Disease Overview</b>	<ul style="list-style-type: none"><li>Basal Cell Carcinomas, or BCCs, a form of skin tumor, are universally driven by overactivation of the hedgehog pathway</li><li>Gorlin Syndrome is caused by a genetic mutation in Patched1, or PTCH1, the primary inhibitor of the hedgehog signaling pathway</li><li>Uninhibited hedgehog signaling can cause tumorigenesis, leading to the formation of BCCs, particularly on the face and sun-exposed regions</li><li>HF-BCC is the presentation of more than nine BCCs over a period of three years, without having the genetic mutation present in Gorlin Syndrome</li><li>Gorlin Syndrome has a prevalence of approximately 1/31,000 individuals worldwide, approximately 10,000 patients in the United States and 17,000 in the European Union. HF-BCC has a larger patient group of approximately 1/9,000 individuals, approximately 35,000 patients in the United States and 57,000 in the European Union</li><li>Diagnosis: Clinical diagnosis, with potential for genetic confirmation</li><li>No FDA or EMA approved therapies for Gorlin Syndrome currently exist. Current treatments involve surgical resection of BCCs, or topical 5-FU or imiquimod, which are effective only in treating superficial BCCs</li></ul>

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**Our Product Concept**

- BBP-009 is a topically formulated small molecule hedgehog inhibitor, or HHI
- Designed to treat BCCs using the HHI mechanism validated by studies with oral HHIs
- Potential to have fewer safety and tolerability concerns than those associated with oral therapies that leave them unsuited to long-term or chronic treatment
- We believe Phase 2 clinical trials completed in Gorlin Syndrome and sporadic, nodular BCC patients demonstrated proof of concept and signs of efficacy against BCCs without the adverse events of oral HHIs

**Key Competitors**

- SUBA-itraconazole, an anti-fungal currently in Phase 2b clinical development for Basal Cell Carcinoma Nevus Syndrome

**Disease Overview**

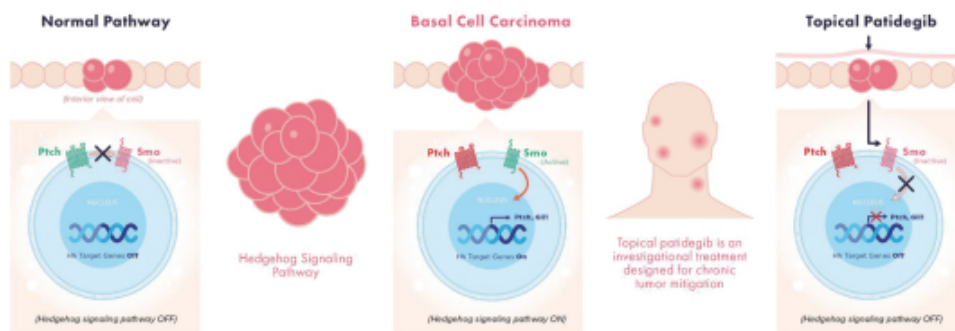
*Gorlin Syndrome*

Gorlin Syndrome, also known as Basal Cell Carcinoma Nevus Syndrome, is a condition that increases the risk of developing basal cell carcinomas, or BCCs, a form of skin tumors. This disease is driven by a genetic mutation in the tumor suppressor gene encoding Patched1, or PTCH1, which acts as the primary inhibitor of the hedgehog signaling pathway. PTCH1 inhibits hedgehog signaling by down-regulating the signal receptor protein known as “Smoothed,” or SMO. When PTCH1 cannot block SMO, the hedgehog signaling pathway is not inhibited, and it can act tumorigenically, leading to the formation of BCCs. Patients with Gorlin Syndrome can develop hundreds of BCCs, particularly on the face and other sun-exposed areas. At present, Gorlin Syndrome is not well-addressed by the available treatment options; the main intervention is surgery to remove BCCs, which is painful for the patient, can lead to scarring, and is not an ideal option for a condition that requires long-term chronic treatment. Gorlin Syndrome patients can undergo as many as 30 surgeries per year. Worldwide prevalence is approximately one in 31,000 individuals and there are approximately 10,000 Gorlin Syndrome patients in the United States and 17,000 in the European Union.

*HF-BCC*

Patients with HF-BCC are understood to have a Gorlin-like phenotype and more than three BCCs per year, without actually having Gorlin Syndrome. These patients exist at a prevalence of about one in 9,000, representing an estimated 35,000 patients in the United States and 57,000 in the European Union.

**Patidegib: A Small Molecule That Inhibits Hedgehog Signaling**



### ***Our Product Concept***

Patidegib is under development to treat the BCCs that characterize Gorlin Syndrome at their source by turning off the hedgehog signaling pathway. It is designed to block the SMO signal, thus turning off the oncogenic hedgehog activity. Oral HHIs like the FDA-approved vismodegib have demonstrated significant efficacy against BCCs in the clinic via this mechanism. However, oral HHIs' high toxicity makes them poor candidates for long-term chronic use; in a 42-person trial in Gorlin Syndrome patients assigned 2:1 to vismodegib versus placebo, 54% of patients discontinued oral vismodegib due to on-target class side effects (hair loss, muscle cramps, taste loss), and only one in five eligible patients could continue vismodegib for 18 months. We believe that a topically formulated HHI has the potential to treat BCCs just as effectively as an orally delivered HHI – while avoiding the systemic toxicity of the oral drug.

Patidegib is designed to bind and inhibit SMO at single-digit nanomolar concentrations. Unlike other HHIs, it can be formulated into a topical gel stable at room temperature. In 2013, PellePharm, Inc. in-licensed the worldwide rights to patidegib from Infinity Pharmaceuticals, Inc., or Infinity, after Infinity had completed an oral Phase 1 clinical trial of patidegib in 94 patients with solid tumors refractory to standard therapy (39 of whom had BCCs). Following the acquisition of the BBP-009 program, PellePharm formulated patidegib into a topical gel. A formulation of 2% patidegib gel delivered skin concentrations of drug above IC<sub>50</sub> for SMO, the concentration at which 50% of SMO is inhibited. In preclinical Gorlin mouse models, a 4% patidegib gel showed comparable effects to oral vismodegib on reduction in BCC size and GLI1 levels, a key biomarker of this disease.

### ***Clinical Data***

We have conducted two Phase 2 clinical trials with topical patidegib gel, as well as a Maximum Use Systemic Exposure, or MUSE, study.

Of these two Phase 2 clinical trials, we conducted a Phase 2a, multi-center, double-blind, randomized, vehicle-controlled proof-of-concept clinical trial of 2% and 4% patidegib topical gel in the United Kingdom, or the '201 trial. In the '201 trial, a total of 17 Gorlin syndrome patients with surgically eligible BCCs were randomized in a 1:1:1 ratio to receive patidegib topical gel, 2% or 4%, or vehicle gel applied twice daily for 26 weeks. The primary efficacy endpoints were decrease in tumor size, which was defined as percent decrease in the sum of the greatest diameter of baseline treatment-targeted surgically-eligible basal cell carcinomas, or SEBs, at Week 26 and change in GLI1 mRNA levels at Week 6.

The '201 trial did not meet its primary efficacy endpoints in the intent-to-treat population. However, post-hoc image analysis performed by a group of four independent dermatologists suggested significant measurement error caused in one subject by dermatitis (rash), which was measured and obscured the BCC, and in another subject by the accidental identification of a non-BCC lesion as a BCC as histological confirmation of BCCs was not required in this study. Upon correcting for these errors in a modified intent-to-treat, or mITT, population, patidegib demonstrated a statistically significant decrease in SEB size in the cohort of patients receiving patidegib 2% cohort vs vehicle (p=0.04). In addition, in the mITT population, complete clinical response was observed in 12 of 45 tumors exposed to either patidegib 2% or 4%, whereas complete clinical response was observed in zero of 16 placebo-treated lesions. Complete clinical response was defined as 0 millimeter measurement of a tumor's longest diameter at Week 26. Most importantly, the ITT population patidegib gel-treated subjects developed a mean three-fold fewer number of new facial SEBs (0.4 new facial SEBs in patidegib gel-treated subjects vs. 1.4 new facial SEBs in vehicle) during the six months of application.

In the '201 trial, SAEs were reported in two subjects, both of which were in the vehicle control group and neither was considered related to study drug. The SOC of these SAEs was infections and infestations (pneumonia, pneumonia pneumococcal), both of which later resolved. One of these patients discontinued from the trial due to the SAE. No patients treated in an active arm of the trial experienced an SAE, or withdrew from the trial due to an AE or SAE. In the '201 trial, treatment-related AEs were observed as follows: in the 2% cohort, one AE of

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muscle spasms, and one AE of trigeminal neuralgia; in the 4% cohort, one AE of gastrointestinal disorders, four AEs of application site conditions, and one AE of dysgeusia; in the vehicle cohort one AE of application site reaction, two AEs of muscle spasms, one AE of dysgeusia, and one AE of alopecia.

We also conducted a Phase 2a, double-blind, randomized, dose-response clinical study of topical patidegib gel in patients with sporadic BCCs, or the '202 trial. In the '202 trial, a total of 36 subjects with BCC were enrolled into one of our sequential cohorts, each randomized 2:1 to receive active study drug or vehicle gel. The cohorts were: 2% patidegib gel once daily, or QD, 4% patidegib gel QD, 2% patidegib gel twice daily, or BID, and 4% patidegib gel BID. The primary efficacy endpoint was the change in GLI1 mRNA levels in drug-treated versus vehicle-treated tumors after 12 weeks of treatment. While the trial failed to meet its primary efficacy endpoint, decreases in GLI1 mRNA levels were observed in the patidegib 2% QD and BID cohorts, as well as the patidegib 4% BID cohort, while the patidegib 4% QD and vehicle cohorts demonstrated an increase in GLI1 mRNA levels.

In the '202 trial, no patients discontinued due to an AE. One patient in the vehicle control group experienced an SAE of diverticulitis that the investigator deemed as unlikely related to study drug. The patient was hospitalized and the patient recovered. 34.3% of subjects experienced at least one AE, and no preferred term was reported for >1 subject in any treatment group. Two treatment-related AEs were reported in one patient in the 4% cohort – eyelid edema and application site edema. Both AEs were mild and resolved.

Based on the data from the '201 trial, and specifically the *post hoc* clinical data that demonstrated a reduction in the number of new SEBs in treated subjects, the FDA granted patidegib breakthrough therapy designation for the reduction of the life-long, serious clinical morbidity and disease burden of persistently developing BCCs in patients with basal cell nevus syndrome (BCNS) (also known as Gorlin syndrome). We also received orphan drug designation from both the FDA and EMA for treatment of nevoid basal cell carcinoma syndrome, or Gorlin syndrome.

In addition to these two Phase 2a clinical trials, a MUSE study was performed in 22 patients. In this study, 4% patidegib topical gel was applied twice per day for two weeks to 25% of body surface area of the healthy adult subjects to assess PK and tolerability. Systemic drug levels in the MUSE study were 500-fold lower than seen in patients on oral HHIs. Twice-daily administration of 4% patidegib topical gel to healthy adults was well tolerated. The only treatment-emergent AE reported for more than one subject was application site pruritus, which was reported for two subjects and resolved without requiring any action. Treatment-emergent AEs considered to be possibly related or probably related to study drug were application site pruritus, application site erythema, and muscle spasms. Across these three trials where subjects were treated with topical patidegib, no AEs commonly associated with oral hedgehog inhibitors were seen (hair loss, cramping, taste loss), and no SAEs were seen amongst the patidegib-exposed subjects.

### **Clinical Development Plan**

We have initiated a multicenter, randomized, double-blind, vehicle-controlled Phase 3 clinical trial for topical patidegib gel, 2%, as a treatment for Gorlin Syndrome. The study is expected to enroll 150 subjects in two arms, randomized 1:1. The primary objective of the trial is to assess the reduction in the number of new surgically eligible BCCs on the face of subjects, over 12 months. The secondary objective is to assess safety and tolerability.

We also intend to study the use of topical patidegib gel in non-Gorlin patients with high-frequency BCCs in a Phase 2b clinical trial, which we expect to initiate in 2019.

### **Partnerships**

In November 2018, we through our investment in PellePharm, Inc., or PellePharm, entered into a partnership with LEO Pharma, pursuant to which LEO has acquired a minority stake in PellePharm and has agreed to provide

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additional non-dilutive capital to fund the development of topical patidegib, including our planned Phase 3 clinical trial. LEO also acquired an option to purchase all shares in PellePharm at a later date. See “—Our Material Agreements—BBP-009 (Patidegib): Option Agreement with LEO Pharma A/S.”

### **Key Competitors**

Mayne Pharma is currently developing SUBA itraconazole, an antifungal agent, for basal cell carcinoma nevus syndrome. This program is currently in Phase 2b.

### **BBP-589/PTR-01 (Phoenix Tissue Repair): Recessive Dystrophic Epidermolysis Bullosa (RDEB)**

<b>Summary</b>	<ul style="list-style-type: none"><li>• We are developing BBP-589, an IV-administered recombinant collagen type VII, or rC7, protein replacement therapy, for the treatment of recessive dystrophic epidermolysis bullosa, or RDEB</li><li>• BBP-589 received orphan drug designation from the FDA and EMA in 2014 for the treatment of dystrophic epidermolysis bullosa and we received fast track designation from the FDA in 2019 for the treatment of dystrophic epidermolysis bullosa, or DEB</li></ul>
<b>Development Status and Catalysts</b>	<ul style="list-style-type: none"><li>• Ongoing – Phase 1/2 clinical trial</li><li>• 2020 – Anticipated data readout from Phase 1/2 clinical trial</li></ul>
<b>Disease Overview</b>	<ul style="list-style-type: none"><li>• DEB is a rare, genetic, multisystem disorder stemming from a lack of collagen type VII fibrils anchoring together layers of epithelial tissue, most notably skin. It is characterized by incurable, extremely fragile blistering skin; deformed limbs; and numerous co-morbidities, including squamous cell carcinoma and early death. RDEB, is a subtype of DEB with more severe symptoms</li><li>• Both DEB and RDEB are driven by mutations in COL7A1 gene coding for collagen type VII. Dysfunctional or deficient collagen type VII cannot anchor epidermal basement membrane to underlying papillary dermis</li><li>• Incidence of DEB is approximately 6/1,000,000 live births and prevalence is in approximately 3/1,000,000 people in certain western countries; RDEB prevalence is estimated at approximately 1.4 to 1.5 in 1,000,000 in western countries</li><li>• Current standard of care for both DEB and RDEB is mostly preventative and palliative symptom management (e.g., dressings, wound care, pain management, and nutritional supplements)</li></ul>
<b>Our Product Concept</b>	<ul style="list-style-type: none"><li>• Designed to be an IV-administered rC7, protein replacement</li><li>• A functional form of collagen type VII is intended to compensate for the dysfunctional protein in patients</li><li>• In <i>in vivo</i> models, rC7 accumulated at the basement membrane zone at the epidermal/dermal junction, formed functional anchoring fibrils, and reversed the DEB phenotype</li></ul>
<b>Key Competitors</b>	<ul style="list-style-type: none"><li>• KB103, a topical HSV-1 gene therapy</li><li>• EB-101, <i>ex vivo</i> autologous gene-corrected keratinocytes</li><li>• QR-313, an RNA oligonucleotide therapy for DEB exon 73</li></ul>

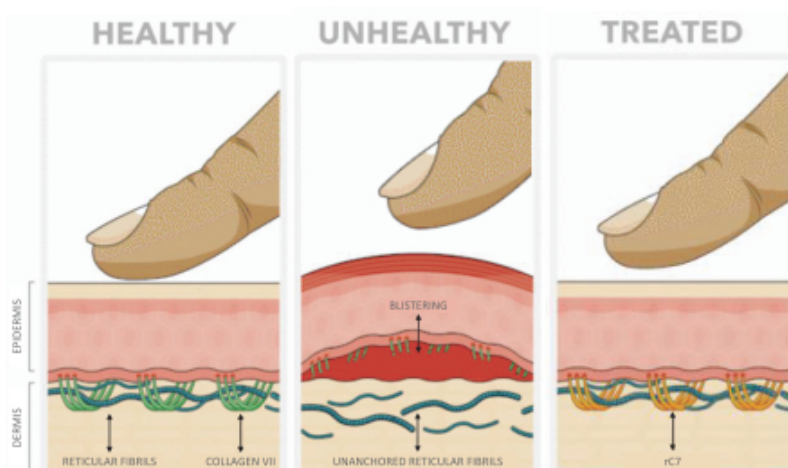
### **Disease Overview**

DEB is a genetic condition caused by mutations in the COL7A1 gene encoding the protein collagen type VII, a type of collagen protein that plays an important structural function. Collagen type VII resides in the basement

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membrane beneath stratified squamous epithelia and forms anchoring fibrils that hold layers of the epithelium together, most notably the epidermal and dermal layers of the skin. In DEB patients, mutations of the COL7A1 gene lead to deficient anchoring fibrils, resulting in normal physical touch or friction upon the epithelium causing severe blistering, wounds, and scarring of the skin as well as the mucous membranes and gastrointestinal tract, which are lined with epithelial cells. These patients can also suffer from joint contractures and pseudosyndactyly as a result of this condition as well as many other comorbidities, and they experience a shortened life expectancy often due to squamous cell carcinomas.

RDEB is a subtype of DEB that tends to have more severe symptoms and clinical outcomes. At present, there is no approved therapy for RDEB. All of the current standard of care treatment approaches rely on protective or palliative interventions. These include bandaging and disinfecting of wounds, nutritional supplementation and pain management none of which address the underlying cause of the disease. We believe there is a significant unmet need for a therapeutic option that can potentially address the cause of disease systemically and offer respite from the effects of the disease.



### ***Our Product Concept***

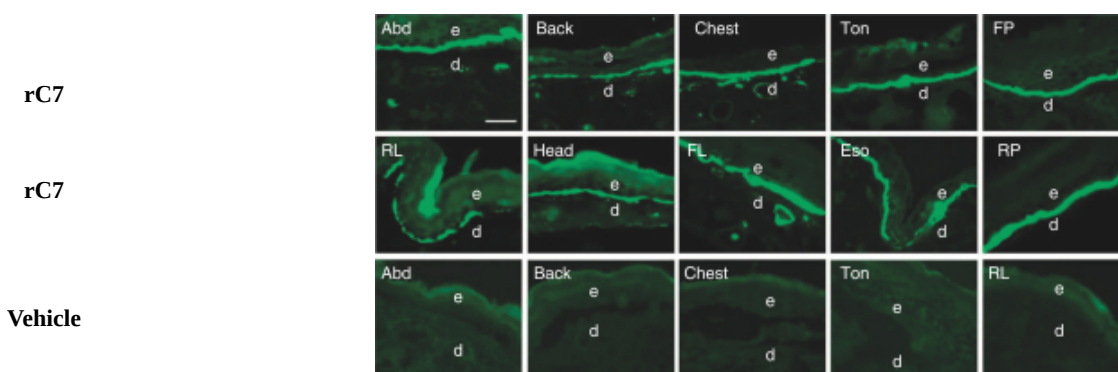
DEB is caused by dysfunctional collagen type VII protein. BBP-589 is a recombinant version of collagen type VII that is intended to take the place of the patient's defective protein and reverse the DEB phenotype by forming the anchoring fibrils needed to hold the dermis and epidermis together. We have successfully generated a Chinese Hamster Ovary, or CHO, cell line to produce rC7 protein for use in further development. BBP-589, also referred to as PTR-01, has received orphan drug designation for the treatment of DEB in both the United States and European Union, respectively. As a systemic protein replacement therapy candidate, rC7 is intended for intravenous delivery, a modality that has proven effective at delivering rC7 to the skin's basement membrane in preclinical animal models.

### ***Preclinical Data***

Preclinical studies have shown that rC7 distributes to the basement membrane of the skin. In COL7A1 knockout mouse models, treatment with intravenously delivered rC7 was observed to restore anchoring fibrils, promote healing and improve survival.

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As shown in the images below, rC7 was detected in target tissues by immunofluorescence (IF) in COL7A1 knockout mice following a single IV injection. Injected rC7 was detected at the dermal—epidermal junction, or DEJ, of multiple skin sites (top two rows); none was detected in vehicle-treated mice (bottom row).



*Newborn RDEB mice were intravenously injected with 16 mg of CHO derived rC7 or vehicle control via the superficial temporal vein, and tissue samples were obtained 5 to 14 days after mice were injected and subjected to immunofluorescence (IF) staining. Abd, abdomen; Eso, esophagus; FL, front leg; FP, front paw; RL, rear leg; RP, rear paw; Ton, tongue; E, epidermis; D, dermis*

Immunogold electron microscopy of skin obtained from COL7A1 knockout mice injected with rC7 showed formation of anchoring fibrils in the correct location. A single IV administration of rC7 to neonatal COL7A1 knockout mice was associated with a statistically significant improvement in survival compared to vehicle-treated controls.

In single bolus dose toxicity studies conducted in rats and non-human primates, or NHP, rC7 was shown to be well-tolerated. In PD studies, the rC7 administered product was detectable at up to four weeks in the tissue of COL7A1 knockout mouse while the serum half-life ranged from one to five hours in mouse, rat, and NHP. A 28-day repeat dose rat toxicology study and two 28-day NHP repeat-dosing IV toxicology studies included histopathology observations consistent with immune complexes and/or compound deposition. The NOAEL was determined at 4 mg/kg for NHP only, which was used to inform the starting dose of the Phase 1/2 clinical trial.

### **Clinical Development Plan**

Going forward, we expect to continue to develop BBP-589 in patients with RDEB. The present Phase 1/2 randomized, saline-controlled, single-blind, multiple ascending dose, dose-escalation trial was initiated in the first quarter of 2019. This first-in human study in adult RDEB patients will evaluate ascending doses of BBP-589 over three cohorts administering a total of three doses of BBP-589 and three doses of saline control IV to all patients in a cross-over design over a 10-week period. The primary objective of the trial is to evaluate the safety, tolerability and PK of BBP-589 in RDEB patients. Additionally, the trial will assess the proof of biologic activity through skin biopsy evaluation of C7, and formation of anchoring fibrils. Wound healing and clinically meaningful patient reported outcomes will also be evaluated at multiple timepoints. We expect to complete the trial in early 2020. BBP-589 received fast track designation from the FDA in 2019 for the treatment of DEB.

### **Key Competitors**

A number of companies are developing potentially competitive products for RDEB. Krystal Biotech, Inc. is developing KB103, a topical HSV-1 gene therapy currently in a Phase 1/2 clinical trial. Abeona Therapeutics, Inc. is developing EB-101, a topical therapy consisting of *ex vivo* autologous gene-corrected keratinocytes which

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has completed a Phase 1/2 clinical trial. ProQR Therapeutics N.V. is developing QR-313, an RNA oligonucleotide therapy for DEB exon 73. Fibrocell Science, Inc. is developing FCX-007, a COL7A1 gene therapy which has completed a Phase 1/2 clinical trial.

### **BBP-681 (Venthera): Venous and Lymphatic Malformations**

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<b>Overview</b>	<ul style="list-style-type: none"><li>We are developing BBP-681, a preclinical transdermal PI3K inhibitor, for the treatment of cutaneous venous and lymphatic malformations</li></ul>
<b>Development Status and Catalysts</b>	<ul style="list-style-type: none"><li>Ongoing – IND-enabling toxicology and formulation</li><li>2020 – Anticipated IND submission</li></ul>
<b>Disease Overview</b>	<ul style="list-style-type: none"><li>Venous malformations, or VMs, are large, disorganized veins that can cause significant morbidity due to functional impairment, pain, bleeding, and disfigurement. Lymphatic malformations, or LMs, involve the lymphatic vessels and cause functional impairment and pain similar to VM, lymphatic leakage and disfigurement. VM and LM can be isolated, mixed, or be part of complex syndromes known collectively as PIK3CA-related overgrowth syndromes, or PROS</li><li>The majority of VMs and LMs are driven by mutations in PIK3CA or its directly upstream activator TEK</li><li>Prevalence of greater than 75,000 VMs in the United States and EU in the skin and greater than 42,000 LMs in United States and European Union in the skin</li><li>These lesions typically present at birth, and diagnosis is confirmed with radiographic imaging</li><li>Standard of care is generally non-disease-modifying and invasive; ranges from compression bandages and aspirin, to laser ablation, surgical resection, and sclerotherapy</li></ul>
<b>Our Product Concept</b>	<ul style="list-style-type: none"><li>Topically formulated gel of proprietary small molecule inhibitor of PI3Ka under development to treat VM and LM</li><li>PI3K inhibition has been observed to cause a regression of lesions in a transgenic VM mouse model</li><li>BYL719, an orally dosed PI3K inhibitor, quickly and markedly improved VMs and LMs in PROS patients in a recent compassionate use study. Compared to oral dosing, we believe the transdermal approach may drive a higher local concentration of drug in the cutaneous lesion, with a lower concentration systemically</li><li>We believe transdermal therapy has the potential to replace or complement treatment across standard of care, and across the spectrum of severity. A topical gel has the potential to be used to treat an isolated VM/LM skin lesion or the skin manifestations of complex PROS</li></ul>
<b>Key Competitors</b>	<ul style="list-style-type: none"><li>ART-001, an orally dosed PI3Ka inhibitor for VMs and PROS</li><li>ARQ-092, an orally dosed AKT inhibitor for Proteus Syndrome and other PROS</li></ul>

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#### ***Disease Overview***

VMs are large, disorganized veins that can cause significant morbidity due to functional impairment, pain, bleeding, and disfigurement. LMs involve lymphatic channels that are overgrown and/or disconnected from the

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lymphatic system. These cause similar functional impairment to VMs, as well as pain, lymphatic leakage, and disfigurement. The standard of care for both VMs and LMs is often invasive and generally not disease-modifying. Intervention consists mostly of laser ablation of lesions, surgical resection and sclerotherapy, each of which are painful, rarely curative, and prone to complications. In the United States and Europe combined, it is estimated there are over 75,000 patients with cutaneous venous malformations, and another 42,000 cutaneous lymphatic malformations. The current standard of care options, like resection, laser ablation, and sclerotherapy, tend to not be curative, and the lesions require repeated, invasive treatment.

The majority of VMs and LMs are caused by mutations in PIK3CA or its directly upstream activator TEK. A recent compassionate use trial in 2018 showed that an orally dosed inhibitor of PI3Ka, the protein encoded by PIK3CA, causes a regression of VMs and LMs. The study was conducted in 19 patients with PIK3CA mutations and showed that vascular lesions, both on the skin and visceral, responded to inhibition of PI3K.

### ***Our Product Concept***

Our product candidate, targeting the cutaneous and subcutaneous manifestations of the disease, is a transdermal gel designed to permeate the skin and inhibit PI3Ka. Similar to observations in other transdermal approaches, we believe BBP-681 may yield a lower systemic concentration than orally dosed PI3Ka inhibitors and a higher concentration in the superficial but symptomatic lesions. In skin flux assays in Franz cell chambers, skin permeability greater than 100 fold above those achieved by topical rapamycin, an off-label therapy for LM and VM, was observed. Our PI3K inhibitor showed consistent suppression of the PI3K/AKT pathway in multiple cell lines including a human VM-derived TEK mutant endothelial cell line, as well as a mouse derived PIK3CA mutant endothelial cell line. Ten-point dose curves using a MTS viability assay in the same cell lines suggest that our compound may be at least as potent in cells as BYL719.

### ***Development Plan***

We are manufacturing GLP-grade quantities of this compound to support ongoing IND-enabling toxicology studies, and expect to file an IND by the end of 2019.

### ***Key Competitors***

Various other compounds are currently in development for PROS, including ART-001, which is under preclinical development by ARTham Therapeutics, Inc. as an orally dosed PI3Ka inhibitor for VMs and PROS, and Miransertib (ARQ-092), an orally available, selective, pan-AKT (protein kinase B) inhibitor in Phase 1/2 clinical development by Arqule, Inc. for Proteus Syndrome and other PROS.

### **BBP-671 (CoA): PKAN and Organic Acidemias**

<b>Summary</b>	<ul style="list-style-type: none"><li>We are developing BBP-671, a series of preclinical oral small molecules, allosteric activators of pantothenate kinases, for the treatment of Pantothenate Kinase Associated Neurodegeneration, or PKAN, and Organic Acidemias, or OAs</li></ul>
<b>Development Status and Catalysts</b>	<ul style="list-style-type: none"><li>Ongoing – IND-enabling development</li><li>2020 – Anticipated IND submission in OAs</li></ul>
<b>Disease Overview</b>	<p><i>PKAN:</i></p> <ul style="list-style-type: none"><li>PKAN is a rare genetic disorder with progressive neurodegeneration. Early onset patients typically demonstrate motor deficits with possible visual problems from retinal degeneration within six years of age. Later onset disease is heterogeneous, with</li></ul>



psychiatric symptoms and progressive parkinsonism developing in late childhood to adulthood

- Inactivating mutations in the rate-limiting coenzyme A (CoA) synthetic enzyme PanK2 is postulated to lead to CoA depletion
- Prevalence of approximately one in 1,000,000, with between 800 to 850 patients in the United States and European Union
- Diagnosed by magnetic resonance imaging, or MRI, and genetic testing
- No currently approved treatments

OA:

- Organic acidemias are caused by mutations in enzymes that disrupt amino acid metabolism leading to acute decompensations requiring hospitalization, as well as long term complications involving multiple organ systems, such as the heart, pancreas, kidney, liver, and brain
  - Mutations in metabolic enzymes lead to organic acid accumulation and is hypothesized to lead to CoA sequestration
  - Incidence of approximately 5 in 100,000 births
  - Newborn screening for OAs occurs in every U.S. state and most European countries
  - Standard of care includes dietary restriction and supplementation, but unmet need remains high due to metabolic decompensations and long-term complications
- Our Product Concept**
- Oral small molecule, allosteric activator of PanK 1-3 enzymes
  - Increased CoA synthesis restores CoA levels, which are thought to be depleted in PKAN, and overcomes metabolic dysregulation in OAs

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**Key Competitors**

*PKAN:*

- Fosmetpantotenate, an oral small molecule, substrate replacement agent
- A substrate replacement agent under development by COMET Therapeutics

*OA:*

- mRNA-3704, mRNA-3927, systemic intracellular therapeutics for select organic acidemias
  - LB-001 and SEL-302, gene therapy agents for methylmalonic acidemia
- 

***Disease Overview***

*Pantothenate Kinase-Associated Neurodegeneration*

PKAN is a rare, progressive neurodegenerative disorder arising in early childhood. The disease is defined by an accumulation of iron in the brain, which can be detected on an MRI and confirmed with genetic tests showing mutations in the PANK2 gene. While the symptomatology and progression vary widely, most patients develop symptoms before six years of age, while others present in late childhood to adulthood. Early onset patients typically demonstrate motor deficits with possible night-blindness from retinal degeneration. Later onset disease is heterogeneous, with psychiatric symptoms and progressive parkinsonism. There are currently no approved disease modifying therapies, with current treatments only partially controlling symptoms. PKAN affects approximately one in 1,000,000 people, and there are estimated to be between 800 and 850 patients in the United States and Europe.

### *Organic Acidemias*

OAs are caused by mutations in key enzymes of amino acid metabolism including propionyl-CoA carboxylase for propionic acidemia, or PA, methylmalonyl-CoA mutase for methylmalonic acidemia, or MMA, isovaleryl-CoA dehydrogenase for isovaleric acidemia and glutaryl-CoA dehydrogenase for glutaric acidemia type 1. Together OAs are identified in approximately five in 100,000 births through standard newborn screening. Toxicity in these diseases is driven by defective amino acid metabolism that results in high concentrations of acyl-CoA, and likely to sequestration of free CoA, and altered mitochondrial energy metabolism. Chronic metabolic disorders such as OA are frequently accompanied by malnutrition and developmental delay, with acute crises of metabolic decompensation that often results in permanent neurological damage. Current treatments include dietary restriction, administration of L-carnitine and glycine to promote transfer and excretion of acyl-CoAs, and liver transplantation. Despite these strategies, unmet need remains high as existing treatment strategies do not altogether prevent metabolic decompensations, nor long term complications such as neurodegeneration (in MMA and PA), kidney disease (in MMA) and cardiomyopathy (in PA).

More broadly, the pathophysiology of a group of up to 60 inborn-errors of metabolism may be associated with decreases in CoA or CoA intermediates. As an upregulator of CoA biosynthesis, BBP-671 may be potentially applicable as a treatment in these diseases, which affect an estimated 200,000 patients globally.

### ***Our Product Concept***

Pantazines are positive allosteric modulators of the Pantothenate Kinase, or PanK, enzyme family (PanK1, PanK2 and PanK3), which are the rate-limiting step in coenzyme A (CoA) synthesis. Using a combination of X-ray crystallography and *in vitro* enzyme activity assays, it has been shown that the pantazines bind to and activate all PanK enzymes by locking the protein in an active conformation to continually catalyze formation of 4'-phosphopantothenate, resulting in increased CoA production. In PKAN, mutations in the PanK2 enzyme lead to the development of neurological deficits, likely as a result from insufficient CoA. It is hypothesized in PKAN, pantazines can increase the activity of the remaining functional PanK1 and PanK3 enzymes to increase CoA to normal levels and improve dysfunction associated with PKAN. Indeed in a mouse model with deletion of PanK1 and PanK2 in neurons only PanK3 is expressed, yet pantazine treatment can increase CoA in the brain of these mice.

In PA, defective amino acid metabolism results in accumulation of toxic metabolites that inhibit key energetic pathways, resulting in a severe metabolic disorder. Increasing free CoA and acetyl-CoA may overcome inhibition of mitochondrial respiration caused by accumulation of toxic metabolites and organic acids.

BBP-671 is a series of small molecules designed to use a novel approach to increasing CoA synthesis through allosteric modulation of the PanK family of enzymes, responsible for the first and rate-limiting step in CoA synthesis. The compounds in our BBP-671 program are intended to be orally administered once-daily, as a highly central nervous system penetrant small molecule, and potentially as a combination therapy.

### ***Preclinical Data***

In preclinical *in vitro* studies, targeting PanK3 with one of our BBP-671 compounds increased PanK activity, and is thought to increase CoA in cells by locking the enzyme in the "on" position. In preclinical studies, this BBP-671 compound prevented feedback inhibition of PanK enzymes by acetyl-CoA and propionyl-CoA, and increased PanK activity in the presence of acyl-CoAs by up to 15-fold. BBP-671 has been optimized for high BBB penetration. This BBP-671 compound dose dependently increased CoA levels in the brain of wild-type mice after 5 doses by oral gavage.

In a mouse model of brain CoA deficiency, which lack PANK1 and PANK2 in neurons, animals have been shown to have reduced median survival of only 52 days, a 21% reduction in brain CoA levels and a significant

locomotor defect measured as a 60% reduction in percent of time moving in the open-field test. The same BBP-671 compound administered in the diet at a dose of 15 mg/kg/day from weaning increased brain CoA and improved motor phenotype by significantly increasing movement in an open-field test. Median survival was significantly prolonged to 150 days.

We have studied our compounds in a hypomorphic mouse model of PA, where the mouse PCCA gene is deleted and the human A138T PCCA mutant protein is expressed. In the mouse model, plasma biomarkers associated with the human disease are increased and growth is impaired. The same BBP-671 compound administered in the diet at 10 mg/kg/day from weaning in this mouse model of PA increased free CoA in the liver to a level comparable to that observed in untreated wild-type animals. Mice treated with this BBP-671 compound gained weight at a similar rate as wild-type mice, unlike untreated mice that gained weight more slowly. The BBP-671 compound decreased the C3-carnitine/C2-carnitine ratio by more than 50%, consistent with increasing free CoA leading to improvements relevant to the disease.

In non-clinical toxicology studies, we have observed dose-limiting corneal toxicity in a 14 day repeat dosing experiment in dogs. This BBP-671 compound did not achieve a NOAEL in these test subjects; however a NOAEL was achieved in rodents. We believe the toxicity observed is consistent with a species and chemotype-specific mechanism. Therefore, we have initiated the process of manufacturing scale-up for two backup BBP-671 compounds, which we intend to use for non-GLP toxicology studies in 2019.

### **Development Plan**

We are continuing IND-enabling work and subject to toxicology findings, we anticipate filing an IND in 2020.

### **Key Competitors**

For PKAN, Retrophin, Inc.'s substrate replacement approach is currently in a Phase 3 clinical trial. Consistent with preclinical pharmacokinetic data suggesting fosmetpantotenate has a short half-life, their treatment is currently dosed three times a day in the trial. COMET Therapeutics has a similar substrate replacement therapy in preclinical development.

For OA, Moderna, Inc. has filed an IND for mRNA-3704 for MMA, and mRNA-3927 for PA is in preclinical testing. Hemoshear Therapeutics, LLC also has two preclinical programs for MMA and PA. Two gene therapy programs for MMA have been disclosed by Selecta Biosciences Inc. and LogicBio Therapeutics, Inc., with both planning for IND filings in 2019. Gene therapies, similar to liver transplantation, are expected to address systemic sequelae, but may not address CNS manifestations. Synlogic, Inc. also has a discovery program for PA.

### **BBP-711 (Orfan): Primary Hyperoxaluria and Frequent Stone Formers**

<b>Summary</b>	<ul style="list-style-type: none"><li>We are advancing BBP-711, a preclinical development program for potential oral small molecule inhibitors of glycolate oxidase, or GO, for the treatment of primary hyperoxaluria and patients who experience frequent kidney stone formation</li></ul>
<b>Development Status and Catalysts</b>	<ul style="list-style-type: none"><li>Ongoing – Lead optimization</li><li>2019 – Expect to nominate a development candidate</li></ul>
<b>Disease Overview</b>	<ul style="list-style-type: none"><li>Primary hyperoxaluria, or PH1, is a rare, autosomal-recessive inborn error of metabolism driven by a defect in the AGXT gene, which codes for the enzyme alanine-glyoxylate aminotransferase, or AGXT</li><li>Deficiencies in the AGXT enzyme translate into the incapacity of PH1 patients to detoxify glyoxylate into glycine. As a result, glyoxylate is oxidized into oxalate which cannot be metabolized by humans. Elevated oxalate levels form calcium oxalate crystals, and subsequently kidney stones, which damage the kidneys, culminating in renal dysfunction</li></ul>

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- Prevalence for PH1 is estimated to be 5,000 patients in the United States and EU. Due to heterogeneous symptom presentation and similarity with other diseases, we believe that the disease is underdiagnosed. Prevalence for FSF is estimated to be 1.5 million in the United States and European Union
- Diagnosis: Early in life (median 7.7 years) after initial presentation with urinary problems, including kidney stones, blood in urine and progressive renal failure; confirmed by genotyping
- Standard of care involves symptomatic management through supplementation with vitamin B6, increased fluid intake, and citrate, to intensive dialysis and lithotripsy. Ultimately, the only curative treatment is a combined liver and kidney transplant

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### **Our Product Concept**

- We are selecting an oral small molecule candidate designed to inhibit GO, an enzyme that converts glycolate to glyoxylate
- By inhibiting GO, BBP-711 is designed to prevent the conversion of glycolate to glyoxylate, reduce the downstream production of oxalate and reduce the concentration of urinary oxalate; glycolate can be safely eliminated through urine
- In preclinical mouse models of PH1, BBP-711 reduced urinary oxalate
- Additionally, idiopathic recurrent kidney stones occur in approximately 1% of the world's population, who are also referred to as frequent stone formers, or FSF. Approximately 80% of kidney stones are primarily composed of calcium oxalate. We believe BBP-711 has the potential to show efficacy in such patients

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### **Key Competitors**

- Lumasiran, a RNAi agent targeting GO
  - DCR-PHXC, a RNAi agent targeting hepatic LDHA
- 

## ***Disease Overview***

### *Primary Hyperoxaluria*

PH1 is a severe, rare, autosomal-recessive inborn error of metabolism driven by the overproduction of endogenous oxalate due to deficiencies in the liver peroxisomal enzyme alanine-glyoxylate aminotransferase, or AGXT. PH1 presents early in life after initial presentation with urinary problems, including kidney stone formation, blood in the urine and progressive renal dysfunction. The median progression to end-stage renal disease, or ESRD, in patients with PH1 is approximately 24 years. Diagnosis is difficult, as symptoms are non-specific and disease awareness amongst physicians is relatively low.

In normal individuals, AGT is responsible for the catalysis of glyoxylate to glycine, which can safely be eliminated in urine. However, in patients with PH1, as AGT is inactive, glyoxylate levels increase and another enzyme, LDH, converts glyoxylate to oxalate. The elevated excretion of urinary oxalate, or UOx, promotes the generation of calcium oxalate crystals in the kidney and the urinary tract triggering recurrent nephrolithiasis and/or nephrocalcinosis which culminates in progressive kidney disease and kidney failure.

There are no pharmacologic disease-modifying therapies for PH1, and treatment is focused on symptomatic management. Patients receive supplementation with vitamin B6 and citrate, and are directed to increased fluid intake in order to dilute UOx. Approximately 40% of the PH1 patients are responsive to vitamin B6 supplementation; however, responders are typically unable to achieve UOx normalization with this approach. Intensive dialysis and lithotripsy may be indicated as the disease progresses and symptoms become more severe. The only potentially curative therapy for the disease is combined liver and kidney transplant, which has significant associated morbidity and mortality.

*FSF*

Kidney stones affect approximately 10% and 5% of the male and female populations, respectively, and after the first stone, the recurrence rate is between 4% to 5% for both males and females. Calcium oxalate is found in approximately 80% of urinary stones, with 60% of stones composed of pure calcium oxalate.

**Our Product Concept**

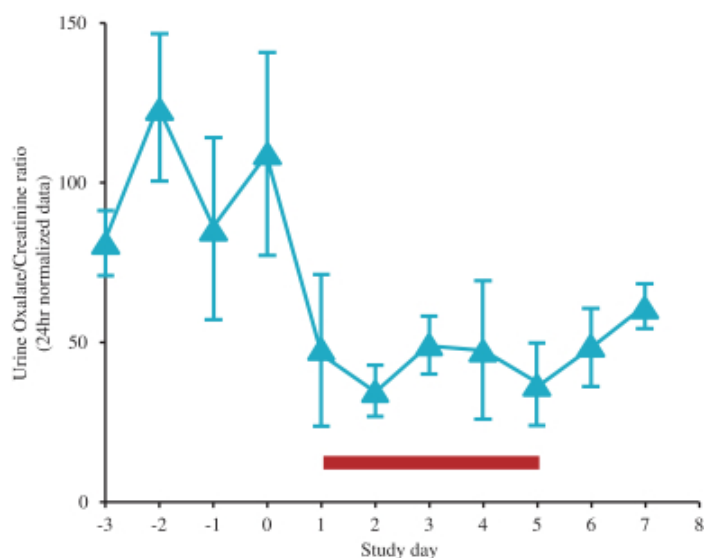
GO is a hepatic, peroxisomal enzyme that plays a key role in the production of oxalate, the molecule responsible for driving PH1 disease pathophysiology. Specifically, GO catalyzes the conversion of glycolate to glyoxylate, which is then converted to oxalate by LDH.

BBP-711 is a preclinical development program for oral small molecules designed to inhibit GO. By inhibiting GO, the production of glyoxylate is reduced, leading to the reduction in production of oxalate. By reducing oxalate formation, we believe that BBP-711 has the potential to reduce UOx in PH1 patients, and may therefore have potential as a future treatment for PH1.

In addition, there are many FSF that have recurrent idiopathic kidney stones made from calcium oxalate. While the present understanding of disease pathophysiology is incomplete, elevated levels of UOx and calcium increase the risk of calcium oxalate kidney stone formation. Treatments aimed at reducing dietary uptake of oxalate have shown efficacy in some, but not all, patients. We believe that BBP-711 has the potential to serve as a treatment for patients with calcium oxalate kidney stones by reducing levels of the key substrate necessary for intrinsic production of UOx, a key driver of kidney stone formation in these patients. As such, we may pursue an IND for BBP-711 in FSF as a potential expansion indication.

**Preclinical Data**

We are currently in the lead-optimization stage of preclinical development of BBP-711. Our lead series of potent and selective inhibitors of GO have been observed to reduce hyperoxaluria in mice homozygous for a null mutation in the AGXT1 gene, a well-validated model for PH1. The figure below shows UOx levels of AGXT1 knockout mice before and after five days of dosing (indicated with red line) with our lead development candidate.



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### **Development Plan**

We continue to optimize our initial lead series of product candidates and intend to nominate a development candidate for IND-enabling studies in 2019.

### **Key Competitors**

Key competitors for PH1 include Alnylam Pharmaceuticals, Inc., or Alnylam, and Dicerna Pharmaceuticals, Inc., or Dicerna. Alnylam is developing a Phase 3 RNAi therapeutic candidate targeting GO (Lumasiran) for the treatment of PH1. Dicerna is developing a Phase 2/3 RNAi therapeutic candidate targeting LDHA for the treatment of all subtypes of PH.

### **BBP-561 (MoST): Netherton Syndrome**

<b>Summary</b>	<ul style="list-style-type: none"><li>We are developing BBP-561, a preclinical program for the development of topical KLK5/7 inhibitors, for the treatment of Netherton Syndrome</li></ul>
<b>Development Status and Catalysts</b>	<ul style="list-style-type: none"><li>Ongoing – Lead optimization</li><li>2019 – Anticipated development candidate nomination</li></ul>
<b>Disease Overview</b>	<ul style="list-style-type: none"><li>Netherton Syndrome is a devastating genetic disease characterized by skin breakdown complicated by risk of sepsis, severe malnutrition, and dehydration in affected neonates. It can additionally lead to chronic problems including allergy, infection, and inflammation</li><li>Driven by a loss of function mutation in the gene SPINK5, which encodes the serine protease inhibitor LEKTI, the subsequent, uninhibited serine protease activity by KLK5 and KLK7 leads to premature loss or disruption of the outer layer of the skin, or stratum corneum, as the proteins holding the skin together are degraded.</li><li>Prevalence of 4,000 – 17,000 patients in the United States and European Union</li><li>No disease-modifying therapy exists. Palliative and preventative treatments are used to manage symptoms</li></ul>
<b>Our Product Concept</b>	<ul style="list-style-type: none"><li>We are developing a topically formulated, small molecule serine protease inhibitor designed to be active against both KLK5 and KLK7</li><li>Restored inhibition of KLK5 and KLK7 prevents breakdown of proteins required for maintaining intact outer skin</li><li>Studies in a LEKTI-knockout mouse indicated that both KLK5 and KLK7 inhibition were required to reverse Netherton Syndrome phenotype</li></ul>
<b>Key Competitors</b>	<ul style="list-style-type: none"><li>LM-030, a topical kallikrein inhibitor</li><li>SXR1096, a preclinical small molecule kallikrein inhibitor</li><li>AZT-02, a Staphylococcus epidermis strain engineered to express LEKTI</li><li>KB-104, a HSV-1 based topical gene therapy</li></ul>

### **Disease Overview**

Netherton Syndrome is a devastating genetic disease caused by an autosomal recessive mutation in the gene SPINK5 (Serine Protease Inhibitor of Kazal Type 5), which encodes protein LEKTI (lympho-epithelial kazal type related inhibitor type 5). LEKTI is a serine protease inhibitor expressed in skin where a key function is to

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inhibit Kallikrein 5 and 7, or KLK5/7. Unregulated KLK5/7 serine protease activity leads to premature detachment of the stratum corneum, the outer skin layer. Loss of stratum corneum can be life-threatening to neonates due to systemic sepsis or severe dehydration due to the compromised epithelial barrier, and it can lead to chronic problems for patients, including allergy, infection, and inflammation.

Netherton Syndrome has an estimated prevalence of between one out of 50,000-200,000, from which a potential population of 1,600-6,500 patients are estimated in the United States and another 3,700-15,000 patients in Europe. Currently, no disease-modifying therapy exists for Netherton Syndrome.

### **Our Product Concept**

A 2017 paper in the Public Library of Science Genetics journal indicated that in a SPINK5/LEKTI-knockout mouse, only the combined knockout of KLK5 and KLK7 was sufficient to reverse the Netherton Syndrome phenotype. With this in mind, we evaluated previously described KLK inhibitors and used this information as a starting point for our lead optimization work to identify a molecule with properties suitable for IND-track development. Our medicinal chemistry work yielded BBP-561, which are serine protease inhibitors that have been observed to inhibit KLK5 and KLK7 *in vitro*.

### **Development Plan**

We intend to continue our IND-enabling work with BBP-561 pursuing improved synthesis, topical formulation development, development candidate finalization and selection. We anticipate nominating a development candidate in 2019.

### **Key Competitors**

LifeMax Healthcare International Corp. is developing LM-030, a topical kallikrein inhibitor, which has completed a Phase 2a clinical trial. Sixera Pharma Ab is developing SXR1096, a preclinical small molecule kallikrein inhibitor. Azitra Inc. is developing AZT-02, a Staphylococcus epidermis strain engineered to express LEKTI in preclinical development. Krystal Biotech, Inc. is developing KB-104, an HSV-1 based topical gene therapy in preclinical development.

### **BBP-761 (Fortify): Leber's Hereditary Optic Neuropathy**

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<b>Summary</b>	<ul style="list-style-type: none"><li>• We are developing BBP-761, a preclinical program for the treatment of Leber's Hereditary Optic Neuropathy, or LHON</li></ul>
<b>Development Status and Catalysts</b>	<ul style="list-style-type: none"><li>• Ongoing – Lead optimization</li><li>• 2020 – Anticipated development candidate nomination</li></ul>
<b>Disease Overview</b>	<ul style="list-style-type: none"><li>• LHON is a rare mitochondrial disease of the eye, which manifests as rapidly progressive and severe loss of central vision predominantly in young adults</li><li>• Onset occurs most frequently in a single eye and is followed by the second eye, while bilateral presentation occurs in approximately 25% of cases. Most patients reach legal blindness several months after disease onset</li><li>• Caused by mutations in subunits of Complex I of the electron transport chain, a key protein complex for energy metabolism found in mitochondria, which results in mitochondrial dysfunction</li><li>• Prevalence of approximately 20,000 patients in the United States and European Union and incidence of approximately 500 new patients annually</li><li>• Patients are typically diagnosed based on clinical history and examination findings, and often confirmed with a genetic test</li></ul>

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- There are currently no treatments for the disease approved in the United States. Idebenone (Santhera) was approved in the European Union for LHON in 2015 under exceptional circumstances, as its pivotal trial did not meet its primary endpoint

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**Our Product Concept**

- We are developing an intravitreal injectable of a succinate pro-drug
- Increasing the levels of succinate is thought to restore mitochondrial function by upregulating Complex II activity, which is intended to compensate for Complex I dysfunction

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**Key Competitors**

- GS010, an AAV2 gene therapy vector encoding mitochondrial gene ND4
  - Elamipretide, a topical cardiolipin stabilizer
  - Idebenone, a small molecule antioxidant and electron carrier
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***Disease Overview***

LHON manifests as rapidly progressive and severe loss of central vision predominantly in young adults. Onset occurs most frequently in a single eye and is followed by the second eye, while bilateral presentation occurs in approximately 25% of cases. Most patients reach legal blindness within several months following disease onset. LHON is driven by mutations in subunits of Complex I of the electron transport chain, causing dysfunctional oxidative phosphorylation in the mitochondria (reduced mitochondrial function and energy production). This leads to degeneration of the retinal ganglion cells of the eye.

There are approximately 20,000 patients in the United States and European Union with LHON and there are approximately 500 new patients diagnosed annually.

***Our Product Concept***

BBP-761 is in preclinical development as a therapy for LHON for which there are no currently approved drug therapies in the United States. Succinate is a substrate of Complex II of the electron transport chain. As a succinate pro-drug, we believe BBP-761 may have potential to increase Complex II activity. The *in vitro* application of succinate pro-drugs to recessive NDUFS2-mutant fibroblast models of Complex I dysfunction has shown that the compounds increased oxygen consumption rate and normalized spare respiratory capacity, measures of mitochondrial function, through increasing Complex II activity. Our product concept is to provide a succinate prodrug to the retina through regular intravitreal injections in order to increase Complex II activity to compensate for Complex I dysfunction. We believe that BBP-761 has the potential to treat LHON if it is found to be effective in increasing mitochondrial activity.

***Development Plan***

BBP-761 is currently in the lead-optimization stage of preclinical development. We intend to pursue further preclinical development around ocular drug delivery as well as *in vitro* and *in vivo* efficacy and safety and intend to nominate a development candidate in 2020.

***Key Competitors***

GenSight Biologics S.A. is developing GS010, an intravitreal AAV2-ND4 gene therapy that is currently in Phase 3 clinical development for the treatment of LHON. Stealth BioTherapeutics Inc. is developing elamipretide, a peptide that stabilizes cardiolipin delivered through topical eye drops. The Phase 2 clinical trial of elamipretide did not reach its primary endpoint. Santhera Pharmaceuticals Holding AG received an exceptional circumstances marketing authorization from the EMA for Idebenone as a treatment for LHON in 2015.



**ONCOLOGY**

**BBP-398 (Navire): Targeting Multiple Oncology Indications**

**Summary**

- BBP-398 is a preclinical small molecule inhibitor of SHP2 for the potential treatment of cancers driven by hyperactive receptor tyrosine kinase, or RTK, or MAPK signaling

**Development Status and Catalysts**

- Ongoing – IND-enabling development
- 2020 – Anticipated IND submission

**Pathway Overview**

- SHP2 is a phosphatase that acts downstream of receptor tyrosine kinases in the MAPK signaling pathway. SHP2 is critical in signaling in these pathways. Increased MAPK signaling is a hallmark of a number of cancer types, including: cancers driven by RTK genetic alterations, cancers with RTK fusion mutations, and cancers with constitutively active MAPK signaling. Additionally, SHP2 is implicated as a downstream mediator of PD-1 signaling, a key target of immuno-oncology treatment

- A summary of key cancer types in which SHP2 signaling is implicated includes:

Category	Mutation	Key Cancer Types	U.S. Incidence
RTK Genetic Alterations	EGFR	NSCLC	35,000
	HER2	Breast cancer	35,000
RTK Fusions	ALK, RET, ROS1	NSCLC	23,000
Constitutively Active RAS/MAPK Signaling	NF-1 Loss of Function	Melanoma, NSCLC, Ovarian, Bladder, others	65,000
	KRAS Amplification	Esophageal, gastric, ovarian	9,000
	BRAF	Melanoma	45,000
PD-1/PD-L1 Treated Tumors	N/A	NSCLC, HNSCC, UC, Gastric, HCC, Cervical cancer, others	>100,000

**Our Product Concept**

- Oral small molecule, allosteric SHP2 inhibitor
- Designed to lock the SHP2 enzyme in its inactive conformation, abrogating signaling via the MAPK pathway, and hindering growth of cancers driven by overactivation of the MAPK pathway, either as a monotherapy or in combination with established tyrosine kinase inhibitors (TKI)
- We have developed highly potent compounds with pERK IC<sub>50</sub> in the 10-100nM range and anti-proliferative effects in KRAS-mutant pancreatic cancer, and RTK-driven esophageal squamous cell carcinoma cells *in vitro* and xenograft tumor models *in vivo*
- Designed to have limited activity against the hERG channel, which is implicated in cardiac toxicity, giving a high therapeutic index, which we believe will be particularly important for use in combination with other anti-cancer therapies in the clinic

<b>Key Competitors</b>	<ul style="list-style-type: none"> <li>• TNO-155 (Novartis International AG)</li> <li>• RMC-4630 (Revolution Medicines)</li> <li>• JAB-3068 (Jacobio Pharmaceuticals, Inc.)</li> <li>• Undisclosed preclinical asset (Relay Therapeutics, Inc.)</li> <li>• Undisclosed preclinical asset (Redx Pharma Plc)</li> </ul>
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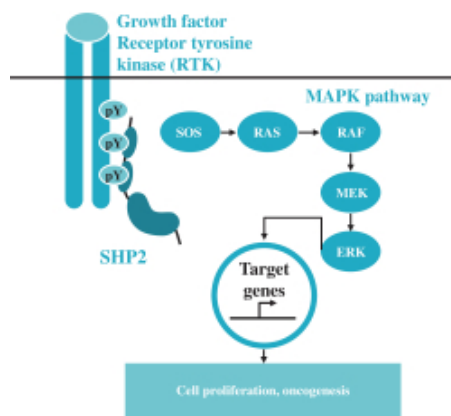
**Pathway Overview**

Cellular signaling networks governing cell proliferation, survival, adhesion and migration are frequently dysregulated in a variety of cancers. A key component of these networks are protein tyrosine phosphatases (PTPs), which are enzymes that remove phosphate groups from specific residues on substrate proteins, often changing their cellular location or function. SHP2 is an important member of this family, which has been shown to interact with growth factor receptors, scaffolding adaptor proteins, and immune inhibitory receptors. While key substrates remain incompletely validated, SHP-2 has been demonstrated to be a positive contributor to growth signaling pathways upstream of RAS and MAPK activity.

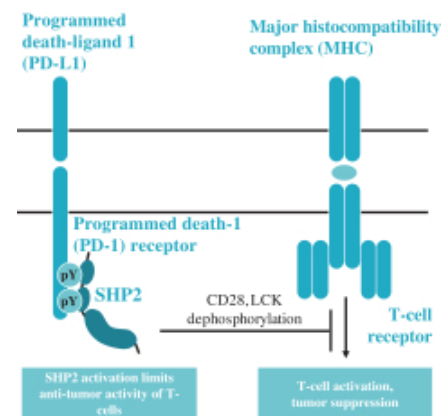
Upregulated MAPK signaling has been implicated as a driver of multiple cancer types, including cancers with RTK genetic alterations, RTK gene fusions, and cancers driven by constitutively active KRAS/MAPK signaling.

Outside of the MAPK pathway, active SHP2 limits T-cell response by binding to the receptor PD-1 and dephosphorylating CD28 and LCK, limiting signaling through the major histocompatibility complex (MHC). Reducing the amount of active SHP2 with an inhibitor may allow stronger T-cell activation, resulting in more effective tumor suppression.

**Inhibiting SHP2 may suppress tumors driven by excess RTK/MAPK signaling**



**Inhibiting SHP2 may sensitize tumors to immune checkpoint inhibition**



**Our Product Concept**

BBP-398 has been designed to have a higher therapeutic index and reduced off-target activity on hERG potassium channels, which are often implicated in cardiac toxicity. A higher therapeutic index is highly desirable in a SHP2 inhibitor to enhance tolerability when used in combination with other anti-cancer therapies.

Because of its involvement in signaling pathways aberrant in many cancers, we believe the inhibition of SHP2 activity is a promising therapeutic hypothesis applicable to multiple cancer types either as a monotherapy or in

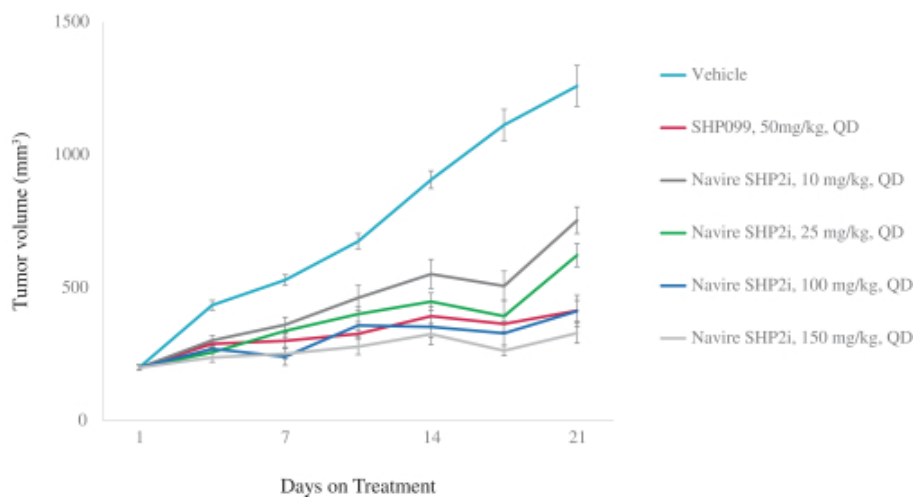
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combination with other drugs. Current molecules in development interact with the catalytic, N-SH2 and C-SH2 domains simultaneously to stabilize SHP2 in its closed, inactive conformation, reducing the amount of active SHP2 in the cell, and thus limiting its contribution to signaling pathways downstream.

Our therapeutic hypotheses have been supported by data from multiple preclinical studies including:

- Inhibition of RAS and MAPK signaling and strong anti-proliferative effects of SHP2 single agent treatment in KRAS G12C mutant cell lines *in vitro*
- Inhibition of MAPK signaling and tumor-stasis upon SHP-2 single agent treatment of KRAS G12C mutant MiaPaCa and RTK-driven KYSE xenograft models *in vivo*

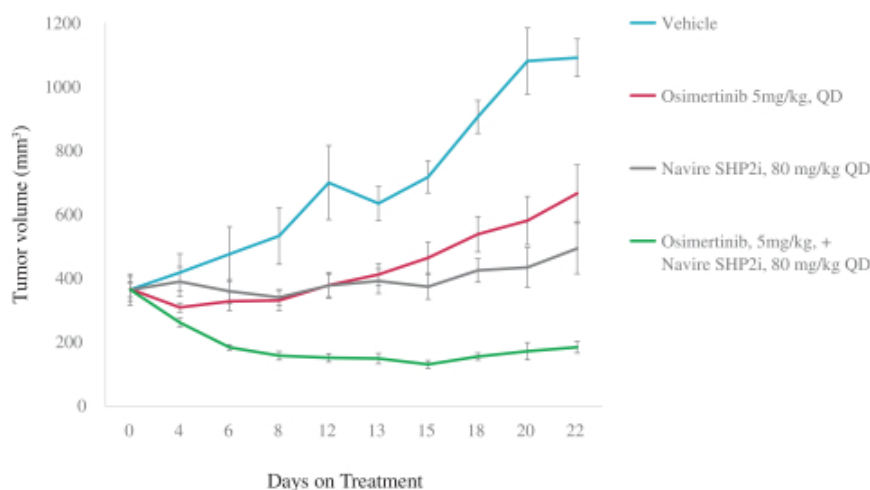
### **KYSE-520 xenograft tumor volumes upon treatment with Navire SHP2i QD**



Above, a representative experiment indicating dose-dependent anti-tumor efficacy of our second generation series in RTK-driven esophageal squamous cell carcinoma (KYSE-520) xenograft tumors over 21 days of dosing.

- Therapeutic synergy of SHP2 inhibition in combination with MEK inhibitor trametinib in MiaPaCa xenograft model *in vivo*
- Enhanced efficacy upon SHP2 inhibition in combination with anti-PD-1 therapy in H22 hepatocarcinoma xenograft model
- Restored sensitization of EGFR-mutated HCC827 NSCLC xenograft tumors to EGFR targeted therapy, osimertinib, upon SHP2 inhibition resulting in tumor regression

### Osimertinib resistant HCC827-ER1 xenograft tumor volumes upon treatment with osimertinib alone, Navire SHP2i alone or in combination



Above, a representative experiment demonstrating the first generation Navire SHP2 inhibitor sensitized RTK inhibitor, osimertinib, resistant NSCLC xenograft tumors to osimertinib when given in combination.

#### Development Plan

We recently nominated our development candidate, BBP-398, which is currently in IND-enabling development. BBP-398 was nominated from a series of second-generation SHP2 inhibitors that have been optimized for minimal off-target activity on hERG potassium channels, an off-target interaction implicated in cardiac toxicity.

In preclinical PK studies in rodents, dogs and monkeys, the second-generation series demonstrated favorable PK, which we believe may support once daily dosing in humans. To date, consistent with our in vitro hERG assays, no evidence of QT/QTc prolongation has been observed in a dog cardiovascular safety study with one of our second-generation SHP2 inhibitors.

Currently, IND-enabling studies are ongoing, and we anticipate filing an IND in 2020.

#### Key Competitors

Multiple third parties are in active development of SHP2 inhibitors with similar mechanisms of action. These include three molecules in Phase 1 clinical trials in patients with solid tumors. Novartis International AG is testing TNO-155 in advanced EGFR-mutant NSCLC and KRAS-mutant NSCLC, CRC, esophageal squamous cell cancer (SCC), and head and neck SCC, while Revolution Medicines, Inc. is testing RMC-4630 in advanced relapsed or refractory solid tumors. Jacobio Pharmaceuticals, Inc. is testing their SHP-2 inhibitor, JAB-3068, in a first in human dose escalation study in NSCLC, head and neck, and esophageal cancer in China and the United States. Two other programs are in preclinical development by Relay Therapeutics and Redx Pharma.

#### BBP-954 (Ferro): Multiple Oncology Indications

##### Summary

- BBP-954 is our preclinical discovery program for irreversible inhibitors of glutathione peroxidase 4, or GPX4, for the treatment of solid and hematological cancers

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|---|---|
| <b>Development Status and Catalysts</b> | <ul style="list-style-type: none"><li>• Ongoing – Lead optimization</li><li>• 2020 – Anticipated development candidate nomination</li></ul> |
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|-------------------------|--|
| <b>Pathway Overview</b> | <ul style="list-style-type: none"><li>• Ferroptosis is a form of oxidative programmed cell death that cancer cells must avoid in order to survive and form tumors</li><li>• GPX4 is an enzyme that protects cancer cells from ferroptosis by neutralizing toxic lipid free radicals. By inhibiting GPX4, we aim to trigger ferroptosis in cancer cells</li><li>• Preclinical data generated by us and third parties suggest many of the most common cancers are sensitive to GPX4 inhibition, both in monotherapy and combination with standard anti-cancer agents such as kinase inhibitors and chemotherapy</li><li>• Cancer types which may be treatable with a GPX4 inhibitor include:</li></ul> |
|-------------------------|--|

Key Cancer Types	U.S. Incidence
Renal Cell Carcinoma (RCC)	65,000
Non-Hodgkin's Lymphoma (NHL)	75,000
Pancreatic	55,000
Sarcoma	13,000
NSCLC	200,000
Breast	270,000
Melanoma	45,000

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| <b>Our Product Concept</b> | <ul style="list-style-type: none"><li>• Oral small molecule irreversible GPX4 inhibitor</li><li>• Based on <i>in vitro</i> preclinical data, we believe BBP-954 has the potential to be effective in many cancer types; sensitivity to BBP-954 <i>in vitro</i> (less than 100nM cellular IC<sub>50</sub>) included 100% of RCC, 94% of NHL, 92% of pancreatic, 88% of sarcoma</li><li>• Early derivatives of BBP-954 provided <i>in vivo</i> proof-of-concept with 50% tumor growth inhibition in a sarcoma xenograft model</li></ul> |
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| <b>Key Competitors</b> | <ul style="list-style-type: none"><li>• Bayer AG and the Broad Institute are collaborating to develop GPX4 inhibitors</li></ul> |
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#### **Pathway Overview**

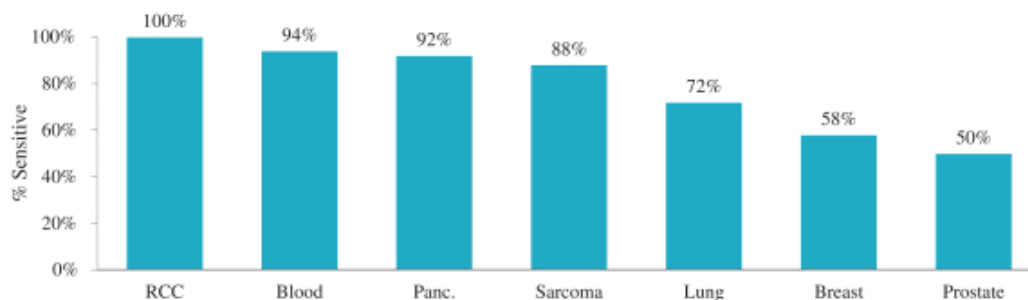
GPX4 is a lipid hydroperoxidase which protects cells from ferroptosis, a form of non-apoptotic cell death that is the result of toxic accumulation of lipid free radicals. Evasion of programmed cell death, including ferroptosis, is a hallmark of cancer and a growing body of evidence implicates GPX4 inhibition as a novel and potentially effective approach to treating cancer, both as a monotherapy and in combination with multiple standard anti-cancer agents including targeted kinase inhibitors and chemotherapy. Thus, we believe BBP-954 may be applicable in several common cancer types (see table above) which account for more than 500,000 new cases each year in the United States alone.

#### **Our Product Concept**

BBP-954 is our discovery program for a selective, oral, irreversible GPX4 inhibitors for the treatment of solid and hematologic cancer. Our preclinical data to date suggest that compounds in our BBP-954 program can be

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highly potent and specific GPX4 inhibitors and ferroptosis inducers. Internal *in vitro* and *in vivo* preclinical data, together with recent academic studies, suggest GPX4 inhibitors may have anti-tumor activity in many of the most common cancers (including lung, melanoma, pancreatic, kidney, lymphoma), as well as less common tumors such as bone and soft-tissue sarcomas, for which there are currently few effective treatment options. In a cell-based screen of more than 300 cancer cell-lines derived from more than 10 tumor types, greater than 70% of lines tested were sensitive to our compounds (sensitivity defined as IC<sub>50</sub> less than or equal to 100 nM). The most uniformly sensitive tumor types in our *in vitro* panel are summarized below:



Cell-based screen of more than 300 cancer cell-lines derived from more than 10 tumor types. Samples were considered sensitive if they showed an IC<sub>50</sub> of 100 nM or less.

We established preliminary *in vivo* proof-of-concept data in a sarcoma xenograft model using one of our initial compounds, which showed a 50% decrease in tumor growth relative to vehicle treated animals. The compound was well-tolerated over the 28-day dosing period (measured by change from baseline in body weight) despite clear target engagement in several normal tissues including kidney, liver, lung, heart and pancreas. Additionally, multiple independent academic studies have shown tumor regression using genetic inactivation of GPX4, including xenograft models of RCC, melanoma and sarcoma. Work at leading academic centers has also generated preclinical data suggesting the potential for synergy between GPX4 inhibition and major classes of anti-cancer agents, including chemotherapy, targeted therapy and immuno-oncology.

### Development Plan

We are currently in the lead-optimization stage of preclinical development. We anticipate nominating a development candidate in 2020.

### Key Competitors

Bayer AG and the Broad Institute are developing small molecule inhibitors of GPX4. We are not aware of any other active GPX4 inhibitor discovery programs.

## GENE THERAPY

### BBP-812 (Aspa): Canavan Disease

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<b>Summary</b>	<ul style="list-style-type: none"><li>We are developing BBP-812, a preclinical AAV, gene transfer therapy candidate, for the treatment of Canavan Disease</li></ul>
<b>Development Status and Catalysts</b>	<ul style="list-style-type: none"><li>Ongoing – IND-enabling development</li><li>2020 – Anticipated IND submission</li></ul>

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<b>Disease Overview</b>	<ul style="list-style-type: none"><li>• Canavan Disease is a fatal, progressive neurodegenerative disorder that begins in infancy. The disease is a leukodystrophy, caused by degradation of white matter in the brain. Patients typically miss developmental milestones, have a rapidly increasing head circumference, progressive lack of motor control, and often do not live past their mid-teens</li><li>• Caused by inactivating mutations in the gene encoding aspartocylase (ASPA) that normally catalyzes diacylation of N-acetyl-l-aspartate (NAA) into aspartate and acetate. Results in elevated NAA levels</li><li>• Incidence of one in 100,000 births worldwide</li><li>• Diagnosis: Clinical suspicion after missed developmental milestones is confirmed by elevated NAA in urine</li><li>• No treatments approved for Canavan Disease, care is focused on symptom management</li></ul>
<b>Our Product Concept</b>	<ul style="list-style-type: none"><li>• Designed to replace ASPA gene with AAV9 gene therapy restoring missing enzymatic activity</li><li>• BBP-812 uses a self-complementary AAV9 vector</li></ul>
<b>Key Competitors</b>	<ul style="list-style-type: none"><li>• We are not currently aware of any key competitors</li></ul>

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***Disease Overview***

Canavan Disease is a fatal, progressive neurodegenerative disorder that begins in infancy. The disease is a leukodystrophy, caused by degradation of white matter in the brain, which prevents proper synaptic communication of neurons. Canavan Disease typically presents within three to five months of birth with missed or regressed developmental milestones, a rapidly increasing head circumference, lack of head and motor control, seizures, low tone, which then progresses to spasticity, and sometimes blindness. Many of the patients do not live past their mid-teens, though some do survive longer.

Canavan Disease is exceedingly rare, with an estimated prevalence of less than approximately one in 100,000 births, corresponding to approximately 90 births annually in the United States and Europe. The current total estimated disease population is around 1,000 in the United States and Europe, some of whom are catalogued in patient registries and patient advocacy groups. Current treatment is limited to supportive symptomatic care, and there are no drugs currently approved or registered in clinical development for Canavan disease.

Canavan Disease is caused by a deficit in the ASPA gene, which encodes Aspa, an enzyme that catalyzes the diacylation of NAA into aspartate and acetate. Though the exact molecular pathophysiology of the disease has not yet been elucidated, significant demyelination in the deep cortical white matter is a hallmark of the disease. Additionally, Aspa inactivity results in a significant increase in urinary NAA, which can be quantified by mass spectrometry and confirm diagnosis.

***Our Product Concept***

BBP-812 is a self-complementary AAV, or AAV9 gene transfer product candidate intended to treat Canavan Disease by replacing the aspartocylase enzyme. The vector uses a CB6 promoter, which is constitutively active. A recent publication using the same vector construct showed that expression of Aspa was sufficient to rescue the phenotype and lifespan of mice lacking the ASPA gene. Data in a knockout mouse model of Canavan Disease showed strong evidence of activity out to one year, which was the latest time point evaluated. No notable adverse effects were observed, and the therapy at the high dose restored survival, motor function, and NAA to wild-type

levels. While little data have been presented publicly, a single compassionate use case performed at the University of Florida for a Canavan Disease patient treated with simultaneous intravenous and intracerebroventricular injection of rAAV9-CB6-ASPA was associated with improved functional outcomes in a 6-month update as measured by motor abilities. In a Phase 1 clinical trial for Spinal Muscular Atrophy Type 1, AAV9 was well-tolerated in humans at doses as high as  $2 \times 10^{14}$  vector genomes per kilogram.

#### ***Development Status***

Following ongoing route-of-administration, dose-finding, and IND-enabling toxicology, we expect to file an IND submission for BBP-812 in 2020. Given the small number of patients and the extremely high unmet need, we intend to explore an abbreviated and accelerated clinical development plan, as well as to pursue a rare pediatric disease designation from the FDA.

#### ***Key Competitors***

We are not aware of commercially active small molecule or conventional biologic programs aimed at treating Canavan Disease. A gene therapy developed by Dr. Paula Leone and licensed to Bamboo Therapeutics (acquired by Pfizer Inc.) was in preclinical development at the time of the Bamboo acquisition, but its current development status is unknown.

#### **Additional Program Related Information**

##### **Manufacturing**

We do not own or operate, and currently have no plans to establish, any manufacturing facilities. We currently depend on third-party CMOs, for all of our requirements of raw materials, drug substance and drug product for our preclinical research and our ongoing clinical trials of our product candidates. We have not entered into long-term agreements with our current CMOs. We intend to continue to rely on CMOs for later-stage development and commercialization of our product candidates, including any additional product candidates that we may identify. Although we rely on CMOs, we have personnel and third-party consultants with extensive manufacturing experience to oversee the relationships with our contract manufacturers. Several of our development candidates have or are in the near term expected to have redundant and overlapping drug substance and drug product supply chains.

##### ***BBP-631 (Adrenas), and BBP-812 (Aspa)***

We are working with a leading contract manufacturer to produce our vector for toxicology experiments and our first-in-human clinical trial. We believe this contract manufacturer can scale all the way through commercial production. Currently, our vector is being produced in a HEK293 triple transfection suspension system for both AAV programs.

##### ***BBP-009 (PellePharm)***

BBP-009, or patidegib, is a semi-synthetic small molecule derived from a natural product raw material, cyclopamine. The current source of cyclopamine is from the wild harvest of *Veratrum californicum*, or corn lily, in the western United States. The roots of the plant are harvested on private and federal land by a third-party contractor who holds contracts and/or permits for harvesting *Veratrum californicum* with private landholders and the U.S. Forest Service. After drying and milling, cyclopamine is isolated and purified from the resulting biomass. Cyclopamine is then converted to patidegib in twelve chemical steps at two contract manufacturing facilities. Patidegib is formulated as a hydro-alcoholic gel for topical administration and packaged in laminate tubes. Ongoing R&D studies are aimed at developing a process to produce cyclopamine using plant cell fermentation as an alternative source of cyclopamine.



PellePharm, Inc. currently has development and manufacturing contracts and quality agreements with multiple CMOs for the harvest of *Veratrum californicum*, and the manufacturing of cyclopamine, drug substance and drug product. We anticipate that these CMOs will have capacity to support commercial scale production.

#### ***BBP-598 (Phoenix Tissue Repair)***

We believe the current inventory of BBP-598 drug product is sufficient to complete the ongoing Phase 1/2 proof-of-concept clinical trial. Selection of a CMO to support late-stage development, late-phase clinical supply, and commercial supply is ongoing, and we expect to select a CMO in the first quarter of 2019. Late-stage development activities will be focused on process establishment, facility-fit, and cost of goods optimization. We have acquired/licensed a portfolio of patents that relates to the composition and production of rCOL7 and support manufacture of product for commercial purposes.

#### **Sales and Marketing**

We intend to begin building a commercial infrastructure in the United States and selected other territories to support the commercialization of each of our product candidates when we believe a regulatory approval in a particular territory is likely. Because most of our target indication are rare diseases with a concentrated prescribing audience and a small number of key opinion leaders who influence the treatments prescribed for the relevant patient population, we currently believe that we can effectively address each market using our own targeted, specialty sales and marketing organization supported by internal sales personnel, an internal marketing group and distribution support.

We intend to evaluate our commercialization strategy as we advance each product candidate through clinical development. In any core markets outside of the United States that we may identify, where appropriate, we may utilize strategic partners, distributors or contract sales forces to expand the commercial availability of our product candidates. We currently do not expect that we will require large pharmaceutical partners for the commercialization of any of our product candidates, although we may consider partnering in certain territories or indications or for other strategic purposes.

#### **Intellectual Property**

We strive to protect the proprietary technology that we believe is important to our business, including seeking and maintaining patents and patent applications intended to cover our product candidates and compositions, their methods of use and processes for their manufacture, and any other aspects of inventions that are commercially important to the development of our business. We have entered into various license agreements to obtain the rights to use certain patents for the development and commercialization of our product candidates. See “—Our Material Agreements.” We also rely on trade secrets to protect aspects of our business that are not amenable to, or that we do not consider appropriate for, patent protection.

Our success will depend on our ability to obtain and maintain patent and other proprietary rights protecting our commercially important technology, inventions and know-how related to our business, defend and enforce our current and future issued patents, if any, preserve the confidentiality of our trade secrets and operate without infringing the valid and enforceable patents and proprietary rights of third parties. We also rely on know-how, continuing technological innovation and potential in-licensing opportunities to develop and maintain our intellectual property portfolio. We seek to obtain domestic and international patent protection, and endeavor to promptly file patent applications for new commercially valuable inventions.

The patent positions of biopharmaceutical companies like us are generally uncertain and involve complex legal, scientific and factual questions. In addition, the coverage claimed in a patent application can be significantly reduced before the patent is issued, and patent scope can be reinterpreted by the courts after issuance. Moreover, many jurisdictions permit third parties to challenge issued patents in administrative proceedings, which may

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result in further narrowing or even cancellation of patent claims. We cannot predict whether the patent applications we are currently pursuing will issue as patents in any particular jurisdiction or whether the claims of any patents, if issued, will provide sufficient protection from competitors.

Because patent applications in the United States and certain other jurisdictions are maintained in secrecy for 18 months or potentially even longer, and since publication of discoveries in the scientific or patent literature often lags behind actual discoveries, we cannot be certain of the priority of inventions covered by pending patent applications. Moreover, we may have to participate in interference proceedings or derivation proceedings declared by the United States Patent and Trademark Office, or USPTO, to determine priority of invention.

### *QED Therapeutics, Inc.*

For our subsidiary, QED Therapeutics, Inc., we license rights from Novartis to two issued U.S. patents, and related pending and issued foreign patents and patent applications in Australia, Canada, China, Europe, Japan and Mexico, as well as in other countries in Asia and in South America, that are directed to compositions of matter of BBP-831. The issued U.S. patents are expected to expire between 2026 and 2029, which takes into account patent term adjustments granted by the USPTO. The foreign patents and patent applications, if issued, are expected to expire between 2025 and 2030.

We also license rights from Novartis to one issued U.S. patent, one pending U.S. patent application, and related pending and issued foreign patents and patent applications in Australia, Canada, China, Europe, Japan and Mexico, as well as in other countries in Asia and in South America, that are directed to pharmaceutical formulations containing BBP-831. The issued patents and patent applications, if issued, are expected to expire in 2034.

We also license rights from Inserm Transfer ESA and Assistance Publique-Hôpitaux de Paris to one issued U.S. patent and one pending U.S. patent application, and one granted patent in Europe, that are directed to methods of treating achondroplasia using BBP-831. The issued U.S. patent, granted patent in Europe, and the pending patent application, if issued, are expected to expire in 2032.

### *Eidos Therapeutics, Inc.*

For our subsidiary Eidos Therapeutics, Inc., we license rights from the Board of Trustees of the Leland Stanford Junior University, or Stanford, to five issued U.S. patents with claims directed to composition of matter and methods of use relating to BBP-265. These patents are expected to expire in 2031 or 2033. We also license rights from Stanford to one pending U.S. patent application, one issued European patent, one pending European patent application, and one issued Japanese patent with claims directed to composition of matter and methods of use relating to BBP-265. These patents and patent applications, if issued, are expected to expire in 2031 or 2033.

In addition, we own a pending U.S. patent application, a pending PCT patent application, and a patent application pending in Taiwan with claims directed to salt and solid forms relating to BBP-265. These patent applications and any patent applications claiming the benefit of the PCT patent application, if issued, are expected to expire in 2038.

### *Origin Biosciences, Inc.*

For our subsidiary Origin Biosciences, Inc., we own two issued U.S. patents with claims directed to methods of use and manufacturing processes relating to BBP-870, and one issued Canadian patent and one pending Canadian patent application that relates to BBP-870. The issued U.S. and Canadian patents are expected to expire in 2025 or 2032. The pending Canadian patent application, if used, is expected to expire in 2032.

### *PellePharm, Inc.*

PellePharm, Inc. owns two pending U.S. patent applications with claims directed to topical formulations and methods of use, including treating basal cell carcinoma relating to BBP-009, and over 10 related foreign patent

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applications pending in various jurisdictions, including Australia, Canada, Europe, Japan, and Mexico. These patent applications, if issued, are expected to expire in 2036.

Additionally, PellePharm, Inc. has licensed rights from Infinity Discovery Inc., or Infinity Pharmaceuticals, Inc. and the Johns Hopkins University, to nine issued U.S. patents with claims directed to composition of matter and methods of use relating to BBP-009, three related pending U.S. patent applications, over 60 related issued foreign patents in various jurisdictions, including Australia, Canada, China, Europe (validated in various countries), Japan, Korea, Mexico, and Singapore, and over four related foreign patent applications pending in various jurisdictions, including Argentina, Brazil, Thailand, and the United Arab Emirates. The issued U.S. and foreign patents are expected to expire between 2025 and 2032. The pending U.S. and foreign patent applications, if issued, are expected to expire in 2027.

### *Adrenas Therapeutics, Inc.*

For our subsidiary Adrenas Therapeutics, Inc., we own one pending PCT patent application with claims directed to recombinant AAV vectors relating to BBP-631. Any patent applications claiming the benefit of this PCT patent application, if issued, are expected to expire in 2039.

### *Aspa Therapeutics, Inc.*

For our subsidiary, Aspa Therapeutics, Inc., we license rights from the University of Massachusetts to one issued U.S. patent with claims directed to methods of treating Canavan disease using BBP-812, three related pending U.S. patent applications, three related foreign patents issued in various jurisdictions, including Europe and Japan, and four related foreign patent applications pending in various jurisdictions, including Canada and Europe. The issued U.S. patent is expected to expire in 2031. The pending U.S. patent applications, if issued, are expected to expire between 2031 and 2036. The foreign patents and any patents issuing from the foreign patent applications are expected to expire between 2031 and 2036.

### *CoA Therapeutics, Inc.*

For our subsidiary, CoA Therapeutics, Inc., we license rights from St. Jude Children's Research Hospital, Inc., or St. Jude, to one pending U.S. patent application with claims directed to composition of matter and methods of use relating to BBP-671, and over 10 related foreign patent applications pending in various jurisdictions, including Australia, Canada, China, Europe, Japan, and Mexico. These patent applications, if issued, are expected to expire in 2037.

Additionally, CoA Therapeutics, Inc., co-owns with St. Jude, three pending PCT patent applications directed to composition of matter and methods of use relating to BBP-671. Any patent applications claiming the benefit of these PCT patent applications, if issued, are expected to expire in 2038.

### *Ferro Therapeutics, Inc.*

For our subsidiary, Ferro Therapeutics, Inc., we own one pending U.S. patent application and one pending PCT patent application, directed to compositions of matter and methods of use relating to BBP-954. The pending U.S. patent application and any patent applications claiming the benefit of the PCT patent application, if issued, are expected to expire in 2039.

### *Fortify Therapeutics*

For our subsidiary, Fortify Therapeutics, we license rights from NeuroVive Pharmaceutical AB, in the field of LHON for use directly in the eye, to one pending U.S. patent application with claims directed to compounds, one related issued European patent, and over 12 related foreign patent applications pending in various jurisdictions, including Australia, Canada, China, Japan, and Mexico. The issued European patent, and pending U.S. patent application and foreign patent applications, if issued, are expected to expire in 2035.

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### *Navire Pharma, Inc.*

For our subsidiary, Navire Pharma, Inc., we license rights from The Board of Regents of the University of Texas System, or the Board of Regents, to one issued U.S. patent, one pending U.S. patent application and five related foreign patent applications pending in Australia, Canada, China, Europe, and Japan with claims directed to composition of matter and methods of use relating to our first generation SHP2 inhibitors. The issued U.S. patent and the patent applications, if issued, are expected to expire in 2037. We also license rights from the Board of Regents to two U.S. provisional applications with claims directed to composition of matter of BBP-398. Patent applications claiming the benefit of and priority to these U.S. provisional patent applications, if issued, are expected to expire in 2039.

### *Orfan Biotech, Inc.*

For our subsidiary Orfan Biotech, Inc., we own one pending PCT patent application and two pending U.S. provisional patent applications with claims directed to glycolate oxidase inhibitor compounds and methods of use thereof. Any patent applications claiming the benefit of the PCT patent application, if issued, are expected to expire in 2038. Any patent applications claiming the benefit of the U.S. provisional patent applications, if issued, are expected to expire in 2039 or 2040.

### *Phoenix Tissue Repair, Inc.*

For our subsidiary Phoenix Tissue Repair, Inc., we license rights from the University of Southern California, or USC, to one issued U.S. patent with claims directed to polypeptides comprising functional fragments of collagen 7, and four pending U.S. patent applications with claims directed to methods of use, including treating epidermolysis bullosa with collagen 7. The issued U.S. patent is expected to expire in 2035 and the pending U.S. patent applications, if issued, are expected to expire between 2027 and 2033. We also license rights from USC to four related foreign patents issued in various jurisdictions including Australia, Europe and Japan, and over seven related foreign patent applications pending in various jurisdictions including Australia, Canada, Europe, and Japan. The foreign patents and patent applications, if issued, are expected to expire between 2027 and 2035.

We also own an issued U.S. patent with claims directed to collagen 7 modification for enhancing the degradability of collagen, and related issued patents in the United Kingdom, France and Germany. The issued U.S. patent is expected to expire in November 2019. The issued patents in the United Kingdom, France and Germany are expected to expire in 2022.

We also own one pending U.S. patent application with claims directed to methods of treating epidermolysis and chronic skin wounds with collagen 7, with one related issued patent in Australia, and two related foreign applications pending in Canada and Europe. The issued patent and related patent applications, if issued, are expected to expire in 2033.

We also own a pending U.S. patent application with claims directed to formulations comprising collagen 7 and seven related foreign patent applications pending in various jurisdictions, including Canada, China, Europe, Japan, and Mexico. These patent applications, if issued, are expected to expire in 2036.

### *TheRas, Inc.*

For our subsidiary TheRas, Inc., we license rights from The Regents of the University of California, or the University of California, and Leidos Biomedical Research, Inc., or Leidos, one pending U.S. patent application with claims directed to modulators of K-RAS, which include claims to the modulators as composition of matter and their use in therapy, including the treatment of cancer, and over ten related foreign patent applications pending in various jurisdictions, including Australia, Canada, China, Europe, Japan, and Mexico. The U.S. patent application and foreign patent applications, if issued, are expected to expire in 2036. We also license rights to one pending PCT patent application. Any patent applications claiming the benefit of this PCT application, if issued, are expected to expire in 2038. Additionally, we co-own with, and license rights from, the University of California and Leidos, three pending PCT applications. If issued, any patent applications claiming the benefit of these PCT applications are expected to expire in 2039.

*Venthera, Inc.*

For our subsidiary Venthera, Inc., we license rights from the Memorial Sloan Kettering Cancer Center to one pending U.S. patent application with claims directed to methods of treating vascular malformation and five related foreign patent applications pending in Australia, Canada, Europe, Israel and New Zealand. These patent applications, if issued, are expected to expire in 2036. We also own one U.S. provisional patent application directed to new compounds, compositions/formulations and methods for treating vascular malformation. Any patent applications claiming priority to this U.S. provisional patent application, if issued, are expected to expire in 2040.

**Our Material Agreements**

***BBP-265: License Agreement with the Board of Trustees of the Leland Stanford Junior University***

In April 2016, through our subsidiary Eidos Therapeutics, Inc., we entered into an exclusive license agreement with Stanford for rights relating to novel transthyretin aggregation inhibitors. Under our agreement, Stanford has granted us an exclusive worldwide license to make, use and sell products that are covered by the licensed patent rights. This license grant expires when the last licensed patent expires. The patent rights exclusively licensed to us under the license are described in more detail above under the heading “—Intellectual property—BBP-265.”

Stanford retains the right, on behalf of itself and all other non-profit academic research institutions, to practice under the patent rights for any non-profit purpose, including sponsored research and collaborations. We may grant sublicenses to third parties so long as we are actively pursuing the development or commercialization of products covered by the patent rights. We may also be required to sublicense our rights under the agreement at Stanford’s request under certain conditions, including if we are unwilling or unable to serve a potential market or territory and there is a third party willing to be a sublicensee in such market or territory.

We are obligated to pay to Stanford a yearly license maintenance fee during the term of the agreement, but we may offset the maintenance fee against earned royalty payments due on net sales occurring in that year. Stanford is entitled to receive a royalty as a percentage of net sales of licensed products, in the low single digits. We have agreed to pay Stanford a percentage of non-royalty revenue we receive from our sublicensees, with the amount owed decreasing annually for three years based on when we enter into the applicable sublicense agreement. In addition, we are obligated to pay Stanford up to approximately \$1.0 million upon the achievement of specific intellectual property, clinical and regulatory milestone events. In the event of a change of control transaction with respect to Eidos, we are obligated to pay Stanford a change of control fee of \$250,000 in connection with the assignment of the license agreement to the acquirer of Eidos.

Under the license agreement with Stanford, we are obligated to use commercially reasonable efforts to develop, manufacture, and commercialize at least one licensed product; to develop markets for such licensed products; and to meet certain development milestones as agreed upon between us and Stanford.

Subject to the expiration of the license grant described above, the agreement does not have a specified term. We may terminate the agreement by providing prior written notice to Stanford, and Stanford has the right to terminate the agreement if we fail to achieve certain milestones or make payments under the agreement, or are not actively pursuing development of a licensed product, or if we otherwise materially breach the agreement and fail to cure such breach within a specified grace period.

***BBP-831: License Agreement with Novartis International Pharmaceutical Ltd.***

In January 2018, through our subsidiary QED Therapeutics, Inc., or QED, we entered into a license agreement with Novartis International Pharmaceutical Ltd., or Novartis, for certain intellectual property rights, including patents and know-how, related to BBP-831 for the treatment of patients with FGFR-driven diseases, including CCA, UC and achondroplasia. We refer to this agreement as the Novartis License.

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Pursuant to the Novartis License, we obtained a license to research, develop, make, have made, use, import, offer for sale, sell, have sold and otherwise commercialize BBP-831, as well as therapeutic products incorporating BBP-831 that would, but for the license grant, infringe Novartis' license patent rights, or that were developed using or that incorporate or embody Novartis' licensed know-how, in all fields of use worldwide. The license grant to us includes the right to sublicense through multiple tiers. We also have certain rights to intellectual property licensed to Novartis' affiliate under a materials transfer agreement with a third party.

The Novartis License is subject to Novartis' existing obligations to supply a third party with BBP-831 to support the third party's clinical trials, and we have an ongoing obligation to inform Novartis of our or our sublicensees' intent to seek regulatory approval for and commercialize BBP-831 for various indications, with potential reversionary rights to Novartis in the event of a subsequent decision not to seek regulatory approval and commercialization, or a determination by Novartis that we have failed to sufficiently pursue regulatory approval and commercialization, for Novartis to grant such third party limited rights to develop and commercialize BBP-831.

Under the terms of the Novartis License, we made a one-time payment of \$15.0 million to Novartis and agreed to issue shares of Series A preferred stock of QED valued at approximately \$1.7 million in the aggregate to Novartis. In addition, we are obligated to make contingent milestone payments totaling \$60.0 million upon achievement of certain regulatory milestones. We are also obligated to make contingent milestone payments totaling \$35.0 million upon achievement of certain sales milestones for therapeutic products incorporating BBP-831. QED also agreed to pay Novartis tiered low double-digit royalties on net sales of therapeutic products incorporating BBP-831.

Under the Novartis License, we are required to use commercially reasonable efforts to develop BBP-831, and to obtain regulatory approval for and commercialize BBP-831 in the United States and the European Union.

We may terminate the Novartis License in its entirety or on a product-by-product or country-by-country basis at any time with 60 days' prior written notice to Novartis. Novartis may terminate if QED ceases to function as a going concern, is the subject of certain bankruptcy or similar proceedings, or otherwise winds down or discontinues its business. Either party may terminate for material breach that is not cured by the other party within a specified time period of receiving notice of such material breach. Otherwise, the Novartis License terminates on a product-by-product and country-by-country basis on the latest of the expiration of licensed patent rights, the expiration of regulatory exclusivity, or the tenth anniversary of the first commercial sale in such country.

### ***BBP-870: Asset Purchase Agreement with Alexion Pharma Holding Unlimited Company***

In June 2018, through our subsidiary Origin Biosciences, Inc., we entered into an asset purchase agreement with Alexion Pharma Holding Unlimited Company, or Alexion, pursuant to which we acquired Alexion's right, title and interest in certain assets relating to BBP-870, including patents and other intellectual property rights.

In the event that a Priority Review Voucher, or PRV, is granted to us by the FDA, we have agreed to pay Alexion a percentage in the mid-teens of any proceeds received by us by from our sale of the PRV to a third party. If we do not sell the PRV to a third party within 180 days after our receipt of the PRV, we are obligated to pay Alexion \$18.75 million, which amount is creditable against any amounts otherwise due to Alexion in accordance with the preceding sentence upon any future sale by us of the PRV. We are obligated to make contingent milestone payments totaling \$3.0 million upon achievement of certain development milestones and \$17.0 million upon achievement of certain sales milestones for products containing the BBP-870 molecule. We also agreed to pay Alexion tiered royalties ranging from the low-to mid-teens on net sales of products containing the BBP-870 molecule.

We are obligated to use commercially reasonable efforts to obtain a PRV, achieve specified milestone events and commercialize at least one product containing the BBP-870 molecule after receipt of regulatory approval.

### ***BBP-009 (Patidegib): Option Agreement with LEO Pharma A/S***

In November 2018, through PellePharm, Inc., or PellePharm, we entered into an option agreement with LEO Pharma A/S, or LEO Pharma, and LEO Spiny Merger Sub, Inc., pursuant to which LEO Pharma was granted an

exclusive, irrevocable option to acquire PellePharm. The option is exercisable by LEO Pharma on or before the occurrence of certain events relating to PellePharm's clinical development programs, and in no event later than July 30, 2021. As consideration for the option, LEO Pharma paid to PellePharm exclusivity payments totaling approximately \$27.9 million in the aggregate and purchased a minority equity interest in PellePharm for approximately \$5.1 million. In addition, LEO Pharma has agreed to pay additional exclusivity payments to PellePharm in an amount not to exceed \$37.0 million in the aggregate under certain circumstances.

Pursuant to the option agreement, we have agreed to conduct the business of PellePharm in the ordinary course and in accordance with applicable laws, comply with the terms of our organizational documents, and use commercially reasonable efforts to operate the business of PellePharm in accordance with a mutually agreed budget and to complete a Phase 2 clinical trial of patidegib for HF-BCC and a Phase 3 clinical trial of patidegib for Gorlin Syndrome. In addition, we and LEO Pharma have formed a joint development committee to oversee the development of, and to make decisions regarding the commercialization of, patidegib.

***BBP-589: Asset Purchase Agreement with Shire Human Genetic Therapies, Inc. and Lotus Tissue Repair***

In July 2017, through our subsidiary, Phoenix Tissue Repair, Inc. or Phoenix, we entered into an asset purchase agreement with Shire Human Genetic Therapies, Inc., or Shire, and Lotus Tissue Repair, Inc. or Lotus, pursuant to which we acquired from Shire and Lotus the right, title and interest in certain assets relating to recombinant human collagen type VII, including patents and other intellectual property rights, as well as data and regulatory filings, relating to the treatment of DEB, and assumed certain liabilities with respect thereto. In connection with the acquisition of such assets, (1) Shire and Lotus granted to us a non-exclusive, worldwide, irrevocable, perpetual, royalty-free, sublicenseable license under certain intellectual property related to the acquired assets but retained by Shire and Lotus, for the exploitation of certain recombinant human collagen type VII products in all fields, and (2) we granted to Shire and Lotus a non-exclusive, worldwide, irrevocable, perpetual, royalty-free, sublicenseable license under certain of the acquired intellectual property assets to exploit products other than recombinant human collagen type VII products and other than for the treatment of DEB in humans.

As partial consideration for our acquisition of the assets, we agreed to pay a purchase price of \$1.5 million and issued shares of common stock in Phoenix at a nominal value to Lotus. We are obligated to make contingent milestone payments totaling \$27.0 million upon achievement of certain regulatory milestones. In addition, we are obligated to make contingent milestone payments totaling \$60.0 million upon achievement of certain sales milestones. We also agreed to pay to Shire and Lotus tiered single-digit royalties on annual net sales for products containing the recombinant human collagen type VII.

We are obligated to use commercially reasonable efforts to develop, obtain FDA approval for and commercialize at least one product for the treatment of DEB in humans.

***BBP-454: License Agreement with Regents of The University of California***

In September 2016, through our subsidiary TheRas, Inc., or TheRas, we entered into a license agreement with the Regents of the University of California, or UCSF, which was amended in January 2017, August 2017 and September 2018, relating to certain patent rights related to KRAS inhibitors and modulators, which we refer to collectively as the UCSF License.

Under the UCSF License, we acquired an exclusive, royalty-bearing, sublicenseable (through multiple tiers), worldwide license to make, have made, use, sell, offer for sale and import products, services, and methods covered by the licensed patent rights, and to perform licensed processes, in each case, in prophylactic and therapeutic uses in humans. In addition, we received an option for certain inventions conceived and reduced to practice during a specified term. Under the UCSF License, UCSF retains, on behalf of itself and a third party, the right to make, use and practice certain of the licensed intellectual property rights for research and educational purposes, and the right to license to other academic and nonprofit organizations to practice the patent rights for research and educational purposes, including with respect to sponsored research performed on behalf of

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commercial entities. The rights and interests of any such commercial entity shall be subject to the licenses granted to us pursuant to the UCSF License. The UCSF License is also subject to pre-existing rights of the U.S. Government and the NIH.

In connection with the UCSF License and subsequent amendments, we paid issue fees totaling \$300,000. In addition, under the terms of the UCSF License, we are required to pay to UCSF certain annual license maintenance fees unless we are selling or otherwise exploiting licensed products or services paying royalties to UCSF on net sales for such licensed products or services. With respect to such royalty obligations, we agreed to pay UCSF low single-digit tiered royalties on annual net sales of licensed products and services, with a minimum royalty requirement of \$100,000. Our obligation to pay royalties continues on a country-by-country basis until the expiration of all licensed patent rights covering licensed products in such country. In addition, we are obligated to make contingent milestone payments totaling up to \$22.4 million upon the achievement of certain clinical or regulatory milestones. In the event that we sublicense the patent rights, UCSF is also entitled to receive a percentage of the sublicensing income received by us.

We are also required to make a one-time "Index Milestone Payment" to UCSF in the event of (i) an initial public offering, or (ii) a change of control transaction, in each case with respect to TheRas. Such Index Milestone Payment is calculated by multiplying (a) a number of shares equal to a specified percentage of the then-outstanding fully-diluted shares of common stock of TheRas by (b)(1) in the case of an initial public offering by TheRas, the offering price per share of the securities sold to the underwriters in the offering, or (2) in the case of a change of control transaction with respect to TheRas, the per share consideration that would be received by TheRas' shareholders in such transaction, in each case subject to certain adjustments. To the extent that an Index Milestone Payment becomes due prior to a bona fide financing transaction of at least \$45 million, such Index Milestone Payment is equal to the greater of the amount calculated as described above, or \$1.8 million.

Under the UCSF License, we also assumed certain obligations with respect to fund-raising, and must report on our progress in achieving the milestones set forth in the UCSF License on a periodic basis. The UCSF License also includes certain participation rights pursuant to which UCSF has the right to purchase specified amounts of securities offered by TheRas in financing transactions.

Under the UCSF License, we are obligated to diligently proceed with the development, manufacture and sale of at least one licensed product and/or service, and to earnestly and diligently market such licensed product and/or service after receipt of any requisite regulatory approvals and in quantities sufficient to meet market demand. We are also required to use good faith and diligent efforts to meet the milestones set forth in the UCSF License, subject to any revisions that may be permitted under certain circumstances. UCSF has the right to either terminate the UCSF License or reduce the license to a nonexclusive license if we are unable to perform our diligence obligations.

The agreement will continue until the last to expire or abandonment of the patent rights on a licensed product-by-licensed product and country-by-country basis. We may terminate the agreement by providing prior written notice to UCSF or we may terminate the rights under patent rights on a country-by-country basis by giving notice in writing to UCSF. UCSF has the right to terminate the agreement if we fail to make any payments, challenge any UCSF patent rights or otherwise materially breach the agreement and fail to cure such breach within a specified grace period.

### ***BBP-398: Collaboration and License Agreement with the Board of Regents of The University of Texas System and The University of Texas M.D. Anderson Cancer Center***

In March 2017, through our subsidiary Navire Pharma, Inc. (formerly known as PTP Pharmaceuticals, Inc.), or Navire, we entered into a collaboration and license agreement with The Board of Regents of the University of Texas System, or the Board of Regents, and The University of Texas M.D. Anderson Cancer Center, or MD Anderson. Under the agreement, we acquired an exclusive, royalty-bearing, sublicensable, worldwide license to



develop, make, use and sell SHP2 and PTPN11 inhibitors covered by the licensed technology in all fields. The Board of Regents and MD Anderson each retain the right to practice the licensed patent rights for non-commercial, research and academic purposes, and also to grant non-exclusive licenses to other academic and nonprofit organizations to practice the patent rights for non-commercial, research and educational purposes (but excluding any research sponsored by a for-profit entity). Our license is also subject to a non-exclusive license granted to the U.S. government. To further the goals of the collaboration agreement, we granted a non-exclusive license to our technology to MD Anderson for the purpose of carrying out the development plan.

In partial consideration for the exclusive license grant, we issued the Board of Regents shares of common stock of Navire valued at approximately \$280,000 pursuant to a stock purchase agreement entered into simultaneously. If commercial sales of a licensed product commence, we will pay MD Anderson royalties at percentage rates ranging in the low single digits on net sales of licensed products. We may offset payments made to third parties to obtain rights needed for the commercialization of a licensed product against royalties payable to MD Anderson provided that such expenses in a given year may not be credited against more than a specified percentage of the royalties in such year and subject to a minimum floor in the low single digits. Our obligation to pay various royalties continues on a country by country basis with respect to any licensed product depends on regulatory status, patent coverage, and financing status. For licensed products that satisfy certain regulatory conditions, the related royalty extends for three years after the first sale. Additionally, if certain financing conditions are achieved, then (i) for licensed products covered by licensed patents, the royalty obligation continues until the expiration of all licensed patent rights covering such licensed product in such country, and (ii) for licensed products without coverage by licensed patents, the royalty obligation extends for 10 years after first sale.

Under the collaboration and license agreement, we are obligated to use commercially reasonable efforts to conduct all development activities under the agreement and to commercialize the licensed products following regulatory approval.

The agreement will continue for thirty years unless earlier terminated. We may terminate the agreement for convenience, provided that MD Anderson shall not be required to forego payments made or equity issued to MD Anderson under the collaboration and license agreement or the stock purchase agreement. MD Anderson has the right to terminate the agreement if we fail to pay royalties or otherwise materially breach the agreement or the stock purchase agreement and fail to cure such breach within a specified cure period, or if BridgeBio Pharma LLC commits a material breach of its obligations under any agreement with Navire, or if Navire breaches obligations under a Series A Preferred Stock Purchase Agreement between Navire and BridgeBio Pharma LLC.

## **Government Regulation**

Government authorities in the United States at the federal, state and local level and in other countries regulate, among other things, the research, development, manufacture, testing, quality control, approval, labeling, packaging, storage, record-keeping, promotion, advertising, distribution, post-approval monitoring and reporting, marketing and export and import of drug and biological products, including gene therapies, as well as diagnostics, and any future product candidates. Generally, before a new drug, biologic or diagnostic can be marketed, considerable data demonstrating its quality, safety and efficacy must be obtained, organized into a format specific for each regulatory authority, submitted for review and approved, authorized, or cleared by the applicable regulatory authority.

### ***U.S. Government Regulation of Drug and Biological Products***

In the United States, the FDA regulates drugs under the FDCA, and its implementing regulations and biologics under the FDCA and the Public Health Service Act, or PHSA, and their implementing regulations. Both drugs and biologics also are subject to other federal, state and local statutes and regulations, such as those related to competition. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, and local statutes and regulations requires the expenditure of substantial time and financial

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resources. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or following approval may subject an applicant to administrative actions or judicial sanctions. These actions and sanctions could include, among other actions, the FDA's refusal to approve pending applications, withdrawal of an approval, license revocation, a clinical hold, untitled or warning letters, voluntary or mandatory product recalls or market withdrawals, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement and civil or criminal fines or penalties. Any agency or judicial enforcement action could have a material adverse effect on our business, the market acceptance of our products and our reputation.

Our product candidates must be approved by the FDA through either an NDA, or a BLA, process before they may be legally marketed in the United States. The process generally involves the following:

- completion of extensive preclinical studies in accordance with applicable regulations, including studies conducted in accordance with GLP, requirements;
- submission to the FDA of an IND application, which must become effective before human clinical trials may begin;
- approval by an IRB, or independent ethics committee at each clinical trial site before each human trial may be initiated;
- performance of adequate and well-controlled human clinical trials in accordance with applicable IND regulations, GCP, requirements and other clinical trial-related regulations to establish the safety and efficacy of the investigational product for each proposed indication;
- submission to the FDA of an NDA or BLA;
- a determination by the FDA within 60 days of its receipt of an NDA or BLA to accept the filing for review;
- satisfactory completion of one or more FDA pre-approval inspections of the manufacturing facility or facilities where the drug or biologic will be produced to assess compliance with Current Good Manufacturing Practices, or cGMP, requirements to assure that the facilities, methods and controls are adequate to preserve the drug or biologic's identity, strength, quality and purity;
- potential FDA audit of the clinical trial sites that generated the data in support of the NDA or BLA;
- payment of user fees for FDA review of the NDA or BLA; and
- FDA review and approval of the NDA or BLA, including consideration of the views of any FDA advisory committee, prior to any commercial marketing or sale of the drug or biologic in the United States.

The preclinical and clinical testing and approval process requires substantial time, effort and financial resources, and the regulatory scheme for drugs and biologics is evolving and subject to change at any time. We cannot be certain that any approvals for our product candidates will be granted on a timely basis, or at all.

### ***Preclinical Studies***

Before testing any drug, biological or gene therapy candidate in humans, the product candidate must undergo rigorous preclinical testing. Preclinical studies include laboratory evaluation of product chemistry and formulation, as well as *in vitro* and animal studies to assess safety and in some cases to establish a rationale for therapeutic use. The conduct of preclinical studies is subject to federal and state regulations and requirements, including GLP regulations for safety/toxicology studies.

An IND sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and plans for clinical trials, among other things, to the FDA as part of an IND. An IND is a request for authorization from the FDA to administer an investigational product to humans, and must become effective before human clinical trials may begin. Some long-term

preclinical testing, such as animal tests of reproductive AEs and carcinogenicity, may continue after the IND is submitted. An IND automatically becomes effective 30 days after receipt by the FDA, unless before that time, the FDA raises concerns or questions related to one or more proposed clinical trials and places the trial on clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. As a result, submission of an IND may not result in the FDA allowing clinical trials to commence. Additionally, the review of information in an IND submission may prompt FDA to, among other things, scrutinize existing INDs or marketed products and could generate requests for information or clinical holds on other product candidates or programs.

### ***Clinical Trials***

The clinical stage of development involves the administration of the investigational product to healthy volunteers or patients under the supervision of qualified investigators, generally physicians not employed by or under the trial sponsor's control, in accordance with GCP requirements, which include the requirement that all research subjects provide their informed consent for their participation in any clinical trial. Clinical trials are conducted under protocols detailing, among other things, the objectives of the clinical trial, dosing procedures, subject selection and exclusion criteria and the parameters to be used to monitor subject safety and assess efficacy. Each protocol, and any subsequent amendments to the protocol, must be submitted to the FDA as part of the IND. Furthermore, each clinical trial must be reviewed and approved by an IRB for each institution at which the clinical trial will be conducted to ensure that the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the informed consent form that must be provided to each clinical trial subject or his or her legal representative, and must monitor the clinical trial until completed. There also are requirements governing the reporting of ongoing clinical trials and completed clinical trial results to public registries. Information about certain clinical trials, including clinical trial results, must be submitted within specific timeframes for publication on the [www.clinicaltrials.gov](http://www.clinicaltrials.gov) website.

In addition to the submission of an IND to the FDA before initiation of a clinical trial in the United States, certain human clinical trials involving recombinant or synthetic nucleic acid molecules had historically been subject to review by the RAC, of the NIH Office of Biotechnology Activities, or the OBA, pursuant to the NIH Guideline. On August 17, 2018, the NIH issued a notice in the Federal Register and issued a public statement proposing changes to the oversight framework for gene therapy trials, including changes to the applicable NIH Guidelines to modify the roles and responsibilities of the RAC with respect to human clinical trials of gene therapy products, and requesting public comment on its proposed modifications. During the public comment period, which closed October 16, 2018, the NIH announced that it will no longer accept new human gene transfer protocols for review as a part of the protocol registration process or convene the RAC to review individual clinical protocols. In April 2019, NIH announced the updated guidelines, which reflect these proposed changes, and clarify that these trials will remain subject to the FDA's oversight and other clinical trial regulations, and oversight at the local level will continue as set forth in the NIH Guidelines. Specifically, under the NIH Guidelines, supervision of human gene transfer trials includes evaluation and assessment by an IBC, a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment, and such review may result in some delay before initiation of a clinical trial. While the NIH Guidelines are not mandatory unless the research in question being conducted at or sponsored by institutions receiving NIH funding of recombinant or synthetic nucleic acid molecule research, many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them.

A sponsor who wishes to conduct a clinical trial outside of the United States may, but need not, obtain FDA authorization to conduct the clinical trial under an IND. If a foreign clinical trial is not conducted under an IND, the sponsor may submit data from the clinical trial to the FDA in support of an NDA or BLA. The FDA will accept a well-designed and well-conducted foreign clinical study not conducted under an IND if the study was conducted in accordance with GCP requirements, and the FDA is able to validate the data through an onsite inspection if deemed necessary.

Clinical trials generally are conducted in three sequential phases, known as Phase 1, Phase 2 and Phase 3, and may overlap.

- *Phase 1* clinical trials generally involve a small number of healthy volunteers or disease-affected patients who are initially exposed to a single dose and then multiple doses of the product candidate. The primary purpose of these clinical trials is to assess the metabolism, pharmacologic action, side effect tolerability and safety of the product candidate.
- *Phase 2* clinical trials involve studies in disease-affected patients to evaluate proof of concept and/or determine the dose required to produce the desired benefits. At the same time, safety and further PK and PD information is collected, possible adverse effects and safety risks are identified and a preliminary evaluation of efficacy is conducted.
- *Phase 3* clinical trials generally involve a large number of patients at multiple sites and are designed to provide the data necessary to demonstrate the effectiveness of the product for its intended use, its safety in use and to establish the overall benefit/risk relationship of the product and provide an adequate basis for product labeling.

In August 2018, the FDA released a draft guidance entitled “Expansion Cohorts: Use in First-In-Human Clinical Trials to Expedite Development of Oncology Drugs and Biologics,” which outlines how drug developers can utilize an adaptive trial design commonly referred to as a seamless trial design in early stages of oncology drug development, i.e., the first-in-human clinical trial, to compress the traditional three phases of trials into one continuous trial called an expansion cohort trial. Information to support the design of individual expansion cohorts are included in IND applications and assessed by FDA. Expansion cohort trials can potentially bring efficiency to drug development and reduce developmental costs and time.

Post-approval trials, sometimes referred to as Phase 4 clinical trials, may be conducted after initial marketing approval. These trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication and are commonly intended to generate additional safety data regarding use of the product in a clinical setting. In certain instances, the FDA may mandate the performance of Phase 4 clinical trials as a condition of approval of an NDA or BLA.

Progress reports detailing the results of the clinical trials, among other information, must be submitted at least annually to the FDA and written IND safety reports must be submitted to the FDA and the investigators 15 days after the trial sponsor determines the information qualifies for reporting for serious and unexpected suspected AEs, findings from other studies or animal or *in vitro* testing that suggest a significant risk for human subjects and any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. The sponsor must also notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction as soon as possible but in no case later than seven calendar days after the sponsor’s initial receipt of the information.

Phase 1, Phase 2, Phase 3 and other types of clinical trials may not be completed successfully within any specified period, if at all. The FDA or the sponsor may suspend or terminate a clinical trial at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB’s requirements or if the drug or biologic has been associated with unexpected serious harm to patients. Additionally, some clinical trials are overseen by an independent group of qualified experts organized by the clinical trial sponsor, known as a data safety monitoring board or committee. This group provides authorization for whether a trial may move forward at designated check points based on access to certain data from the trial. Concurrent with clinical trials, companies usually complete additional animal studies and also must develop additional information about the chemistry and physical characteristics of the drug or biologic as well as finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently

producing quality batches of the product and, among other things, companies must develop methods for testing the identity, strength, quality and purity of the final product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the product candidates do not undergo unacceptable deterioration over their shelf life.

### ***FDA Review Process***

Following completion of the clinical trials, data are analyzed to assess whether the investigational product is safe and effective for the proposed indicated use or uses. The results of preclinical studies and clinical trials are then submitted to the FDA as part of an NDA or BLA, along with proposed labeling, chemistry and manufacturing information to ensure product quality and other relevant data. The NDA or BLA is a request for approval to market the drug or biologic for one or more specified indications and must contain proof of safety and efficacy for a drug or safety, purity and potency for a biologic. The application may include both negative and ambiguous results of preclinical studies and clinical trials, as well as positive findings. Data may come from company-sponsored clinical trials intended to test the safety and efficacy of a product's use or from a number of alternative sources, including studies initiated by investigators. To support marketing approval, the data submitted must be sufficient in quality and quantity to establish the safety and efficacy of the investigational product to the satisfaction of FDA. FDA approval of an NDA or BLA must be obtained before a drug or biologic may be marketed in the United States.

Under the Prescription Drug User Fee Act, or PDUFA, as amended, each NDA or BLA must be accompanied by a user fee. FDA adjusts the PDUFA user fees on an annual basis. Fee waivers or reductions are available in certain circumstances, including a waiver of the application fee for the first application filed by a small business. Additionally, no user fees are assessed on NDAs or BLAs for products designated as orphan drugs, unless the product also includes a non-orphan indication.

The FDA reviews all submitted NDAs and BLAs before it accepts them for filing, and may request additional information rather than accepting the NDA or BLA for filing. The FDA must make a decision on accepting an NDA or BLA for filing within 60 days of receipt, and such decision could include a refusal to file by the FDA. Once the submission is accepted for filing, the FDA begins an in-depth review of the NDA or BLA. Under the goals and policies agreed to by the FDA under PDUFA, the FDA targets ten months, from the filing date, in which to complete its initial review of a new molecular entity NDA or original BLA and respond to the applicant, and six months from the filing date of a new molecular entity NDA or original BLA designated for priority review. The FDA does not always meet its PDUFA goal dates for standard and priority NDAs or BLAs, and the review process is often extended by FDA requests for additional information or clarification.

Before approving an NDA or BLA, the FDA will conduct a pre-approval inspection of the manufacturing facilities for the new product to determine whether they comply with cGMP requirements. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. The FDA also may audit data from clinical trials to ensure compliance with GCP requirements. Additionally, the FDA may refer applications for novel products or products which present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions, if any. The FDA is not bound by recommendations of an advisory committee, but it considers such recommendations when making decisions on approval. The FDA likely will reanalyze the clinical trial data, which could result in extensive discussions between the FDA and the applicant during the review process. After the FDA evaluates an NDA or BLA, it will issue an approval letter or a Complete Response Letter. An approval letter authorizes commercial marketing of the drug or biologic with specific prescribing information for specific indications. A Complete Response Letter indicates that the review cycle of the application is complete and the application will not be approved in its present form. A Complete Response Letter usually describes all of the specific deficiencies in the NDA or BLA identified by the FDA. The Complete Response Letter may require the applicant to obtain

additional clinical data, including the potential requirement to conduct additional pivotal Phase 3 clinical trial(s) and/or to complete other significant and time-consuming requirements related to clinical trials, or to conduct additional preclinical studies or manufacturing activities. If a Complete Response Letter is issued, the applicant may either resubmit the NDA or BLA, addressing all of the deficiencies identified in the letter, or withdraw the application or request an opportunity for a hearing. Even if such data and information are submitted, the FDA may decide that the NDA or BLA does not satisfy the criteria for approval. Data obtained from clinical trials are not always conclusive and the FDA may interpret data differently than we interpret the same data.

#### ***Orphan Drug Designation and Exclusivity***

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biological product intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making the product available in the United States for this type of disease or condition will be recovered from sales of the product.

Orphan drug designation must be requested before submitting an NDA or BLA. After the FDA grants orphan drug designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Orphan drug designation does not convey any advantage in or shorten the duration of the regulatory review and approval process.

If a product that has orphan drug designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan drug exclusivity, which means that the FDA may not approve any other applications to market the same drug for the same indication for seven years from the date of such approval, except in limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity by means of greater effectiveness, greater safety or providing a major contribution to patient care or in instances of drug supply issues. Competitors, however, may receive approval of either a different product for the same indication or the same product for a different indication but that could be used off-label in the orphan indication. Orphan drug exclusivity also could block the approval of one of our products for seven years if a competitor obtains approval before we do for the same product, as defined by the FDA, for the same indication we are seeking approval, or if our product is determined to be contained within the scope of the competitor's product for the same indication or disease. If we pursue marketing approval for an indication broader than the orphan drug designation we have received, we may not be entitled to orphan drug exclusivity. Orphan drug status in the European Union has similar, but not identical, requirements and benefits.

#### ***Rare Pediatric Disease Designation and Priority Review Vouchers***

Under the FDCA, as amended, the FDA incentivizes the development of drugs and biologics that meet the definition of a "rare pediatric disease," defined to mean a serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years and the disease affects fewer than 200,000 individuals in the United States or affects more than 200,000 in the United States and for which there is no reasonable expectation that the cost of developing and making in the United States a drug for such disease or condition will be recovered from sales in the United States of such drug. The sponsor of a product candidate for a rare pediatric disease may be eligible for a voucher that can be used to obtain a priority review for a subsequent human drug or biologic application after the date of approval of the rare pediatric disease drug product, referred to as a priority review voucher, or PRV. A sponsor may request rare pediatric disease designation from the FDA prior to the submission of its NDA or BLA. A rare pediatric disease designation does not guarantee that a sponsor will receive a PRV upon approval of its NDA or BLA. Moreover, a sponsor who chooses not to submit a rare pediatric disease designation request may nonetheless receive a PRV upon approval of their marketing application if they request such a voucher in their original marketing application and meet all of the eligibility criteria. If a PRV is received, it may be sold or transferred an unlimited number of times. Congress has extended the PRV program until September 30, 2020, with the potential for PRVs to be granted until 2022.

### ***Expedited Development and Review Programs***

A sponsor may seek to develop and obtain approval of its product candidates under programs designed to accelerate the development, FDA review and approval of new drugs and biologics that meet certain criteria. For example, the FDA has a fast track program that is intended to expedite or facilitate the process for reviewing new drugs and biologics that are intended to treat a serious or life threatening disease or condition and demonstrate the potential to address unmet medical needs for the condition. Fast track designation applies to both the product and the specific indication for which it is being studied. For a fast track-designated product, the FDA may consider sections of the NDA or BLA for review on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the application, the FDA agrees to accept sections of the application and determines that the schedule is acceptable and the sponsor pays any required user fees upon submission of the first section of the application. The sponsor can request the FDA to designate the product for fast track status any time before receiving NDA or BLA approval, but ideally no later than the pre-NDA or pre-BLA meeting.

A product submitted to the FDA for marketing, including under a fast track program, may be eligible for other types of FDA programs intended to expedite development or review, such as priority review and accelerated approval. Priority review means that, for a new molecular entity or original BLA, the FDA sets a target date for FDA action on the marketing application at six months after accepting the application for filing as opposed to ten months. A product is eligible for priority review if it is designed to treat a serious or life-threatening disease condition and, if approved, would provide a significant improvement in safety and effectiveness compared to available therapies. The FDA will attempt to direct additional resources to the evaluation of an application for a new drug or biologic designated for priority review in an effort to facilitate the review. If criteria are not met for priority review, the application for a new molecular entity or original BLA is subject to the standard FDA review period of ten months after FDA accepts the application for filing. Priority review designation does not change the scientific/medical standard for approval or the quality of evidence necessary to support approval.

A product may also be eligible for accelerated approval if it is designed to treat a serious or life-threatening disease or condition and demonstrates an effect on either a surrogate endpoint that is reasonably likely to predict clinical benefit or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, or IMM, that is reasonably likely to predict an effect on IMM or other clinical benefit, taking into account the severity, rarity, or prevalence of the disease or condition and the availability or lack of alternative treatments. As a condition of approval, the FDA may require that a sponsor of a drug or biologic receiving accelerated approval perform adequate and well-controlled post-marketing clinical trials. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product. FDA may withdraw approval of a drug or indication approved under accelerated approval if, for example, the confirmatory trial fails to verify the predicted clinical benefit of the product.

Additionally, a drug or biologic may be eligible for designation as a breakthrough therapy if the product is intended, alone or in combination with one or more other drugs or biologics, to treat a serious or life-threatening condition and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over currently approved therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. If the FDA designates a breakthrough therapy, it may take actions appropriate to expedite the development and review of the application, which may include holding meetings with the sponsor and the review team throughout the development of the therapy; providing timely advice to, and interactive communication with, the sponsor regarding the development of the drug to ensure that the development program to gather the nonclinical and clinical data necessary for approval is as efficient as practicable; involving senior managers and experienced review staff, as appropriate, in a collaborative, cross-disciplinary review; assigning a cross-disciplinary project lead for the FDA review team to facilitate an efficient review of the development program and to serve as a scientific liaison between the review team and the sponsor; and considering alternative clinical trial designs when scientifically appropriate, which may result in

smaller trials or more efficient trials that require less time to complete and may minimize the number of patients exposed to a potentially less efficacious treatment. Breakthrough therapy designation comes with all of the benefits of fast track designation, which means that the sponsor may file sections of the BLA for review on a rolling basis if certain conditions are satisfied, including an agreement with the FDA on the proposed schedule for submission of portions of the application and the payment of applicable user fees before the FDA may initiate a review.

Even if a product qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or the time period for FDA review or approval may not be shortened. Furthermore, fast track designation, priority review, accelerated approval and breakthrough therapy designation do not change the standards for approval.

### ***Pediatric Information and Pediatric Exclusivity***

Under the Pediatric Research Equity Act, or PREA, certain NDAs and BLAs and certain supplements to an NDA or BLA must contain data to assess the safety and efficacy of the drug for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may grant deferrals for submission of pediatric data or full or partial waivers. The Food and Drug Administration Safety and Innovation Act, or FDASIA, amended the FDCA to require that a sponsor who is planning to submit a marketing application for a drug that includes a new active ingredient, new indication, new dosage form, new dosing regimen or new route of administration submit an initial Pediatric Study Plan, or PSP, within 60 days of an end-of-Phase 2 meeting or, if there is no such meeting, as early as practicable before the initiation of the Phase 3 or Phase 2/3 study. The initial PSP must include an outline of the pediatric study or studies that the sponsor plans to conduct, including study objectives and design, age groups, relevant endpoints and statistical approach, or a justification for not including such detailed information, and any request for a deferral of pediatric assessments or a full or partial waiver of the requirement to provide data from pediatric studies along with supporting information. The FDA and the sponsor must reach an agreement on the PSP. A sponsor can submit amendments to an agreed-upon initial PSP at any time if changes to the pediatric plan need to be considered based on data collected from preclinical studies, early phase clinical trials and/or other clinical development programs.

A drug or biologic product can also obtain pediatric market exclusivity in the United States. Pediatric exclusivity, if granted, adds six months to existing exclusivity periods and patent terms. This six-month exclusivity, which runs from the end of other exclusivity protection or patent term, may be granted based on the voluntary completion of a pediatric study in accordance with an FDA-issued "Written Request" for such a study.

### ***Post-Marketing Requirements***

Following approval of a new product, the manufacturer and the approved product are subject to continuing regulation by the FDA, including, among other things, monitoring and record-keeping activities, reporting of adverse experiences, complying with promotion and advertising requirements, which include restrictions on promoting products for unapproved uses or patient populations (known as "off-label use") and limitations on industry-sponsored scientific and educational activities. Although physicians may prescribe legally available products for off-label uses, manufacturers may not market or promote such uses. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability, including investigation by federal and state authorities. Prescription drug promotional materials must be submitted to the FDA in conjunction with their first use or first publication. Further, if there are any modifications to the drug or biologic, including changes in indications, labeling or manufacturing processes or facilities, the applicant may be required to submit and obtain FDA approval of a new NDA/BLA or NDA/BLA supplement, which may require the development of additional data or preclinical studies and clinical trials.



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The FDA may also place other conditions on approvals including the requirement for a REMS, to assure the safe use of the product. If the FDA concludes a REMS is needed, the sponsor of the NDA or BLA must submit a proposed REMS. The FDA will not approve the NDA or BLA without an approved REMS, if required. A REMS could include medication guides, physician communication plans or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. Any of these limitations on approval or marketing could restrict the commercial promotion, distribution, prescription or dispensing of products. Product approvals may be withdrawn for non-compliance with regulatory standards or if problems occur following initial marketing.

FDA regulations require that products be manufactured in specific approved facilities and in accordance with cGMP regulations. We rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of our products in accordance with cGMP regulations. These manufacturers must comply with cGMP regulations that require, among other things, quality control and quality assurance, the maintenance of records and documentation and the obligation to investigate and correct any deviations from cGMP. Manufacturers and other entities involved in the manufacture and distribution of approved drugs or biologics are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP requirements and other laws. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain cGMP compliance. The discovery of violative conditions, including failure to conform to cGMP regulations, could result in enforcement actions, and the discovery of problems with a product after approval may result in restrictions on a product, manufacturer or holder of an approved NDA or BLA, including recall.

Once an approval is granted, the FDA may issue enforcement letters or withdraw the approval of the product if compliance with regulatory requirements and standards is not maintained or if problems occur after the drug or biologic reaches the market. Corrective action could delay drug or biologic distribution and require significant time and financial expenditures. Later discovery of previously unknown problems with a drug or biologic, including AEs of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical trials to assess new safety risks; or imposition of distribution or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the drug or biologic, suspension of the approval, complete withdrawal of the drug from the market or product recalls;
- fines, warning letters or holds on post-approval clinical trials;
- refusal of the FDA to approve applications or supplements to approved applications, or suspension or revocation of drug or biologic approvals;
- drug or biologic seizure or detention, or refusal to permit the import or export of drugs; or
- injunctions or the imposition of civil or criminal penalties.

### ***Regulation of Companion Diagnostics***

We believe that the success of certain of our product candidates may depend, in part, on the development and commercialization of a companion diagnostic. Companion diagnostics identify patients who are most likely to benefit from a particular therapeutic product; identify patients likely to be at increased risk for serious side effects as a result of treatment with a particular therapeutic product; or monitor response to treatment with a particular therapeutic product for the purpose of adjusting treatment to achieve improved safety or effectiveness. Companion diagnostics are regulated as medical devices by the FDA. In the United States, the FDCA and its implementing regulations, and other federal and state statutes and regulations govern, among other things, medical device design and development, preclinical and clinical testing, premarket clearance or approval,

registration and listing, manufacturing, labeling, storage, advertising and promotion, sales and distribution, export and import, and post-market surveillance. Unless an exemption or FDA exercise of enforcement discretion applies, diagnostic tests generally require marketing clearance or approval from the FDA prior to commercialization. The two primary types of FDA marketing authorization applicable to a medical device are premarket notification, also called 510(k) clearance, and approval of a premarket, or PMA approval.

To obtain 510(k) clearance for a medical device, or for certain modifications to devices that have received 510(k) clearance, a manufacturer must submit a premarket notification demonstrating that the proposed device is substantially equivalent to a previously cleared 510(k) device or to a preamendment device that was in commercial distribution before May 28, 1976, or a predicate device, for which the FDA has not yet called for the submission of a PMA. In making a determination that the device is substantially equivalent to a predicate device, the FDA compares the proposed device to the predicate device or predicate devices and assesses whether the subject device is comparable to the predicate device or predicate devices with respect to intended use, technology, design and other features which could affect safety and effectiveness. If the FDA determines that the subject device is substantially equivalent to the predicate device or predicate devices, the subject device may be cleared for marketing. The 510(k) premarket notification pathway generally takes from three to twelve months from the date the application is completed, but can take significantly longer.

PMA applications must be supported by valid scientific evidence, which typically requires extensive data, including technical, preclinical, clinical and manufacturing data, to demonstrate to the FDA's satisfaction the safety and effectiveness of the device. For diagnostic tests, a PMA application typically includes data regarding analytical and clinical validation studies. As part of its review of the PMA, the FDA will conduct a pre-approval inspection of the manufacturing facility or facilities to ensure compliance with the Quality System Regulation, or QSR, which requires manufacturers to follow design, testing, control, documentation and other quality assurance procedures. The FDA's review of an initial PMA application is required by statute to take between six to ten months, although the process typically takes longer, and may require several years to complete. If the FDA evaluations of both the PMA application and the manufacturing facilities are favorable, the FDA will either issue an approval letter or an approvable letter, which usually contains a number of conditions that must be met in order to secure the final approval of the PMA. If the FDA's evaluation of the PMA or manufacturing facilities is not favorable, the FDA will deny the approval of the PMA or issue a not approvable letter. A not approvable letter will outline the deficiencies in the application and, where practical, will identify what is necessary to make the PMA approvable. Once granted, PMA approval may be withdrawn by the FDA if compliance with post-approval requirements, conditions of approval or other regulatory standards is not maintained or problems are identified following initial marketing.

On July 31, 2014, the FDA issued a final guidance document addressing the development and approval process for "In Vitro Companion Diagnostic Devices." According to the guidance document, for novel therapeutic products that depend on the use of a diagnostic test and where the diagnostic device could be essential for the safe and effective use of the corresponding therapeutic product, the premarket application for the companion diagnostic device should be developed and approved or cleared contemporaneously with the therapeutic, although the FDA recognizes that there may be cases when contemporaneous development may not be possible. However, in cases where a drug cannot be used safely or effectively without the companion diagnostic, the FDA's guidance indicates it will generally not approve the drug without the approval or clearance of the diagnostic device. The FDA also issued a draft guidance in July 2016 setting forth the principles for co-development of an in vitro companion diagnostic device with a therapeutic product. The draft guidance describes principles to guide the development and contemporaneous marketing authorization for the therapeutic product and its corresponding in vitro companion diagnostic.

Once cleared or approved, the companion diagnostic device must adhere to post-marketing requirements including the requirements of FDA's quality system regulation, adverse event reporting, recalls and corrections along with product marketing requirements and limitations. Like drug and biologic makers, companion diagnostic makers are subject to unannounced FDA inspections at any time during which the FDA will conduct an audit of the product(s) and the company's facilities for compliance with its authorities.

### ***Biosimilars and Exclusivity***

Certain of our product candidates are regulated as biologics. An abbreviated approval pathway for biological products shown to be similar to, or interchangeable with, an FDA-licensed reference biological product was created by the Biologics Price Competition and Innovation Act of 2009, or BPCI Act, as part of the ACA. This amendment to the PHS Act, in part, attempts to minimize duplicative testing. Biosimilarity, which requires that the biological product be highly similar to the reference product notwithstanding minor differences in clinically inactive components and that there be no clinically meaningful differences between the product and the reference product in terms of safety, purity and potency, can be shown through analytical studies, animal studies and a clinical trial or trials. Interchangeability requires that a biological product be biosimilar to the reference product and that the product can be expected to produce the same clinical results as the reference product in any given patient and, for products administered multiple times to an individual, that the product and the reference product may be alternated or switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biological product without such alternation or switch. Complexities associated with the larger, and often more complex, structure of biological products as compared to small molecule drugs, as well as the processes by which such products are manufactured, pose significant hurdles to implementation that are still being worked out by the FDA.

A reference biological product is granted four and twelve year exclusivity periods from the time of first licensure of the product. FDA will not accept an application for a biosimilar or interchangeable product based on the reference biological product until four years after the date of first licensure of the reference product, and FDA will not approve an application for a biosimilar or interchangeable product based on the reference biological product until twelve years after the date of first licensure of the reference product. “First licensure” typically means the initial date the particular product at issue was licensed in the United States. Date of first licensure does not include the date of licensure of (and a new period of exclusivity is not available for) a biological product if the licensure is for a supplement for the biological product or for a subsequent application by the same sponsor or manufacturer of the biological product (or licensor, predecessor in interest, or other related entity) for a change (not including a modification to the structure of the biological product) that results in a new indication, route of administration, dosing schedule, dosage form, delivery system, delivery device or strength, or for a modification to the structure of the biological product that does not result in a change in safety, purity, or potency. Therefore, one must determine whether a new product includes a modification to the structure of a previously licensed product that results in a change in safety, purity, or potency to assess whether the licensure of the new product is a first licensure that triggers its own period of exclusivity. Whether a subsequent application, if approved, warrants exclusivity as the “first licensure” of a biological product is determined on a case-by-case basis with data submitted by the sponsor.

### ***Other Regulatory Matters***

Manufacturing, sales, promotion and other activities following product approval are also subject to regulation by numerous regulatory authorities in the United States in addition to the FDA, including the CMS, including the Office of Inspector General and Office for Civil Rights, other divisions of the Department of HHS, the Department of Justice, the Drug Enforcement Administration, the Consumer Product Safety Commission, the Federal Trade Commission, the Occupational Safety & Health Administration, the Environmental Protection Agency and state and local governments.

Healthcare providers, physicians, and third party payors will play a primary role in the recommendation and prescription of any products for which we obtain marketing approval. Our current and future arrangements with healthcare providers and physicians and any future arrangements with third party payors, may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute any drugs for which we obtain marketing approval. In the United States, these laws include: the federal Anti-Kickback Statute, the False Claims Act, and HIPAA.

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The Anti-Kickback Statute makes it illegal for any person, including a prescription drug manufacturer (or a party acting on its behalf), to knowingly and willfully solicit, receive, offer or pay any remuneration, directly or indirectly, in cash or in kind, that is intended to induce or reward referrals, including the purchase, recommendation, order or prescription of a particular drug, for which payment may be made under a federal healthcare program, such as Medicare or Medicaid. Violations of this law are punishable by imprisonment, criminal fines, administrative civil money penalties and exclusion from participation in federal healthcare programs. In addition, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it. Moreover, the ACA provides that the government may assert that a claim including items or services resulting from a violation of the federal Anti Kickback Statute constitutes a false or fraudulent claim for purposes of the federal civil False Claims Act.

Although we would not submit claims directly to payors, drug manufacturers can be held liable under the federal civil False Claims Act, which imposes civil penalties, including through civil whistleblower or qui tam actions, against individuals or entities (including manufacturers) for, among other things, knowingly presenting, or causing to be presented to federal programs (including Medicare and Medicaid) claims for items or services, including drugs, that are false or fraudulent, claims for items or services not provided as claimed, or claims for medically unnecessary items or services. Penalties for a False Claims Act violation include three times the actual damages sustained by the government, plus mandatory civil penalties for each separate false claim, the potential for exclusion from participation in federal healthcare programs and, although the federal False Claims Act is a civil statute, conduct that results in a False Claims Act violation may also implicate various federal criminal statutes. The government may deem manufacturers to have “caused” the submission of false or fraudulent claims by, for example, providing inaccurate billing or coding information to customers or promoting a product off-label. Claims which include items or services resulting from a violation of the federal Anti-Kickback Statute are false or fraudulent claims for purposes of the False Claims Act. Our future marketing and activities relating to the reporting of wholesaler or estimated retail prices for our products, if approved, the reporting of prices used to calculate Medicaid rebate information and other information affecting federal, state and third-party reimbursement for our products, and the sale and marketing of our product candidates, are subject to scrutiny under this law.

HIPAA created new federal criminal statutes that prohibit among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud or to obtain, by means of false or fraudulent pretenses, representations or promises, any money or property owned by, or under the control or custody of, any healthcare benefit program, including private third party payors, knowingly and willfully embezzling or stealing from a healthcare benefit program, willfully obstructing a criminal investigation of a healthcare offense, and knowingly and willfully falsifying, concealing or covering up by trick, scheme or device, a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services. Like the federal Anti-Kickback Statute a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation.

The civil monetary penalties statute imposes penalties against any person or entity that, among other things, is determined to have presented or caused to be presented a claim to a federal health program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent.

We may be subject to data privacy and security regulations by both the federal government and the states in which we conduct our business. HIPAA, as amended by HITECH, and their implementing regulations, mandates, among other things, the adoption of uniform standards for the electronic exchange of information in common healthcare transactions, as well as standards relating to the privacy and security of individually identifiable health information, which require the adoption of administrative, physical and technical safeguards to protect such information. Among other things, HITECH makes HIPAA’s security standards directly applicable to business associates, defined as independent contractors or agents of covered entities, which include certain health care providers, health plans, and healthcare clearinghouses, that create, receive or obtain protected health information in connection with providing a service for or on behalf of a covered entity. HITECH also increased the civil and

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criminal penalties that may be imposed against covered entities and business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorney's fees and costs associated with pursuing federal civil actions. In addition, certain state laws govern the privacy and security of health information in certain circumstances, some of which are more stringent than HIPAA and many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts. Failure to comply with these laws, where applicable, can result in the imposition of significant civil and criminal penalties.

Additionally, the federal Physician Payments Sunshine Act, or the Sunshine Act, within the ACA, and its implementing regulations, require that certain manufacturers of drugs, devices, biological and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) report annually to CMS information related to certain payments or other transfers of value made or distributed to physicians and teaching hospitals, or to entities or individuals at the request of, or designated on behalf of, physicians, certain other healthcare professionals, and teaching hospitals and to report annually certain ownership and investment interests held by physicians, certain other healthcare professionals, and their immediate family members. In addition, many states also govern the reporting of payments or other transfers of value, many of which differ from each other in significant ways, are often not pre-empted, and may have a more prohibitive effect than the Sunshine Act, thus further complicating compliance efforts.

Similar federal, state and foreign fraud and abuse laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services. Such laws are generally broad and are enforced by various state agencies and private actions. Also, many states have similar fraud and abuse statutes or regulations that may be broader in scope and may apply regardless of payor, in addition to items and services reimbursed under Medicaid and other state programs. Some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant federal government compliance guidance, and require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures.

In order to distribute products commercially, we must comply with state laws that require the registration of manufacturers and wholesale distributors of drug and biological products in a state, including, in certain states, manufacturers and distributors who ship products into the state even if such manufacturers or distributors have no place of business within the state. Some states also impose requirements on manufacturers and distributors to establish the pedigree of product in the chain of distribution, including some states that require manufacturers and others to adopt new technology capable of tracking and tracing product as it moves through the distribution chain. Several states have enacted legislation requiring pharmaceutical and biotechnology companies to establish marketing compliance programs, file periodic reports with the state, make periodic public disclosures on sales, marketing, pricing, clinical trials and other activities, and/or register their sales representatives, as well as to prohibit pharmacies and other healthcare entities from providing certain physician prescribing data to pharmaceutical and biotechnology companies for use in sales and marketing, and to prohibit certain other sales and marketing practices. All of our activities are potentially subject to federal and state consumer protection and unfair competition laws.

The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform, especially in light of the lack of applicable precedent and regulations. Federal and state enforcement bodies have recently increased their scrutiny of interactions between healthcare companies and healthcare providers, which has led to a number of investigations, prosecutions, convictions and settlements in the healthcare industry. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, disgorgement, contractual damages, reputational harm, diminished

profits and future earnings, individual imprisonment, exclusion of drugs from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our financial results. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs. Ensuring business arrangements comply with applicable healthcare laws, as well as responding to possible investigations by government authorities, can be time- and resource-consuming and can divert a company's attention from the business.

### ***Current and Future Legislation***

In the United States and some foreign jurisdictions, there have been, and likely will continue to be, a number of legislative and regulatory changes and proposed changes regarding the healthcare system directed at broadening the availability of healthcare, improving the quality of healthcare, and containing or lowering the cost of healthcare.

For example, in March 2010, the ACA was enacted in the United States. The ACA includes measures that have significantly changed, and are expected to continue to significantly change, the way healthcare is financed by both governmental and private insurers. Among the provisions of the ACA of greatest importance to the pharmaceutical industry are that the ACA:

- made several changes to the Medicaid Drug Rebate Program, including increasing pharmaceutical manufacturers' rebate liability by raising the minimum basic Medicaid rebate on most branded prescription drugs to 23.1% of average manufacturer price, or AMP, and adding a new rebate calculation for "line extensions" (i.e., new formulations, such as extended release formulations) of solid oral dosage forms of branded products, as well as potentially impacting their rebate liability by modifying the statutory definition of AMP.
- imposed a requirement on manufacturers of branded drugs to provide a 70% point-of-sale discount off the negotiated price of branded drugs dispensed to Medicare Part D beneficiaries in the coverage gap (i.e., "donut hole") as a condition for a manufacturer's outpatient drugs being covered under Medicare Part D.
- extended a manufacturer's Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations.
- expanded the entities eligible for discounts under the 340B Drug Discount Program.
- established a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted, or injected.
- imposed an annual, nondeductible fee on any entity that manufactures or imports certain branded prescription drugs, apportioned among these entities according to their market share in certain government healthcare programs.
- established a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research. The research conducted by the Patient-Centered Outcomes Research Institute may affect the market for certain pharmaceutical products. The ACA established the Center for Medicare and Medicaid Innovation within CMS to test innovative payment and service delivery models to lower Medicare and Medicaid spending, potentially including prescription drug spending. Funding has been allocated to support the mission of the Center for Medicare and Medicaid Innovation through 2019.

Some of the provisions of the ACA have yet to be fully implemented, while certain provisions have been subject to judicial and Congressional challenges. Further, on January 20, 2017, President Trump signed an Executive Order directing federal agencies with authorities and responsibilities under the ACA waive, defer, grant

exemptions from, or delay the implementation of any provision of the ACA that would impose a fiscal burden on states or a cost, fee, tax, penalty or regulatory burden on individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. In addition, CMS has recently finalized regulations that would give states greater flexibility in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the ACA for plans sold through such marketplaces. The Tax Act includes a provision repealing, effective January 1, 2019, the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the “individual mandate.” Additionally, on January 22, 2018, President Trump signed a continuing resolution on appropriations for fiscal year 2018 that delayed the implementation of certain ACA-mandated fees, including the so-called “Cadillac” tax on certain high cost employer-sponsored insurance plans, the annual fee imposed on certain health insurance providers based on market share, and the medical device exercise tax on non-exempt medical devices. Further, the Bipartisan Budget Act of 2018, or the BBA, among other things, amends the ACA, effective January 1, 2019, to reduce the coverage gap in most Medicare drug plans, commonly referred to as the “donut hole.” In July 2018, CMS announced that it is suspending further collections and payments to and from certain ACA qualified health plans and health insurance issuers under the ACA risk adjustment program pending the outcome of federal district court litigation regarding the method CMS uses to determine this risk adjustment. On December 14, 2018, a U.S. District Court Judge in the Northern District of Texas, or the Texas District Court Judge, ruled that the individual mandate is a critical and inseparable feature of the ACA, and therefore, because it was repealed as part of the Tax Act, the remaining provisions of the ACA are invalid as well. The Texas District Court Judge, as well as the Trump Administration and CMS, have stated that the ruling will have no immediate effect, and on December 30, 2018 the Texas District Court Judge issued an order staying the judgment pending appeal. It is unclear how this decision, subsequent appeals, and other efforts to repeal and replace the ACA will impact the ACA.

Congress also could consider additional legislation to repeal, replace, or further modify elements of the ACA. Thus, the full impact of the ACA, or any law replacing elements of it, and the political uncertainty regarding any repeal and replacement on the ACA, on our business remains unclear. Many of the details regarding the implementation of the ACA are yet to be determined, and at this time, it remains unclear the full effect that the ACA would have on our business. There have been judicial and Congressional challenges to the ACA, and we expect such challenges and amendments to continue in the future.

Moreover, in May 2018, the Trump administration released its “Blueprint to Lower Drug Prices and Reduce Out-of-Pocket Costs,” or the Blueprint. The Blueprint contains several potential regulatory actions and legislative recommendations aimed at lowering prescription drug prices, including measures to promote innovation and competition for biologics, changes to Medicare Part D to give plan sponsors more leverage when negotiating prices with manufacturers, and updating the Medicare drug-pricing dashboard to make price increases and generic competition more transparent. In addition, the Department of HHS released a Request for Information, or RFI, soliciting public input on ways to lower drug pricing. Together, the recommendations in the Blueprint and RFI, if enacted by Congress and HHS, could lead to changes to Medicare Parts B and D, including the transition of certain drugs covered under Part B to Part D or the offering of alternative purchasing options under the Competitive Acquisition Program that currently applies to selected drugs and biologics covered under Part B. In September 2018, CMS announced that it will allow Medicare Advantage Plans the option to use step therapy for Part B drugs beginning January 1, 2019. While most of the proposed measures will require authorization through additional legislation to become effective, Congress and the Trump administration have each indicated that it will continue to seek new legislative, administrative and/or additional measures to control drug costs.

Other legislative changes have been proposed and adopted in the United States since the ACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least \$1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation’s automatic reduction to several government programs. This includes aggregate reductions of

Medicare payments to providers up to 2% per fiscal year, which went into effect in April 2013, following passage of the Bipartisan Budget Act of 2013, and will remain in effect through 2027 unless additional congressional action is taken. Further, in January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several providers, including hospitals, imaging centers and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors, which may adversely affect our future profitability. Additionally, there has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices.

Specifically, there have been several recent U.S. Congressional inquiries and proposed bills designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs and reform government program reimbursement methodologies for drugs.

Further, on May 30, 2018, the Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017, or the Right to Try Act, was signed into law. The law, among other things, provides a federal framework for certain patients to request access to certain investigational new drug products that have completed a Phase I clinical trial and that are undergoing investigation for FDA approval. There is no obligation for a pharmaceutical manufacturer to make its drug products available to eligible patients as a result of the Right to Try Act.

We cannot predict what healthcare reform initiatives may be adopted in the future. Further federal, state and foreign legislative and regulatory developments are likely, and we expect ongoing initiatives to increase pressure on drug pricing. Such reforms could have an adverse effect on anticipated revenues from product candidates and may affect our overall financial condition and ability to develop product candidates.

### ***Packaging and Distribution in the United States***

If our products are made available to authorized users of the Federal Supply Schedule of the General Services Administration, additional laws and requirements apply. Products must meet applicable child-resistant packaging requirements under the U.S. Poison Prevention Packaging Act. Manufacturing, sales, promotion and other activities also are potentially subject to federal and state consumer protection and unfair competition laws.

The distribution of pharmaceutical products is subject to additional requirements and regulations, including extensive record-keeping, licensing, storage and security requirements intended to prevent the unauthorized sale of pharmaceutical products.

The failure to comply with any of these laws or regulatory requirements subjects firms to possible legal or regulatory action. Depending on the circumstances, failure to meet applicable regulatory requirements can result in criminal prosecution, fines or other penalties, injunctions, exclusion from federal healthcare programs, requests for recall, seizure of products, total or partial suspension of production, denial or withdrawal of product approvals, or refusal to allow a firm to enter into supply contracts, including government contracts. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. Prohibitions or restrictions on sales or withdrawal of future products marketed by us could materially affect our business in an adverse way.

Changes in regulations, statutes or the interpretation of existing regulations could impact our business in the future by requiring, for example: (i) changes to our manufacturing arrangements; (ii) additions or modifications to product labeling; (iii) the recall or discontinuation of our products; or (iv) additional record-keeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business.



***Other U.S. Environmental, Health and Safety Laws and Regulations***

We may be subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. From time to time and in the future, our operations may involve the use of hazardous and flammable materials, including chemicals and biological materials, and may also produce hazardous waste products. Even if we contract with third parties for the disposal of these materials and waste products, we cannot completely eliminate the risk of contamination or injury resulting from these materials. In the event of contamination or injury resulting from the use or disposal of our hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

We maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees, but this insurance may not provide adequate coverage against potential liabilities. However, we do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. Current or future environmental laws and regulations may impair our research, development or production efforts. In addition, failure to comply with these laws and regulations may result in substantial fines, penalties or other sanctions.

***U.S. Patent Term Restoration and Marketing Exclusivity***

Depending upon the timing, duration and specifics of FDA approval of our future product candidates, some of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit restoration of the patent term of up to five years as compensation for patent term lost during the FDA regulatory review process. Patent-term restoration, however, cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date and only those claims covering such approved drug product, a method for using it or a method for manufacturing it may be extended. The patent-term restoration period is generally one-half the time between the effective date of an IND and the submission date of an NDA or BLA plus the time between the submission date of an NDA or BLA and the approval of that application, except that the review period is reduced by any time during which the applicant failed to exercise due diligence. Only one patent applicable to an approved drug is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. The USPTO, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we may apply for restoration of patent term for our currently owned or licensed patents to add patent life beyond its current expiration date, depending on the expected length of the clinical trials and other factors involved in the filing of the relevant NDA or BLA.

Marketing exclusivity provisions under the FDCA also can delay the submission or the approval of certain applications. The FDCA provides a five-year period of non-patent marketing exclusivity within the United States to the first applicant to gain approval of an NDA for a new chemical entity. A drug is a new chemical entity if the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the action of the drug substance. During the exclusivity period, the FDA may not accept for review an ANDA, or a 505(b)(2) NDA submitted by another company for another version of such drug where the applicant does not own or have a legal right of reference to all the data required for approval. However, an application may be submitted after four years if it contains a certification of patent invalidity or non-infringement. The FDCA also provides three years of marketing exclusivity for an NDA, 505(b)(2) NDA or supplement to an existing NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application, for example, new indications, dosages or strengths of an existing drug. This three-year exclusivity

covers only the conditions of use associated with the new clinical investigations and does not prohibit the FDA from approving ANDAs for drugs containing the original active agent. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA. However, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

### ***European Union Drug Development***

In the European Union, our future products also may be subject to extensive regulatory requirements. As in the United States, medicinal products can be marketed only if a marketing authorization from the competent regulatory agencies has been obtained.

Similar to the United States, the various phases of preclinical and clinical research in the European Union are subject to significant regulatory controls. Although the EU Clinical Trials Directive 2001/20/EC has sought to harmonize the EU clinical trials regulatory framework, setting out common rules for the control and authorization of clinical trials in the European Union, the EU Member States have transposed and applied the provisions of the Directive differently. This has led to significant variations in the member state regimes. Under the current regime, before a clinical trial can be initiated it must be approved in each of the EU countries where the trial is to be conducted by two distinct bodies: the National Competent Authority, or NCA, and one or more Ethics Committees, or ECs. Under the current regime all suspected unexpected serious adverse reactions to the investigated drug that occur during the clinical trial have to be reported to the NCA and ECs of the Member State where they occurred.

The EU clinical trials legislation currently is undergoing a transition process mainly aimed at harmonizing and streamlining clinical-trial authorization, simplifying adverse-event reporting procedures, improving the supervision of clinical trials and increasing their transparency. Recently enacted Clinical Trials Regulation EU No 536/2014 ensures that the rules for conducting clinical trials in the European Union will be identical.

### ***European Union Drug Marketing***

Much like the Anti-Kickback Statute prohibition in the United States, the provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is also prohibited in the European Union. The provision of benefits or advantages to physicians is governed by the national anti-bribery laws of European Union Member States, such as the UK Bribery Act 2010. Infringement of these laws could result in substantial fines and imprisonment.

Payments made to physicians in certain European Union Member States must be publically disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her competent professional organization and/or the regulatory authorities of the individual EU Member States. These requirements are provided in the national laws, industry codes or professional codes of conduct, applicable in the EU Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

### ***European Union Drug Review and Approval***

In the European Economic Area, or EEA, which is comprised of the 27 Member States of the European Union (including Norway and excluding Croatia), Iceland and Liechtenstein, medicinal products can only be commercialized after obtaining a Marketing Authorization, or MA. There are two types of marketing authorizations.

- The Community MA is issued by the European Commission through the Centralized Procedure, based on the opinion of the Committee for Medicinal Products for Human Use, or CHMP, of the EMA, and is valid

throughout the entire territory of the EEA. The Centralized Procedure is mandatory for certain types of products, such as biotechnology medicinal products, orphan medicinal products, advanced-therapy medicines such as gene-therapy, somatic cell-therapy or tissue-engineered medicines and medicinal products containing a new active substance indicated for the treatment of HIV, AIDS, cancer, neurodegenerative disorders, diabetes, auto-immune and other immune dysfunctions and viral diseases. The Centralized Procedure is optional for products containing a new active substance not yet authorized in the EEA, or for products that constitute a significant therapeutic, scientific or technical innovation or which are in the interest of public health in the European Union.

- National MAs, which are issued by the competent authorities of the Member States of the EEA and only cover their respective territory, are available for products not falling within the mandatory scope of the Centralized Procedure. Where a product has already been authorized for marketing in a Member State of the EEA, this National MA can be recognized in another Member States through the Mutual Recognition Procedure. If the product has not received a National MA in any Member State at the time of application, it can be approved simultaneously in various Member States through the Decentralized Procedure. Under the Decentralized Procedure an identical dossier is submitted to the competent authorities of each of the Member States in which the MA is sought, one of which is selected by the applicant as the Reference Member State, or RMS. The competent authority of the RMS prepares a draft assessment report, a draft summary of the product characteristics, or SPC, and a draft of the labeling and package leaflet, which are sent to the other Member States (referred to as the Member States Concerned) for their approval. If the Member States Concerned raise no objections, based on a potential serious risk to public health, to the assessment, SPC, labeling, or packaging proposed by the RMS, the product is subsequently granted a national MA in all the Member States (i.e., in the RMS and the Member States Concerned).

Under the above described procedures, before granting the MA, the EMA or the competent authorities of the Member States of the EEA make an assessment of the risk-benefit balance of the product on the basis of scientific criteria concerning its quality, safety and efficacy.

### ***European Union New Chemical Entity Exclusivity***

In the European Union, new chemical entities, sometimes referred to as new active substances, qualify for eight years of data exclusivity upon marketing authorization and an additional two years of market exclusivity. The data exclusivity, if granted, prevents regulatory authorities in the European Union from referencing the innovator's data to assess a generic application for eight years, after which generic marketing authorization can be submitted, and the innovator's data may be referenced, but not approved for two years. The overall ten-year period will be extended to a maximum of 11 years if, during the first eight years of those ten years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are determined to bring a significant clinical benefit in comparison with currently approved therapies.

### ***European Union Orphan Designation and Exclusivity***

In the European Union, the EMA's Committee for Orphan Medicinal Products grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting not more than 5 in 10,000 persons in the European Union community (or where it is unlikely that the development of the medicine would generate sufficient return to justify the investment) and for which no satisfactory method of diagnosis, prevention or treatment has been authorized (or, if a method exists, the product would be a significant benefit to those affected).

In the European Union, orphan drug designation entitles a party to financial incentives such as reduction of fees or fee waivers and ten years of market exclusivity is granted following medicinal product approval. This period may be reduced to six years if the orphan drug designation criteria are no longer met, including where it is shown

that the product is sufficiently profitable not to justify maintenance of market exclusivity. Orphan drug designation must be requested before submitting an application for marketing approval. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

### ***European Pediatric Investigation Plan***

In the EEA, MAAs for new medicinal products not authorized have to include the results of studies conducted in the pediatric population, in compliance with a pediatric investigation plan, or PIP, agreed with the EMA's Pediatric Committee, or PDCO. The PIP sets out the timing and measures proposed to generate data to support a pediatric indication of the drug for which marketing authorization is being sought. The PDCO can grant a deferral of the obligation to implement some or all of the measures of the PIP until there are sufficient data to demonstrate the efficacy and safety of the product in adults. Further, the obligation to provide pediatric clinical trial data can be waived by the PDCO when this data is not needed or appropriate because the product is likely to be ineffective or unsafe in children, the disease or condition for which the product is intended occurs only in adult populations, or when the product does not represent a significant therapeutic benefit over existing treatments for pediatric patients. Once the marketing authorization is obtained in all Member States of the European Union and trial results are included in the product information, even when negative, the product is eligible for six months' supplementary protection certificate extension.

### ***European Data Collection***

The collection and use of personal health data in the European Economic Area, or the EEA, is governed by the GDPR, which became effective May 25, 2018. The GDPR applies to any company established in the EEA and to companies established outside the EEA that process personal data in connection with the offering of goods or services to data subjects in the EU or the monitoring of the behavior of data subjects in the European Union. The GDPR enhances data protection obligations for data controllers of personal data, including stringent requirements relating to the consent of data subjects, expanded disclosures about how personal data is used, requirements to conduct privacy impact assessments for "high risk" processing, limitations on retention of personal data, mandatory data breach notification and "privacy by design" requirements, and creates direct obligations on service providers acting as data processors. The GDPR also imposes strict rules on the transfer of personal data outside of the EEA to countries that do not ensure an adequate level of protection, like the U.S. Failure to comply with the requirements of the GDPR and the related national data protection laws of the EEA Member States may result in fines up to 20 million Euros or 4% of a company's global annual revenues for the preceding financial year, whichever is higher. Moreover, the GDPR grants data subjects the right to claim material and non material damages resulting from infringement of the GDPR. Given the breadth and depth of changes in data protection obligations, maintaining compliance with the GDPR, will require significant time, resources and expense, and we may be required to put in place additional mechanisms ensuring compliance with the new data protection rules. This may be onerous and adversely affect our business, financial condition, results of operations and prospects.

### ***Rest of the World Regulation***

For other countries outside of the European Union and the United States, such as countries in Eastern Europe, Latin America or Asia, the requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. Additionally, the clinical trials must be conducted in accordance with GCP requirements and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

If we fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

### ***Additional Laws and Regulations Governing International Operations***

If we further expand our operations outside of the United States, we must dedicate additional resources to comply with numerous laws and regulations in each jurisdiction in which we plan to operate. The Foreign Corrupt Practices Act, or FCPA, prohibits any U.S. individual or business from paying, offering, authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations.

Compliance with the FCPA is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

Various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. If we expand our presence outside of the United States, it will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain products and product candidates outside of the United States, which could limit our growth potential and increase our development costs.

The failure to comply with laws governing international business practices may result in substantial civil and criminal penalties and suspension or debarment from government contracting. The SEC also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

### ***Coverage and Reimbursement***

Successful commercialization of new drug products depends in part on the extent to which reimbursement for those drug products will be available from government health administration authorities, private health insurers, and other organizations. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which drug products they will pay for and establish reimbursement levels. The availability and extent of reimbursement by governmental and private payors is essential for most patients to be able to afford a drug product. Sales of drug products depend substantially, both domestically and abroad, on the extent to which the costs of drugs products are paid for by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or reimbursed by government health administration authorities, private health coverage insurers and other third-party payors.

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular drug products. In many countries, the prices of drug products are subject to varying price control mechanisms as part of national health systems. In general, the prices of drug products under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for drug products, but monitor and control company profits. Accordingly, in markets outside the United States, the reimbursement for drug products may be reduced compared with the United States.

In the United States, the principal decisions about reimbursement for new drug products are typically made by CMS, an agency within the HHS. CMS decides whether and to what extent a new drug product will be covered and reimbursed under Medicare, and private payors tend to follow CMS to a substantial degree. However, no uniform policy of coverage and reimbursement for drug products exists among third-party payors and coverage and reimbursement levels for drug products can differ significantly from payor to payor.

The Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or the MMA, established the Medicare Part D program to provide a voluntary prescription drug benefit to Medicare beneficiaries. Under Part D, Medicare beneficiaries may enroll in prescription drug plans offered by private entities that provide coverage of outpatient prescription drugs. Unlike Medicare Parts A and B, Part D coverage is not standardized. Part D prescription drug plan sponsors are not required to pay for all covered Part D drugs, and each drug plan can develop its own drug formulary that identifies which drugs it will cover and at what tier or level. While all Medicare drug plans must give at least a standard level of coverage set by Medicare, Part D prescription drug plan sponsors are not required to pay for all covered Part D drugs, and each drug plan can develop its own drug formulary that identifies which drugs it will cover and at what tier or level. However, Part D prescription drug formularies must include drugs within each therapeutic category and class of covered Part D drugs, though not necessarily all the drugs in each category or class. Any formulary used by a Part D prescription drug plan must be developed and reviewed by a pharmacy and therapeutic committee. Government payment for some of the costs of prescription drugs may increase demand for drugs for which we obtain marketing approval. Any negotiated prices for any of our products covered by a Part D prescription drug plan will likely be lower than the prices we might otherwise obtain. Moreover, while the MMA applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own payment rates. Any reduction in payment that results from the MMA may result in a similar reduction in payments from non-governmental payors.

For a drug product to receive federal reimbursement under the Medicaid or Medicare Part B programs or to be sold directly to U.S. government agencies, the manufacturer must extend discounts to entities eligible to participate in the 340B drug pricing program. The required 340B discount on a given product is calculated based on the average manufacturer price, or AMP, and Medicaid rebate amounts reported by the manufacturer. As of 2010, the ACA expanded the types of entities eligible to receive discounted 340B pricing, although under the current state of the law these newly eligible entities (with the exception of children's hospitals) will not be eligible to receive discounted 340B pricing on orphan drugs. As 340B drug pricing is determined based on AMP and Medicaid rebate data, the revisions to the Medicaid rebate formula and AMP definition described above could cause the required 340B discount to increase. The American Recovery and Reinvestment Act of 2009 provides funding for the federal government to compare the effectiveness of different treatments for the same illness. The plan for the research was published in 2012 by the Department of HHS, the Agency for Healthcare Research and Quality and the National Institutes for Health, and periodic reports on the status of the research and related expenditures are made to Congress. Although the results of the comparative effectiveness studies are not intended to mandate coverage policies for public or private payors, it is not clear what effect, if any, the research will have on the sales of our drug candidates, if any such drug or the condition that they are intended to treat are the subject of a trial. It is also possible that comparative effectiveness research demonstrating benefits in a competitor's drug could adversely affect the sales of our drug candidate. If third-party payors do not consider our drugs to be cost-effective compared to other available therapies, they may not cover our drugs after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow us to sell our drugs on a profitable basis.

These laws, and future state and federal healthcare reform measures may be adopted in the future, any of which may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

Outside of the United States, the pricing of pharmaceutical products and medical devices is subject to governmental control in many countries. For example, in the European Union, pricing and reimbursement schemes vary widely from country to country. Some countries provide that products may be marketed only after a reimbursement price has been agreed. Some countries may require the completion of additional studies that compare the cost effectiveness of a particular therapy to currently available therapies or so-called health technology assessments, in order to obtain reimbursement or pricing approval. Other countries may allow companies to fix their own prices for products, but monitor and control product volumes and issue guidance to

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physicians to limit prescriptions. Efforts to control prices and utilization of pharmaceutical products and medical devices will likely continue as countries attempt to manage healthcare expenditures.

### **Employees**

As of March 31, 2019, we had 152 full-time employees, including 19 through our wholly-owned subsidiary, BridgeBio Services Inc. We have never had a work stoppage, and none of our employees is represented by a labor organization or under any collective-bargaining arrangements. We consider our employee relations to be good.

### **Facilities**

We lease our office space, which consists of approximately 3,900 square feet located in Palo Alto, California. Our lease expires on April 30, 2023. We believe our current office is sufficient to meet our needs for the foreseeable future.

### **Legal Proceedings**

As of the date of this prospectus, we were not party to any legal matters or claims that, in the opinion of our management, are likely to have a material adverse effect on our business. In the future, we may become party to legal matters and claims arising in the ordinary course of business, the resolution of which we do not anticipate would have a material adverse impact on our financial position, results of operations or cash flows.

## MANAGEMENT

### Executive Officers and Directors

The following table and discussion sets forth the name, age as of March 31, 2019 and position of the individuals who currently serve as directors and executive officers of BridgeBio Pharma LLC and will begin to serve as the directors and executive officers of BridgeBio Pharma, Inc. in connection with this offering. The following also includes certain information regarding our directors' and officers' individual experience, qualifications, attributes and skills and brief statements of those aspects of our directors' backgrounds that led us to conclude that they are qualified to serve as directors.

<u>Name</u>	<u>Age</u>	<u>Position(s)</u>
<b>Executive Officers</b>		
Neil Kumar, Ph.D.	40	Chief Executive Officer, Director
Brian C. Stephenson, Ph.D., CFA	38	Chief Financial Officer
Uma Sinha, Ph.D.	62	Chief Scientific Officer
Charles Homcy, M.D.(3)	70	Chairman of Pharmaceuticals, Director
Frank McCormick, Ph.D., F.R.S., D.Sc. (Hon)	68	Chairman of Oncology
Richard H. Scheller, Ph.D.(3)	65	Chairman of Research & Development, Director
Michael Henderson, M.D.	29	Senior Vice President, Asset Acquisition, Strategy and Operations
Cameron Turtle, D. Phil.	29	Senior Vice President, Portfolio Management and Corporate Development
<b>Non-Management Directors</b>		
Eric Aguiar, M.D.(1)(2)(3)	56	Director
James C. Momtazee(1)(2)	47	Director
Ali J. Satvat(1)(2)(3)	41	Director

(1) Member of the Audit Committee.

(2) Member of the Compensation Committee.

(3) Member of the Nominating and Corporate Governance Committee.

### Executive Officers

**Neil Kumar, Ph.D.**, is a co-founder and has served as our Chief Executive Officer and a member of our board of directors since April 2015. Dr. Kumar has also served as the Chief Executive Officer of our subsidiary, Eidos Therapeutics, Inc. (Nasdaq: EIDX), a clinical stage biopharmaceutical company, and a member of Eidos Therapeutics' board of directors since March 2016. Prior to that, he served as the interim vice president of business development at MyoKardia, Inc. (Nasdaq: MYOK), a clinical stage biopharmaceutical company, from 2012 to 2014. Prior to that, Dr. Kumar served as a principal at Third Rock Ventures, a venture capital firm, from 2011 to 2014. Before joining Third Rock Ventures, he served as an associate principal at McKinsey & Company, a worldwide management consulting firm, from 2007 to 2011. He received his B.S. and M.S. degrees in chemical engineering from Stanford University and received his Ph.D. in chemical engineering from the Massachusetts Institute of Technology.

We believe that Dr. Kumar is qualified to serve as a member of our board of directors based on his perspective and experience he brings as our founder and Chief Executive Officer and his extensive experience in the health care industry.

**Brian C. Stephenson, Ph.D., CFA** has served as our Chief Financial Officer since October 2018. Prior to joining us, Dr. Stephenson served as Partner and the Head of Life Sciences for Capital IP Investment Partners, a special situation investment fund, from 2015 to 2018. From 2011 to 2014, Dr. Stephenson was a Director/Vice President Leerink Partners, an investment bank. Prior to that, Dr. Stephenson was an Engagement Manager at McKinsey & Company, a worldwide management consulting firm. He received his Ph.D. and M.S. degrees in chemical engineering from the Massachusetts Institute of Technology and his B.S. in chemical engineering from Brigham Young University. Dr. Stephenson is also a Chartered Financial Analyst charterholder.



**Uma Sinha, Ph.D.** has served as Chief Scientific Officer since April 2016 and serves as the chief scientific officer of other BridgeBio subsidiaries, including Eidos Therapeutics. Prior to that, Dr. Sinha served as chief scientific officer of Global Blood Therapeutics, Inc. (Nasdaq: GBT), a clinical stage biopharmaceutical company, from 2014 to 2015 and previously as senior vice president of research from 2013 to 2014. She was vice president, head of biology at Portola Pharmaceuticals, Inc. (Nasdaq: PTLA), a clinical stage biotechnology company, from 2010 to 2012 and was the vice president of translational biology from 2004 to 2010. Previously, Dr. Sinha held senior research positions at Millennium Pharmaceuticals, Inc., a biopharmaceutical company, and COR Therapeutics, Inc., a biopharmaceutical company. Dr. Sinha received her Ph.D. in biochemistry from the University of Georgia and her B.Sc. with honors in chemistry from Presidency College.

**Charles Homcy, M.D.** has served as a member of our board of directors since November 2018 and as our Chairman of Pharmaceuticals since February 2019. In 2010, Dr. Homcy joined Third Rock Ventures, a venture capital firm, where he is currently a partner. In 2003, he co-founded Portola Pharmaceuticals (Nasdaq: PTLA), a clinical biotechnology company, and he served as their president and chief executive officer until 2010. Prior to that, Dr. Homcy served as the president of research and development at Millennium Pharmaceuticals, Inc. (currently, Takeda Oncology), a biopharmaceutical company, following its acquisition of COR Therapeutics, Inc. in 2002. He joined COR Therapeutics, Inc., a biopharmaceutical company, in 1995 as executive vice president of research and development, and he served as a director of the company from 1998 to 2002. Dr. Homcy was a clinical professor of medicine at the University of California, San Francisco Medical School, and attending physician at the San Francisco Veterans Affairs Hospital from 1997 to 2011. He was previously president of the medical research division of American Cyanamid-Lederle Laboratories, a division of Wyeth-Ayest Laboratories. He currently serves on the board of directors of Portola Pharmaceuticals, Inc., a position he has held since 2004, and of Global Blood Therapeutics, Inc., a position he has held since 2012. Dr. Homcy holds a B.A. and an M.D. from Johns Hopkins University and currently serves on its board of trustees.

We believe that Dr. Homcy is qualified to serve as a member of our board of directors based on his significant experience building and leading successful biotechnology companies and his scientific expertise.

**Frank McCormick, Ph.D., F.R.S., D.Sc. (Hon)** has served as our Chairman of Oncology since April 2019. Dr. McCormick has held the positions of Director the UCSF Helen Diller Family Comprehensive Cancer Center, a multidisciplinary research and medical care organization and served as Associate Dean of the UCSF School of Medicine from 1997-2014. Prior to joining the UCSF faculty, Dr. McCormick pursued cancer-related work with several biotechnology firms, including Cetus Corporation as Director of Molecular Biology from 1981 to 1990 and Vice President of Research from 1990 to 1991, and Chiron Corporation as Vice President of Research from 1991 to 1992. In 1992, Dr. McCormick founded Onyx Pharmaceuticals and served as its Chief Scientific Officer until 1996. Dr. McCormick received his B.Sc. in biochemistry from the University of Birmingham, and his Ph.D. in biochemistry from the University of Cambridge and held postdoctoral fellowships in the U.S. at the State University of New York at Stony Brook and in London at the Imperial Cancer Research Fund. Dr. McCormick is a Fellow of the Royal Society, an institution dedicated to science, since 1996, a member of the National Academy of Sciences since 2014 and has served as President, 2012-2013, for the American Association for Cancer Research. Since 2013, Dr. McCormick has led the National Cancer Institute's Ras Initiative at the Frederick National Laboratories for Cancer Research overseeing the national effort to develop therapies against Ras-driven cancers.

**Richard H. Scheller, Ph.D.** has served as a member of our board of directors since January 2018 and as our Chairman of Research & Development effective as of January 2019. Dr. Scheller has been Chief Science Officer and Head of Therapeutics at 23andMe, a personal genetics company, since 2015. Previously, Dr. Scheller was the Executive Vice President of Research and Early Development and a member of the Executive Committee at Genentech, Inc., a biotechnology corporation, from February 2001 to December 2014. From January 2009 to December 2014, Dr. Scheller was also a member of the Enlarged Executive Committee at Hoffmann-La Roche Ltd, a pharmaceutical company. Dr. Scheller currently serves as a member of the board of directors of Xenon Pharmaceuticals Inc., a clinical stage biopharmaceutical company, ORIC Pharmaceuticals, Inc., a biopharmaceutical company, Affinita Biotech, Inc., a preclinical stage biotech company and Alector, Inc., a

clinical-stage biopharmaceutical company. Dr. Scheller holds a B.Sc. in Biochemistry from the University of Wisconsin-Madison and a Ph.D. in Chemistry from the California Institute of Technology. He completed his post-doctorate in Molecular Neurobiology at Columbia University and was also a post-doctorate fellow at California Institute of Technology.

We believe that Dr. Scheller is qualified to serve as a member of our board of directors based on his scientific background and his senior management experience in the pharmaceutical industry.

**Michael Henderson, M.D.** has served as our Senior Vice President, Asset Acquisition, Strategy and Operations since December 2017. Dr. Henderson joined BridgeBio as our Vice President of Asset Acquisition, Strategy and Operations in April 2016. Dr. Henderson serves as the Chief Business Officer of certain of our subsidiaries, QED Therapeutics, Inc. and Origin Biosciences, Inc., since December 2017 and June 2018, respectively. Dr. Henderson also serves on the board of directors for certain of our subsidiaries, including Quartz Therapeutics, Inc. since September 2016, TheRas, Inc. since September 2016 and Adrenas Therapeutics, Inc. since July 2017. Prior to BridgeBio, Dr. Henderson worked as a Senior Associate at McKinsey & Company from January 2015 to April 2016 and prior to that, he co-founded PellePharm, Inc., in August 2011. Dr. Henderson received his B.A. with high honors in global health with a citation in Spanish from Harvard University and his M.D. with a scholarly concentration in health services and policy from Stanford University where he was a member of both the Ignite and Leadership in Health Disparities Programs.

**Cameron Turtle, D. Phil.** has served as our Senior Vice President, Portfolio Management and Corporate Development, since January 2018 and served as a Director, Portfolio Management, from February 2017 to December 2017. Mr. Turtle has also worked for our subsidiary, Eidos Therapeutics, since February 2017, currently as the chief business officer. Mr. Turtle previously worked as vice president of business development and operations for our subsidiary, Navire Pharmaceuticals, Inc., from February 2017 to March 2018. From August 2016 to January 2017, Mr. Turtle was a consultant at McKinsey & Company. Mr. Turtle received his B.S. with honors in bioengineering from the University of Washington and his D. Phil. in cardiovascular medicine from University of Oxford, St. John's College, where he was a Rhodes Scholar.

#### **Non-Management Directors**

**Eric Aguiar, M.D.** has served as a member of our board of directors since March 2019. Dr. Aguiar has been a partner at Aisling Capital since January 2016 and prior to that was a partner at Thomas, McNerney and Partners, a healthcare venture capital and growth equity fund, since 2007. Prior to joining that firm, he was a Managing Director of HealthCare Ventures, a healthcare focused venture capital firm, from 2001 to 2007. Dr. Aguiar currently serves on the board of directors of Invitae Corporation (NYSE: NVTA) since September 2010, Biohaven Corporation (NYSE: BHVN) since October 2016 and Eidos Therapeutics (Nasdaq: EIDX) since March 2018. Dr. Aguiar is a member of the Board of Overseers of the Tufts School of Medicine and a member of the Council on Foreign Relations. Dr. Aguiar received his medical degree with honors from Harvard Medical School. He graduated with honors from Cornell University as a College Scholar. He was also a Luce Fellow and is a Chartered Financial Analyst.

We believe that Dr. Aguiar is qualified to serve as a member of our board of directors based on his medical and finance background and experience as an investor in life science companies.

**James C. Montazee** has served as a member of our board of directors since March 2016. He is a Member of KKR, and he has been employed by Kohlberg Kravis Roberts & Co. L.P., a private equity and alternative asset management firm, since 1996. Mr. Montazee currently serves on the board of directors of PRA Health Sciences, Inc. (Nasdaq: PRAH), a global contract research organization as well as several private companies. He previously served on the boards of directors of Jazz Pharmaceuticals plc (Nasdaq: JAZZ), a biopharmaceutical company, from 2004 to 2014, HCA Healthcare Inc. (formerly HCA Holdings Inc.; NYSE: HCA), a health care services company, from 2006 to 2014, and Entellus Medical, Inc., a medical technology company, from 2017 to 2018. He received an A.B. from Stanford University and an M.B.A. from the Stanford Graduate School of Business.

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We believe that Mr. Momtazee is qualified to serve as a member of our board of directors based on his expertise in corporate governance, the health care industry and in financing and financial matters, including expertise and experience in structuring complex financial transactions.

**Ali J. Satvat** has served as a member of our board of directors since March 2016. Mr. Satvat has also served on the board of our subsidiary, Eidos Therapeutics, Inc., a clinical stage biopharmaceutical company, since June 2018. Mr. Satvat joined Kohlberg Kravis Roberts & Co. L.P., a global private equity and alternative asset management firm, in January 2012 and is a Member of KKR on the Health Care industry team within KKR's Americas Private Equity platform. Mr. Satvat leads KKR's Health Care Strategic Growth investing efforts and sits on the Health Care Strategic Growth Investment Committee and the Health Care Strategic Growth Portfolio Management Committee. Mr. Satvat has served as a member of the boards of directors of Coherus BioSciences, Inc. (Nasdaq: CHRS), a biotechnology company, since May 2014, PRA Health Sciences, Inc. (Nasdaq: PRAH), a global contract research organization, from September 2013 through April 2018, and multiple privately held companies. Prior to joining KKR, Mr. Satvat was a Principal with Apax Partners LLP, a global private equity firm, where he invested in health care from 2006 to 2012. Previously, Mr. Satvat held various positions with Johnson & Johnson Development Corporation, a venture capital subsidiary of Johnson & Johnson, Audax Group, a private equity firm, and The Blackstone Group, a global private equity and alternative asset management firm. Mr. Satvat holds an A.B. in History and Science from Harvard College and an M.B.A. in Health Care Management and Entrepreneurial Management from the Wharton School of the University of Pennsylvania. Mr. Satvat currently serves on the board of directors of the Healthcare Private Equity Association.

We believe that Mr. Satvat is qualified to serve as a member of our board of directors based on his expertise in corporate governance, financing, and financial matters and his extensive investment experience in the health care industry.

### **Composition of our Board of Directors**

Our board of directors consists of six members, each of whom will be members pursuant to the board composition provisions of our amended and restated certificate of incorporation.

Effective upon the completion of this offering, we intend to form a nominating and corporate governance committee. Our nominating and corporate governance committee and our board of directors may consider a broad range of factors relating to the qualifications and background of director nominees, which may include diversity, which is not only limited to race, gender or national origin, although we currently have no formal policy regarding board diversity. Our nominating and corporate governance committee's and our board of directors' priority in selecting board members is to identify persons who will further the interests of our stockholders through his or her established record of professional accomplishment, the ability to contribute positively to the collaborative culture among board members, knowledge of our business, understanding of the competitive landscape and professional and personal experiences and expertise relevant to our growth strategy. Our directors hold office until their successors have been elected and qualified or until the earlier of their resignation or removal. Our amended and restated certificate of incorporation and amended and restated bylaws that will become effective upon the completion of this offering also provide that our directors may be removed only for cause by the affirmative vote of the holders of at least 75% of the votes that all our stockholders would be entitled to cast in an annual election of directors, and that any vacancy on our board of directors, including a vacancy resulting from an enlargement of our board of directors, may be filled by vote of a majority of our directors then in office.

### **Director Independence**

Upon the completion of this offering, we expect that our common stock will be listed on the Nasdaq Global Market. Applicable rules of the Nasdaq Stock Market LLC, or Nasdaq, require a majority of a listed company's board of directors to be comprised of independent directors within one year of listing. In addition, the Nasdaq

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rules require that, (1) on the date of the completion of the offering, at least one member of each of a listed company's audit, compensation and nominating and corporate governance committees be independent, (2) within 90 days of the date of the completion of the offering, a majority of the members of such committees be independent and (3) within one year of the date of the completion of the offering, all the members of such committees be independent. Audit committee members must also satisfy the independence criteria set forth in Rule 10A-3 under the Exchange Act. Under applicable Nasdaq rules, a director will only qualify as an "independent director" if, in the opinion of the listed company's board of directors, that person does not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director.

In order to be considered independent for purposes of Rule 10A-3, a member of an audit committee of a listed company may not, other than in his or her capacity as a member of the audit committee, the board of directors, or any other board committee, accept, directly or indirectly, any consulting, advisory, or other compensatory fee from the listed company or any of its subsidiaries or otherwise be an affiliated person of the listed company or any of its subsidiaries.

Our board of directors has determined that Dr. Aguiar, Mr. Momtazee and Mr. Satvat are independent directors for purposes of the rules of Nasdaq and the SEC. In making such determination, our board of directors considered the relationships that each director has with us, and all other facts and circumstances that our board of directors deemed relevant in determining their independence, including the beneficial ownership of our capital stock by each director. Our board of directors also considered the association of our directors with the holders of more than 5% of our common stock. Upon the completion of this offering, we expect that the composition and functioning of our board of directors and each of our committees will comply with all applicable requirements of Nasdaq and the rules and regulations of the SEC, subject to the transition rules described above for newly listed companies. There are no family relationships among any of our directors or executive officers.

*Staggered board.* In accordance with the terms of our amended and restated certificate of incorporation and amended and restated bylaws that will become effective upon the completion of this offering, our board of directors will be divided into three classes, Class I, Class II and Class III, with each class serving staggered three-year terms. Upon the expiration of the term of a class of directors, directors in that class will be eligible to be elected for a new three-year term at the annual meeting of stockholders in the year in which their term expires.

- Our Class I directors will be Mr. Momtazee and Dr. Scheller;
- Our Class II directors will be Dr. Aguiar and Mr. Satvat; and
- Our Class III directors will be Dr. Kumar and Dr. Homcy.

Our amended and restated certificate of incorporation and amended and restated bylaws that will become effective upon the completion of this offering provide that the number of directors may be changed only by resolution of our board of directors.

The division of our board of directors into three classes with staggered three-year terms may delay or prevent stockholder efforts to effect a change of our management or a change of control.

### **Committees of our Board of Directors**

Our board of directors plans on establishing an audit committee, a compensation committee and a nominating and corporate governance committee, each of which will operate pursuant to a charter to be adopted by our board of directors and which will be effective upon completion of the offering. Following the completion of this offering, copies of each committee's charter will be posted on the Corporate Governance section of our website, at <https://bridgebio.com>. The inclusion of our website address in this prospectus does not incorporate by reference the information on or accessible through our website into this prospectus.

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*Audit committee.* Effective upon completion of this offering, Dr. Aguiar, Mr. Momtazee and Mr. Satvat will serve on the audit committee, which will be chaired by Dr. Aguiar. Our board of directors has determined that Dr. Aguiar is “independent” for audit committee purposes as that term is defined in the rules of the SEC and the applicable Nasdaq rules, and has sufficient knowledge in financial and auditing matters to serve on the audit committee. Our board of directors has designated Dr. Aguiar as an “audit committee financial expert,” as defined under the applicable rules of the SEC. We intend to rely on the phase-in provisions of Rule 10A-3 of the Exchange Act and the Nasdaq transition rules applicable to companies completing an initial public offering, and we plan to have an audit committee comprised solely of directors that are independent for purposes of serving on an audit committee within one year after our listing. The audit committee’s responsibilities include:

- appointing, approving the compensation of, and assessing the independence of our independent registered public accounting firm;
- pre-approving auditing and permissible non-audit services, and the terms of such services, to be provided by our independent registered public accounting firm;
- reviewing the overall audit plan with our independent registered public accounting firm and members of management responsible for preparing our financial statements;
- reviewing and discussing with management and our independent registered public accounting firm our annual and quarterly financial statements and related disclosures as well as critical accounting policies and practices used by us;
- coordinating the oversight and reviewing the adequacy of our internal control over financial reporting;
- establishing policies and procedures for the receipt and retention of accounting-related complaints and concerns;
- recommending based upon the audit committee’s review and discussions with management and our independent registered public accounting firm whether our audited financial statements shall be included in our Annual Report on Form 10-K;
- monitoring the integrity of our financial statements and our compliance with legal and regulatory requirements as they relate to our financial statements and accounting matters;
- preparing the audit committee report required by SEC rules to be included in our annual proxy statement;
- reviewing all related person transactions for potential conflict of interest situations and approving all such transactions; and
- reviewing quarterly earnings releases.

*Compensation committee.* Effective upon completion of this offering, Dr. Aguiar, Mr. Momtazee and Mr. Satvat will serve on the compensation committee, which will be chaired by Dr. Aguiar. Our board of directors has determined that each of Dr. Aguiar, Mr. Momtazee and Mr. Satvat is “independent” under the applicable rules and regulations of Nasdaq, and is a “non-employee director” as defined in Rule 16b-3 promulgated under the Exchange Act. We intend to rely on the Nasdaq transition rules applicable to companies completing an initial public offering, and we plan to have a compensation committee comprised solely of directors that are independent for purposes of serving on a compensation committee within one year after our listing. The compensation committee’s responsibilities include:

- annually reviewing and approving corporate goals and objectives relevant to the compensation of our Chief Executive Officer;
- evaluating the performance of our Chief Executive Officer in light of such corporate goals and objectives and determining the compensation of our Chief Executive Officer;
- reviewing and approving the compensation of our other executive officers;
- reviewing and establishing our overall management compensation, philosophy and policy;

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- overseeing and administering our compensation and similar plans;
- evaluating and assessing potential and current compensation advisors in accordance with the independence standards identified in the applicable Nasdaq rules;
- retaining and approving the compensation of any compensation advisors;
- reviewing and approving our policies and procedures for the grant of equity-based awards;
- reviewing and making recommendations to the board of directors with respect to director compensation;
- reviewing and discussing with management the compensation disclosure to be included in our annual proxy statement or Annual Report on Form 10-K; and
- reviewing and discussing with the board of directors the corporate succession plans for the Chief Executive Officer and other key officers.

*Nominating and corporate governance committee.* Effective upon completion of this offering, Dr. Aguiar, Dr. Homcy, Mr. Satvat and Dr. Scheller will serve on the nominating and corporate governance committee, which will be chaired by Mr. Satvat. Our board of directors has determined that each of Dr. Aguiar, Mr. Momtazee and Mr. Satvat is “independent” as defined in the applicable Nasdaq rules. We intend to rely on the Nasdaq transition rules applicable to companies completing an initial public offering, and we plan to have a nominating and corporate governance committee comprised solely of directors that are independent for purposes of serving on a nominating and corporate governance committee within one year after our listing. The nominating and corporate governance committee’s responsibilities include:

- developing and recommending to the board of directors criteria for board and committee membership;
- establishing procedures for identifying and evaluating board of director candidates, including nominees recommended by stockholders;
- reviewing the size and composition of the board of directors to ensure that it is composed of members containing the appropriate skills and expertise to advise us;
- identifying individuals qualified to become members of the board of directors;
- recommending to the board of directors the persons to be nominated for election as directors and to each of the board’s committees;
- developing and recommending to the board of directors a code of business conduct and ethics and a set of corporate governance guidelines;
- developing a mechanism by which violations of the code of business conduct and ethics can be reported in a confidential manner; and
- overseeing the evaluation of the board of directors and management.

Our board of directors may from time to time establish other committees.

### **Compensation Committee Interlocks and Insider Participation**

None of the members of our compensation committee has at any time during the prior three years been one of our officers or employees. None of our executive officers currently serves, or in the past fiscal year has served, as a member of the board of directors or compensation committee of any entity that has one or more executive officers serving on our board of directors or compensation committee.

### **Code of Business Conduct and Ethics**

We have adopted a code of business conduct and ethics that applies to all of our employees, officers and directors, including those officers responsible for financial reporting, which will be effective upon completion of

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this offering. Upon the completion of this offering, our code of business conduct and ethics will be available on our website at <https://bridgebio.com>. We intend to disclose any substantive amendments to the code, or any waivers of its requirements, on our website or in a Current Report on Form 8-K.

### **Board Leadership Structure and Board's Role in Risk Oversight**

We do not currently have a chairman of the board; however, once we are a public company, we may establish a role of chairman of the board that is separate from the role of Chief Executive Officer. We believe that separating these positions would allow our Chief Executive Officer to focus on our day-to-day business, while allowing the chairman of the board to lead the board of directors in its fundamental role of providing advice to and independent oversight of management. Our board of directors recognizes the time, effort and energy that the Chief Executive Officer is required to devote to his position in the current business environment, as well as the commitment required to serve as our chairman, particularly as the board of directors' oversight responsibilities continue to grow. While our amended and restated bylaws and corporate governance guidelines will not require that our chairman and Chief Executive Officer positions be separate, our board of directors believes that having separate positions may provide the appropriate leadership structure for us and would demonstrate our commitment to good corporate governance.

Risk is inherent with every business, and how well a business manages risk can ultimately determine its success. We face a number of risks, including risks relating to our financial condition, development and commercialization activities, operations, strategic direction and intellectual property as more fully discussed in the section titled "Risk Factors" appearing elsewhere in this prospectus. Management is responsible for the day-to-day management of risks we face, while our board of directors, as a whole and through its committees, has responsibility for the oversight of risk management. In its risk oversight role, our board of directors has the responsibility to satisfy itself that the risk management processes designed and implemented by management are adequate and functioning as designed.

The role of the board of directors in overseeing the management of our risks is conducted primarily through committees of the board of directors, as disclosed in the descriptions of each of the committees above and in the charters of each of the committees. The full board of directors (or the appropriate board committee in the case of risks that are under the purview of a particular committee) discusses with management our major risk exposures, their potential impact on us, and the steps we take to manage them. When a board committee is responsible for evaluating and overseeing the management of a particular risk or risks, the chairman of the relevant committee reports on the discussion to the full board of directors during the committee reports portion of the next board meeting. This enables the board of directors and its committees to coordinate the risk oversight role, particularly with respect to risk interrelationships.

### **Limitation on Liability and Indemnification Matters**

Our amended and restated certificate of incorporation, which will become effective immediately prior to the consummation of this offering, will contain provisions that limit the liability of our directors for monetary damages to the fullest extent permitted by Delaware law. Consequently, our directors will not be personally liable to us or our stockholders for monetary damages for any breach of fiduciary duties as directors, except liability for:

- any breach of the director's duty of loyalty to us or our stockholders;
- any act or omission not in good faith or that involves intentional misconduct or a knowing violation of law;
- unlawful payments of dividends or unlawful stock repurchases or redemptions as provided in Section 174 of the Delaware General Corporation Law; or
- any transaction from which the director derived an improper personal benefit.

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Each of our amended and restated certificate of incorporation and amended and restated bylaws, which will become effective immediately prior to the consummation of this offering, will provide that we are required to indemnify our directors and officers, in each case to the fullest extent permitted by Delaware law. Our amended and restated bylaws will also obligate us to advance expenses incurred by a director or officer in advance of the final disposition of any action or proceeding, and permit us to secure insurance on behalf of any officer, director, employee or other agent for any liability arising out of his or her actions in that capacity regardless of whether we would otherwise be permitted to indemnify him or her under Delaware law. We intend to enter into agreements to indemnify our directors, executive officers and other employees as determined by our board of directors. With specified exceptions, these agreements provide for indemnification for related expenses including, among other things, attorneys' fees, judgments, fines and settlement amounts incurred by any of these individuals in any action or proceeding. We believe that these bylaw provisions and indemnification agreements are necessary to attract and retain qualified persons as directors and officers. We also maintain directors' and officers' liability insurance.

The limitation of liability and indemnification provisions in our amended and restated certificate of incorporation and amended and restated bylaws may discourage stockholders from bringing a lawsuit against our directors and officers for breach of their fiduciary duty. They may also reduce the likelihood of derivative litigation against our directors and officers, even though an action, if successful, might benefit us and our stockholders. Further, a stockholder's investment may be adversely affected to the extent that we pay the costs of settlement and damage.



## EXECUTIVE AND DIRECTOR COMPENSATION

### Executive Compensation Overview

The following discussion contains forward-looking statements that are based on our current plans, considerations, expectations and determinations regarding future compensation programs. The actual amount and form of compensation and the compensation policies and practices that we adopt in the future may differ materially from currently planned programs as summarized in this discussion.

The compensation provided to our named executive officers for the fiscal year ended December 31, 2018 is detailed in the Summary Compensation Table and accompanying footnotes and narrative that follow this section. Our named executive officers for the fiscal year ended December 31, 2018, which consists of our Chief Executive Officer and our two most highly compensated executive officers other than our Chief Executive Officer, are:

- Neil Kumar, Ph.D., our Chief Executive Officer;
- Brian C. Stephenson, Ph.D., CFA, our Chief Financial Officer; and
- Uma Sinha, Ph.D., our Chief Scientific Officer.

Dr. Sinha is employed by one of our consolidated subsidiaries, Eidos Therapeutics, Inc., or Eidos, as its Chief Scientific Officer. The compensation information set forth below for Dr. Sinha consists of compensation provided to Dr. Sinha through Eidos and CoA Therapeutics, Inc., or CoA, another one of our consolidated subsidiaries, for whom Dr. Sinha provides consulting services.

### Summary Compensation Table

The following table presents total compensation awarded to, earned by or paid to our named executive officers for services rendered to us in all capacities during the fiscal year ended December 31, 2018.

<u>Name &amp; Principal Position</u>	<u>Year</u>	<u>Salary (\$)</u>	<u>Bonus (\$)(1)</u>	<u>Stock Awards (\$)(2)</u>	<u>Option Awards (\$)(3)</u>	<u>Non-Equity Incentive Plan Compensation (\$)</u>	<u>All Other Compensation (\$)</u>	<u>Total (\$)</u>
Neil Kumar, Ph.D. <i>Chief Executive Officer</i>	2018	450,000						450,000
Brian C. Stephenson, Ph.D., CFA(4) <i>Chief Financial Officer</i>	2018	74,375	330,240	1,942,500				2,347,115
Uma Sinha, Ph.D. <i>Chief Scientific Officer</i>	2018	394,362(5)	159,226	896,808	540,670		759,869(6)	2,750,935

- (1) The amounts reported for Dr. Stephenson represent a \$30,240 discretionary cash bonus paid by us for the fiscal year ended December 31, 2018, based on Dr. Stephenson's performance during such fiscal year, and a one-time signing bonus equal to \$300,000 paid in connection with Dr. Stephenson's commencement of employment with us. The amount reported for Dr. Sinha represent a discretionary cash bonus paid by Eidos for the fiscal year ended December 31, 2018, based on Dr. Sinha's performance during such fiscal year.
- (2) The amounts reported represent the aggregate grant-date fair value of restricted stock awards granted by us to Dr. Stephenson and by Eidos to Dr. Sinha calculated in accordance with Financial Accounting Standards Board, Accounting Standards Codification Topic 718, or FASB ASC 718, assuming the consummation of the Reorganization. Such grant-date fair value does not take into account any estimated forfeitures related to service-vesting conditions. The assumptions used in calculating the grant-date fair value of the awards reported in this column are set forth in Note 14 to our audited financial statements included in this

prospectus. These amounts do not correspond to the actual value that may be recognized by the named executive officers upon vesting of the applicable awards or sale of the underlying shares of stock.

- (3) The amounts reported represent the aggregate grant-date fair value of stock option awards granted by Eidos and CoA, calculated in accordance with FASB ASC 718. Such grant-date fair value does not take into account any estimated forfeitures related to service-vesting conditions. The assumptions used in calculating the grant-date fair value of the awards reported in this column are set forth in Note 14 to our audited financial statements included in this prospectus. These amounts do not correspond to the actual value that may be recognized by the named executive officers upon exercise of the applicable awards or sale of the underlying shares of stock.
- (4) Dr. Stephenson commenced employment with us on October 29, 2018, and his annual base salary was pro-rated accordingly.
- (5) The amount includes \$40,385 paid for unused and accrued paid time off.
- (6) The amount represents tax gross ups relating to income recognized by Dr. Sinha in connection with Eidos' forgiveness of the purchase price for Dr. Sinha's Eidos restricted stock grants.

**Narrative to summary compensation table**

***Base salary***

During the fiscal year ended December 31, 2018, the annual base salaries for Dr. Kumar and Dr. Stephenson were \$450,000 and \$420,000, respectively. The annual base salary paid to Dr. Stephenson was pro-rated for the year based on the commencement of his employment with us on October 29, 2018. From January 1, 2018 through November 4, 2018, Dr. Sinha's annual base salary, which was paid through our subsidiary, Eidos, was \$350,000. Effective as of November 5, 2018, Dr. Sinha's annual base salary was increased to \$375,000.

***Bonus Arrangements***

During the fiscal year ended December 31, 2018, Dr. Kumar did not earn any bonuses and Drs. Stephenson and Sinha earned bonuses as set forth in the Summary Compensation Table above.

***Equity compensation***

During the fiscal year ended December 31, 2018, we granted incentive units of BridgeBio Pharma LLC to Dr. Stephenson. In connection with the Reorganization, outstanding vested and unvested incentive units of BridgeBio Pharma LLC will be exchanged for shares of common stock and restricted stock, respectively, of BridgeBio Pharma, Inc. under the 2019 Plan. The shares of restricted stock will be issued with time-based vesting conditions in accordance with the terms and conditions, including time-based vesting terms, of the incentive units from which such shares were converted. See "Reorganization". The number of shares of common stock and restricted stock of BridgeBio Pharma, Inc. to be issued to our named executive officers in connection with the Reorganization with respect to incentive units of BridgeBio Pharma LLC granted in 2018 is set forth in the table below. During the fiscal year ended December 31, 2018, Dr. Sinha was granted restricted shares of Eidos common stock, a stock option to purchase shares of Eidos' common stock as well as a stock option to purchase shares of CoA's common stock.

<u>Named Executive Officer</u>	<u>Number of Vested Incentive Units of BridgeBio Pharma LLC</u>	<u>Number of Shares of Common Stock of BridgeBio Pharma, Inc.</u>	<u>Number of Unvested Incentive Units of BridgeBio Pharma LLC</u>	<u>Number of Shares of Common Stock of BridgeBio Pharma, Inc.</u>
Brian C. Stephenson, Ph.D.	58,333	9,582	1,691,667	277,870

***Employment arrangements with our named executive officers***

We initially entered into an offer letter with each of the named executive officers in connection with his or her employment with us, which set forth the terms and conditions of the named executive officer's employment.

***Arrangements in place during the fiscal year ended December 31, 2018 for named executive officers***

*Neil Kumar, Ph.D.*

On December 14, 2017, we, through our wholly-owned subsidiary, BridgeBio Services, Inc., or Services Company, entered into an offer letter with Dr. Kumar, who currently serves as our Chief Executive Officer. The offer letter provided for Dr. Kumar's at-will employment and set forth his initial annual base salary, initial target annual bonus opportunity, and his eligibility to participate in our employee benefit plans generally. In the event of a termination of his employment by the Services Company without "cause" or Dr. Kumar's resignation from employment with the Services Company for "good reason" (as such terms are defined in the offer letter), in either case subject to Dr. Kumar's execution of an effective release of claims in favor of the Company, Dr. Kumar will be entitled to the following severance benefits: (i) a lump sum payment equal to twelve months of his then-current base salary; (ii) a pro-rated bonus based on Company and individual performance for the year of termination; and (iii) up to twelve months of COBRA reimbursements for Dr. Kumar and his dependents. Dr. Kumar is subject to the Service Company's standard proprietary information and inventions agreement.

*Brian C. Stephenson, Ph.D., CFA*

On October 28, 2018, we, through Services Company, entered into an offer letter with Dr. Stephenson, Ph.D., CFA, who currently serves as our Chief Financial Officer. The offer letter provided for Dr. Stephenson's at-will employment and set forth his initial annual base salary, initial target annual bonus opportunity, one-time sign-on bonus equal to \$300,000 (subject to repayment if Dr. Stephenson voluntarily resigns from the Company within his first year of employment), and his eligibility to participate in our employee benefit plans generally. Dr. Stephenson was also granted 1,750,000 incentive units in the Company as provided in the LLC Agreement. Dr. Stephenson is subject to the Service Company's standard proprietary information and inventions agreement.

*Uma Sinha, Ph.D.*

On June 1, 2016, through our subsidiary Eidos, we entered into an employment offer letter with Dr. Sinha, who currently serves as our Chief Scientific Officer. The offer letter provided for Dr. Sinha's at-will employment and set forth her initial annual base salary and her eligibility to participate in our employee benefit plans. In May 2018, we entered into an amendment to Dr. Sinha's offer letter to provide her with certain severance benefits. This amendment provides that, in the event of a termination of her service relationship by Eidos without "cause" (as defined in Dr. Sinha's offer letter) or Dr. Sinha's resignation from Eidos for "good reason" (as defined in Dr. Sinha's offer letter), in either case within the period commencing one month before and ending twelve months following a change in control, subject to Dr. Sinha's execution of an effective release of claims in favor of Eidos, Dr. Sinha will be entitled to the following severance benefits: (i) a lump sum payment equal to nine months of her then-base salary; (ii) an amount equal to her target bonus for the year in which her employment was terminated (pro-rated in the case of any partial year during which she was employed by Eidos) and (iii) up to nine months of COBRA reimbursements for Dr. Sinha and her dependents. In the event of a termination of her service relationship by Eidos without cause or Dr. Sinha's resignation from Eidos for good reason, in either case other than in connection with a change in control, subject to Dr. Sinha's execution of an effective release of claims in favor of Eidos, Dr. Sinha will be entitled to the following severance benefits: (i) a lump sum payment equal to six months of her then-base salary; (ii) an amount equal to her target bonus for the year in which her employment was terminated (pro-rated in the case of any partial year during which she was employed by Eidos) and (iii) up to six months of COBRA reimbursements for Dr. Sinha and her dependents. Dr. Sinha is subject to Eidos' standard proprietary information and inventions agreement. Additionally, Dr. Sinha provides services to our consolidated subsidiaries, CoA and Navire, as a consultant.

**Outstanding equity awards at fiscal year end**

The following table presents the outstanding equity awards held by each of our named executive officers as of December 31, 2018. The table assumes the completion of the Reorganization prior to the consummation of this offering and the conversion of incentive units as described above.

<u>Name</u>	<u>Vesting Commencement Date</u>	<u>Option Awards</u>				<u>Stock Awards</u>	
		<u>Number of securities underlying unexercised options (#) exercisable</u>	<u>Number of securities underlying unexercised options (#) unexercisable</u>	<u>Option exercise price (\$)</u>	<u>Option expiration date</u>	<u>Number shares or units that have not vested (#)</u>	<u>Market value of shares or units that have not vested (\$)(1)</u>
Neil Kumar, Ph.D.							
Brian C. Stephenson, Ph.D., CFA							
Uma Sinha, Ph.D.	6/1/2016					42,823(2)	589,244
	6/1/2016					24,793(2)	341,151
	9/7/2017					16,207(3)	233,008
	9/7/2017					22,118(2)	304,343
	9/7/2017					80,760(3)	1,111,257
	11/5/2018	— (4)	60,000(4)	13.20	11/5/2028		
	8/1/2017					38,425(5)	9,222
	2/1/2017	16,513(6)	19,516(6)	\$ 0.28	11/15/2027		

- (1) Assumes an initial public offering price of \$15.00 per share, which is the midpoint of the estimated price range set forth on the cover page of this prospectus, except that for Eidos stock awards, assumes the closing market price of a share of Eidos' common stock as of December 31, 2018, which was \$13.76 and for CoA stock awards, assume the fair market value of a share of CoA's common stock as of December 31, 2018, which was \$0.24.
- (2) Dr. Sinha purchased Eidos restricted stock for a price equal to the fair market value per share on the grant date, which vests as follows: 25% on the first anniversary of the vesting commencement date and the remaining 75% in equal monthly installments over the 36 month period following such anniversary, subject to continued service through each applicable vesting date.
- (3) Dr. Sinha purchased Eidos restricted stock, for a price equal to the fair market value per share on the grant date which vests as follows: 100% in equal monthly installments over the 48 month period following the vesting commencement date, subject to continued service through each applicable vesting date.
- (4) The shares underlying this Eidos stock option award vest as follows: 25% on the first anniversary of the vesting commencement date and the remaining 75% in equal monthly installments over the 36 month period following such anniversary, subject to continued service through each applicable vesting date.
- (5) Dr. Sinha early exercised her CoA stock option award and received shares of restricted stock that vest as follows: 25% on the first anniversary of the vesting commencement date and the remaining 75% in equal installments over the 36 month period following such anniversary, subject to continued service through each applicable vesting date.
- (6) The shares underlying this Navire stock option award vest as follows: 25% on the first anniversary of the vesting commencement date and the remaining 75% in equal monthly installments over the 36 month period following such anniversary, subject to continued service through each applicable vesting date.

## **Employee benefits and stock plans**

### ***2019 stock option and incentive plan***

In connection with this offering, our board of directors plans to adopt a 2019 Stock Option and Incentive Plan, or the 2019 Plan. The 2019 Stock Plan will become effective on the date immediately prior to the date on which the registration statement of which this prospectus is part is declared effective by the SEC. The 2019 Stock Plan will provide flexibility to our compensation committee to use various equity-based incentive awards as compensation tools to motivate our workforce.

We will initially reserve 11,500,000 shares of our common stock, or the Initial Limit, for the issuance of awards under the 2019 Stock Plan. The 2019 Stock Plan provides that the number of shares reserved and available for issuance under the plan will automatically increase each January 1, beginning on January 1, 2020, by 5% of the outstanding number of shares of our common stock on the immediately preceding December 31, or such lesser number of shares as determined by our compensation committee. This is referred to herein as the Annual Increase. This number will be subject to adjustment in the event of a stock split, stock dividend or other change in our capitalization.

The shares we issue under the 2019 Stock Plan will be authorized but unissued shares or shares that we reacquire. The shares of common stock underlying any awards that are forfeited, cancelled, held back upon exercise or settlement of an award to satisfy the exercise price or tax withholding, reacquired by us prior to vesting, satisfied without any issuance of stock, expire or are otherwise terminated (other than by exercise) under the 2019 Stock Plan will be added back to the shares of common stock available for issuance under the 2019 Stock Plan.

The maximum aggregate number of shares that may be issued in the form of incentive stock options may not exceed the Initial Limit cumulatively increased on January 1, 2020, and on each January 1 thereafter by the lesser of (i) the Annual Increase for such year or (ii) 11,500,000 shares of common stock.

The grant date fair value of all awards made under our 2019 Stock Plan and all other cash compensation paid by us to any non-employee director in any calendar year may not exceed \$1,250,000.

The 2019 Stock Plan will be administered by our compensation committee. Our compensation committee will have full power to select, from among the individuals eligible for awards, the individuals to whom awards will be granted, to make any combination of awards to participants, and to determine the specific terms and conditions of each award, subject to the provisions of the 2019 Stock Plan. Persons eligible to participate in the 2019 Stock Plan will be those full or part-time employees, non-employee directors and consultants of the Company and its affiliates, as selected from time to time by our compensation committee in its discretion.

The 2019 Stock Plan will permit the granting of both options to purchase common stock intended to qualify as incentive stock options under Section 422 of the Internal Revenue Code, or the Code, and options that do not so qualify. The option exercise price of each option will be determined by our compensation committee but may not be less than 100% of the fair market value of our common stock on the date of grant. The term of each option will be fixed by our compensation committee and may not exceed ten years from the date of grant. Our compensation committee will determine at what time or times each option may be exercised.

Our compensation committee will be able to award stock appreciation rights subject to such conditions and restrictions as it may determine. Stock appreciation rights will entitle the recipient to shares of common stock or cash, equal to the value of the appreciation in our stock price over the exercise price. The exercise price of each stock appreciation right may not be less than 100% of the fair market value of the common stock on the date of grant. The term of each stock appreciation right will be fixed by our compensation committee and may not exceed ten years from the date of grant. Our compensation committee will determine at what time or times each stock appreciation right may be exercised.

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Our compensation committee will be able to award restricted shares of common stock and restricted stock units to participants subject to such conditions and restrictions as it may determine. These conditions and restrictions may include the achievement of certain performance goals and/or continued employment or service relationship with us through a specified vesting period. Our compensation committee may also be permitted to grant shares of common stock that are free from any restrictions under the 2019 Stock Plan. Unrestricted stock may be granted to participants in recognition of past services or other valid consideration and may be issued in lieu of cash compensation due to such participant.

Our compensation committee will be able to grant cash bonuses under the 2019 Stock Plan to participants, subject to the achievement of certain performance goals.

The 2019 Stock Plan will provide that upon the effectiveness of a “sale event,” as defined in the 2019 Stock Plan, an acquirer or successor entity may assume, continue or substitute outstanding awards under the 2019 Stock Plan. To the extent that awards granted under our 2019 Stock Plan are not assumed or continued or substituted by the successor entity, except as may be otherwise provided in the relevant award certificate, all awards with time-based vesting, conditions or restrictions will become fully vested and nonforfeitable as of the effective time of the sale event, and all awards with conditions and restrictions relating to the attainment of performance goals may become vested and nonforfeitable in connection with a sale event in the compensation committee’s discretion or to the extent specified in the relevant award certificate. Upon the effective time of the sale event, all outstanding awards granted under the 2019 Stock Plan will terminate to the extent not assumed, continued or substituted for. In the event of such termination, individuals holding options and stock appreciation rights will be permitted to exercise such options and stock appreciation rights (to the extent exercisable) within a specified period of time prior to the sale event. In addition, in connection with the termination of the 2019 Stock Plan upon a sale event, we may make or provide for a payment, in cash or in kind, to participants holding vested and exercisable options and stock appreciation rights equal to the difference between the per share cash consideration payable to stockholders in the sale event and the exercise price of the options or stock appreciation rights and we may make or provide for a payment, in cash or in kind, to participants holding other vested awards.

Our board of directors will be able to amend or discontinue the 2019 Stock Plan and our compensation committee will be permitted to amend the exercise price of options and amend or cancel outstanding awards for purposes of satisfying changes in law or any other lawful purpose but no such action may adversely affect rights under an award without the holder’s consent. The compensation committee is specifically authorized to exercise its discretion to reduce the exercise price of outstanding stock options or stock appreciation rights or effect the repricing of cash awards through cancellation and re-grants. Certain amendments to the 2019 Stock Plan will require the approval of our stockholders.

No awards will be granted under the 2019 Stock Plan after the date that is 10 years from the date of stockholder approval. No awards under the 2019 Stock Plan will be made prior to the date of this prospectus.

### ***2019 Employee Stock Purchase Plan***

In connection with this offering, our board of directors plans to adopt a 2019 Employee Stock Purchase Plan, or an ESPP, which will become effective on the date immediately prior to the date on which the registration statement of which this prospectus is part is declared effective by the SEC. The ESPP will initially reserve and authorize the issuance of up to a total of 2,000,000 shares of common stock to participating employees. The ESPP will provide that the number of shares reserved and available for issuance will automatically increase each January 1, beginning on January 1, 2020, by the least of 1% of the outstanding number of shares of our common stock on the immediately preceding December 31, 2,000,000 shares of common stock or such lesser number of shares as determined by our compensation committee. This number will be subject to adjustment in the event of a stock split, stock dividend or other change in our capitalization.

All employees whose customary employment is for more than 20 hours per week will be eligible to participate in the ESPP. Any employee who owns 5% or more of the total combined voting power or value of all classes of stock will not be eligible to purchase shares under the ESPP.

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We will make one or more offerings, consisting of one or more purchase periods, each year to our employees to purchase shares under the ESPP. Offerings will usually begin every six months and will continue for six-month periods, referred to as offering periods. Each eligible employee may elect to participate in any offering by submitting an enrollment form at least 15 business days before the relevant offering date.

Each employee who is a participant in the ESPP may purchase shares by authorizing contributions of between 1% and 15% of his or her compensation during an offering period. Unless the participating employee has previously withdrawn from the offering, his or her accumulated contributions will be used to purchase shares on the last business day of the purchase period at a price equal to 85% of the fair market value of the shares on the first business day of the offering period or the last business day of the purchase period, whichever is lower, provided that no more than 3,500 shares of common stock (or a lesser number as established by the plan administrator in advance of the purchase period) may be purchased by any one employee during each purchase period. Under applicable tax rules, an employee may purchase no more than \$25,000 worth of shares of common stock, valued at the start of the offering period, under the ESPP for each calendar year in which a purchase right is outstanding.

The accumulated contributions of any employee who is not a participant on the last day of a purchase period will be refunded. An employee's rights under the ESPP terminate upon voluntary withdrawal from the plan or when the employee ceases employment with us for any reason.

The ESPP may be terminated or amended by our board of directors at any time, but will automatically terminate on the 10-year anniversary of this offering. An amendment that increases the number of shares of common stock that are authorized under the ESPP and certain other amendments will require the approval of our stockholders. The plan administrator may adopt subplans under the ESPP for employees of our non-U.S. subsidiaries, if any.

### ***Senior Executive Cash Incentive Bonus Plan***

In connection with this offering, our board of directors plans to adopt a Senior Executive Cash Incentive Bonus Plan, or the Bonus Plan. The Bonus Plan will provide for cash bonus payments based upon the attainment of performance targets established by our compensation committee. The payment targets will be related to financial and operational measures or objectives with respect to our company, or corporate performance goals, as well as individual performance objectives.

Our compensation committee may select corporate performance goals from among the following: achievement of specified research and development, publication, clinical and/or regulatory milestones, total shareholder return, earnings before interest, taxes, depreciation and amortization, net income (loss) (either before or after interest, taxes, depreciation and/or amortization), changes in the market price of the Company's common stock, economic value-added, funds from operations or similar measure, sales or revenue, acquisitions or strategic transactions, operating income (loss), cash flow (including, but not limited to, operating cash flow and free cash flow), return on capital, assets, equity, or investment, return on sales, gross or net profit levels, productivity, expense, margins, operating efficiency, customer satisfaction, working capital, earnings (loss) per share of the Company's common stock; bookings, new bookings or renewals; sales or market shares; number of customers, number of new customers or customer references; operating income and/or net annual recurring revenue, any of which may be measured in absolute terms, as compared to any incremental increase, in terms of growth, or as compared to results of a peer group.

Each executive officer who is selected to participate in the Bonus Plan will have a target bonus opportunity set for each performance period. The bonus formulas will be adopted in each performance period by the compensation committee and communicated to each executive. The corporate performance goals will be measured at the end of each performance period after our financial reports have been published or such other appropriate time as the compensation committee determines. If the corporate performance goals and individual performance objectives are met, payments will be made as soon as practicable following the end of each performance period, but not later than two and one-half months after the end of the fiscal year in which such

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performance period ends. Subject to the rights contained in any agreement between the executive officer and us, an executive officer must be employed by us on the bonus payment date to be eligible to receive a bonus payment. The Bonus Plan will also permits the compensation committee to approve additional bonuses to executive officers in its sole discretion.

### **401(k) plan and other benefits**

Our eligible U.S. employees participate in a tax-qualified retirement plan sponsored by Services Company that provides an opportunity to save for retirement on a tax-advantaged basis. Plan participants are able to defer eligible compensation subject to applicable annual Code limits. The plan sponsor has the ability to make discretionary contributions to the 401(k) plan, but has not done so to date. The 401(k) plan is intended to be qualified under Section 401(a) of the Code with the 401(k) plan's related trust intended to be tax exempt under Section 501(a) of the Code. As a tax-qualified retirement plan, contributions to the 401(k) plan and earnings on those contributions are not taxable to the participants until distributed from the 401(k) plan.

### **Director Compensation Overview**

Prior to this offering, we did not have a formal policy or plan to compensate our non-employee directors. Non-employee directors not affiliated with KKR generally received a director fee of \$60,000 and were reimbursed for travel, food, lodging and other expenses directly related to their activities as directors. During the fiscal year ended December 31, 2018, James Momtazee and Ali Satvat were affiliated with KKR and did not receive compensation from us for their service as directors on our board of directors. Directors who also serve as employees receive no additional compensation for their service as directors. During the fiscal year ended December 31, 2018, Dr. Kumar received no additional compensation for his service as a director. See the section titled "Executive and Director Compensation—Summary Compensation Table" for more information about Dr. Kumar's compensation for the fiscal year ended December 31, 2018.

In connection with the Reorganization, outstanding vested and unvested incentive units of BridgeBio Pharma LLC will be exchanged for shares of common stock and restricted stock, respectively, of BridgeBio Pharma, Inc. under the 2019 Stock Plan. The shares of restricted stock will be issued with time-based vesting conditions in accordance with the terms and conditions, including time-based vesting terms, of the incentive units from which such shares were converted. See "Reorganization". The number of shares of common stock and restricted stock of BridgeBio Pharma, Inc. to be issued to our directors in connection with the Reorganization with respect to common units and management incentive units, or incentive units, of BridgeBio Pharma LLC granted in 2018 is set forth in the table below:

<b>Director</b>	<b>Number of Vested Incentive Units of BridgeBio Pharma LLC</b>	<b>Number of Shares of Common Stock of BridgeBio Pharma, Inc.</b>	<b>Number of Unvested Incentive Units of BridgeBio Pharma LLC</b>	<b>Number of Shares of Common Stock of BridgeBio Pharma, Inc.</b>
Charles Homcy, M.D.	3,033,586	519,816	5,480,820	721,037
Richard Scheller, Ph.D.	381,283	65,840	687,280	90,955
Neil Kumar, LLC	20,134,171	3,455,623	36,029,195	4,742,829
James Momtazee	—	—	—	—
Ali Satvat	—	—	—	—



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The following table provides certain information concerning compensation earned by our non-employee directors during the year ended December 31, 2018.

<u>Name<sup>(1)</sup></u>	<u>Fees earned or paid in cash (\$)</u>	<u>Stock awards (\$)<sup>(2)</sup></u>	<u>All other compensation (\$)</u>	<u>Total (\$)</u>
Charles Homcy, M.D.	—	—	200,000 <sup>(3)</sup>	200,000
Richard H. Scheller, Ph.D.	60,000	40,000	—	100,000

- (1) Assuming the consummation of the Reorganization, as of December 31, 2018, Drs. Homcy and Scheller had 983,654 and 125,148 shares of common stock (of which 488,529 and 61,891 shares were restricted) of BridgeBio Pharma, Inc. outstanding, respectively, and Messrs. Momtazee and Satvat did not have any equity awards outstanding.
- (2) The amount reported represents the aggregate grant-date fair value of restricted stock awards granted by us calculated in accordance with FASB ASC 718, assuming the consummation of the Reorganization. Such grant-date fair value does not take into account any estimated forfeitures related to service-vesting conditions. The assumptions used in calculating the grant-date fair value of the awards reported in this column are set forth in Note 14 to our audited financial statements included in this prospectus. This amount does not correspond to the actual value that may be recognized by the director upon vesting of the applicable awards or the sale of the underlying shares of stock.
- (3) Pursuant to a consulting agreement by and between Dr. Homcy and the Company, dated May 1, 2017, which expired on May 15, 2018, we paid Mr. Homcy a consulting fee of \$200,000 in 2018 in exchange for consulting services provided by Dr. Homcy in the area of oncology and pipeline matters.

## CERTAIN RELATIONSHIPS AND RELATED PERSON TRANSACTIONS

In addition to the compensation arrangements, including employment, termination of employment and change in control arrangements and indemnification arrangements, discussed in the sections titled “Management” and “Executive and Director Compensation” and the registration rights described in the section titled “Description of Capital Stock—Registration Rights,” the following is a description of each transaction since January 1, 2016 and each currently proposed transaction in which:

- we have been or are to be a participant;
- the amount involved exceeded or exceeds \$120,000; and
- any of our directors, executive officers, or holders of more than 5% of our capital stock, or any immediate family member of, or person sharing the household with, any of these individuals, had or will have a direct or indirect material interest.

### Private Placements of Securities

#### Series B Preferred Unit Financing

In March 2016, BridgeBio LLC issued and sold an aggregate of 15,568,183 Series B preferred units at a price of \$0.44 per unit, for an aggregate purchase price of approximately \$6.9 million. The following table summarizes purchases of Series B preferred units of BridgeBio LLC by related persons:

<u>Purchaser</u>	<u>Series B preferred units of BridgeBio LLC</u>	<u>Total Purchase Price</u>
KKR Genetic Disorder L.P.(1)(2)	13,875,142	\$6,105,063
Neil Kumar, LLC(3)	350,284	\$ 154,125
Charles Homcy	58,381	\$ 25,688

(1) KKR Genetic Disorder L.P., beneficially owns more than 5% of our outstanding units as of December 31, 2018.

(2) James C. Momtazee and Ali J. Satvat, members of our board of directors, are officers of the general partner of KKR Genetic Disorder L.P.

(3) Neil Kumar, our chief executive officer, is the sole member of Neil Kumar, LLC.

From March 2016 through May 2017, BridgeBio Pharma LLC issued and sold an aggregate of 75,340,907 Series B preferred units at a price of \$0.44 per unit, for an aggregate purchase price of approximately \$33.1 million. The following table summarizes purchases of Series B preferred units of BridgeBio Pharma LLC by related persons:

<u>Purchaser</u>	<u>Series B preferred units of BridgeBio Pharma LLC</u>	<u>Total Purchase Price</u>
KKR Genetic Disorder L.P.(1)(2)	67,147,585	\$29,544,937
Neil Kumar, LLC(3)	1,695,170	\$ 745,875
Charles Homcy	282,528	\$ 124,312

In June 2017, BridgeBio LLC and BridgeBio Pharma LLC merged in a common control transaction. In connection with this merger transaction, each outstanding Series B preferred unit of BridgeBio LLC was exchanged for one Series B preferred unit of BridgeBio Pharma LLC.

(1) KKR Genetic Disorder L.P., beneficially owns more than 5% of our outstanding units as of December 31, 2018.

(2) James C. Momtazee and Ali J. Satvat, members of our board of directors, are officers of the general partner of KKR Genetic Disorder L.P.

(3) Neil Kumar, our chief executive officer, is the sole member of Neil Kumar, LLC.

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### **Series C Preferred Unit Financing**

In June 2017, we entered into the Series C Preferred Unit Purchase Agreement for the issuance of up to 141,155,758 Series C preferred units of BridgeBio Pharma LLC at a price of \$0.9656 per unit, for an aggregate purchase price of \$136.3 million. The following table summarizes purchases of our Series C preferred units by related persons:

<u>Unitholder</u>	<u>Series C Preferred Units</u>	<u>Total Purchase Price</u>
KKR Genetic Disorder L.P.(1)(2)	36,246,893	\$35,000,000

(1) KKR Genetic Disorder L.P. beneficially owns more than 5% of our outstanding units as of December 31, 2018.

(2) James C. Momtazee and Ali J. Satvat, members of our board of directors, are officers of the general partner of KKR Genetic Disorder L.P.

### **Series D Preferred Unit Financing**

In November 2018, we sold an aggregate of 150,955,597 Series D preferred units of BridgeBio Pharma LLC at a purchase price of \$1.9823 per unit, for an aggregate purchase price of \$299.2 million, pursuant to our Series D Preferred Unit Purchase Agreement. The following table summarizes purchases of our Series D preferred units by related persons:

<u>Unitholder</u>	<u>Series D Preferred Units</u>	<u>Total Purchase Price</u>
KKR Genetic Disorder L.P.(1)(2)	50,446,451	\$100,000,000
Viking Global Opportunities Illiquid Investments Sub-Master LP(3)	50,446,451	\$100,000,000

(1) KKR Genetic Disorder L.P. beneficially owns more than 5% of our outstanding units as of December 31, 2018.

(2) James C. Momtazee and Ali J. Satvat, members of our board of directors, are officers of the general partner of KKR Genetic Disorder L.P.

(3) Viking Global Opportunities Illiquid Investments Sub-Master LP owns more than 5% of our outstanding units as of December 31, 2018.

## **Agreements with Stockholders**

### **Registration Rights Agreement**

In connection with this offering, pursuant to the Series D Agreement, we intend to enter into a registration rights agreement with each of KKR, Viking and each other holder of 3% or greater of our outstanding equity securities.

### **Promissory Notes**

In May 2016, we issued a full recourse promissory note in the principal amount of \$124,312.50 to Dr. Homcy. The full recourse promissory note accrued interest at the rate of 1.82% per annum, with a delinquent rate of 10%. The principal amount of the note, together with all accrued but unpaid interest, is due and payable in full five years from the date of issuance or earlier upon the occurrence of certain triggering events, and was paid in full in February 2019.

In May 2017, we issued a promissory note of \$4.0 million to KKR Alternative Credit, Inc., an affiliate of KKR Genetic Disorder L.P., to facilitate the purchase of Series C preferred units by KKR Genetic Disorder L.P. The promissory note bore no interest, or redeemable common units. The promissory note was payable in cash upon the earlier of the six-month anniversary of issuance or the issuance by us of any units. On the closing of the Series C preferred unit financing, the promissory note was converted into 4,142,502 Series C preferred units at the same issuance price of \$0.9656 as the purchase price for the other Series C preferred units issued in the Series C preferred unit financing.

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### **Director Relationships**

Certain of our directors serve on our board of directors as representatives of entities which beneficially hold 5% or more of our capital stock, as indicated in the table below:

<u>Director</u>	<u>Principal Stockholder</u>
James C. Momtazee	KKR Genetic Disorder L.P.
Ali J. Satvat	KKR Genetic Disorder L.P.

### **Executive Officer and Director Compensation**

We have granted management incentive units to our executive officers and certain of our directors. See the sections titled “Executive and Director Compensation—Outstanding Equity Awards at Fiscal Year End” and “Executive and Director Compensation—Non-Employee Director Compensation” for a description of these options.

### **Consulting Arrangements**

In May 2017, BBS entered into a consulting agreement with our board member, Dr. Homcy. Under the terms of the agreement, Dr. Homcy’s duties included providing consulting services in the area of oncology and pipeline matters. Dr. Homcy received an annual retainer of \$200,000 for his services and was entitled to reimbursement for expenses incurred in performing his services. During the years ended December 31, 2017 and December 31, 2018, we incurred \$0 and \$200,000, respectively, for Dr. Homcy’s services under the consulting agreement. The consulting agreement expired on May 15, 2018.

In May 2017, BBS entered into a consulting agreement with our Chief Executive Officer, Dr. Kumar. Under the terms of the agreement, Dr. Kumar’s duties included providing consulting services in the area of oncology and pipeline matters. Dr. Kumar received an annual retainer payment of \$450,000 for his services and was entitled to reimbursement of expenses incurred in performing his services. During the years ended December 31, 2017 and December 31, 2018, we incurred \$450,000 and \$0, respectively, for Dr. Kumar’s services under the consulting agreement. The consulting agreement expired on May 15, 2018.

### **LLC Payments**

In each of April 2016 and 2017, BridgeBio Pharma LLC made a payment of \$200,000 to Dr. Homcy for advisory services.

In April 2016, BridgeBio Pharma LLC made a payment of \$450,000 to Dr. Kumar for management and oversight services.

### **Employment Agreements and Change of Control Agreements**

See the “Executive and Director Compensation—Agreements with our Executive Officers” section of this prospectus for a further discussion of these arrangements.

Other than as described above under this section titled “Certain Relationships and Related Person Transactions,” since January 1, 2016, we have not entered into any transactions, nor are there any currently proposed transactions, between us and a related party where the amount involved exceeds, or would exceed, \$120,000, and in which any related person had or will have a direct or indirect material interest.

### **Tax Sharing Agreements**

We intend to enter into a tax sharing agreement with each of our subsidiaries and its respective affiliates. The tax sharing agreement will govern the respective rights, responsibilities and obligations of each of our subsidiaries and us after this offering with respect to certain tax liabilities and benefits, tax attributes, tax contests and other matters regarding income taxes, non-income taxes and related tax returns.

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Under the tax sharing agreement, we will be responsible for any U.S. federal, state, local or other tax returns reportable on a consolidated, combined or unitary return that includes the relevant subsidiary or its affiliates (other than returns that solely include the relevant subsidiary and its affiliates). For tax periods in which the relevant subsidiary is included in such a return, the relevant subsidiary will be responsible for its portion of such income tax liability (with certain technical adjustments) as if the relevant subsidiary and its affiliates had filed a separate tax return that included only such subsidiary and its subsidiaries for that period. We expect some, but not all, of our subsidiaries to be a part of our consolidated U.S. federal return for income tax purposes after this offering. We and our subsidiaries each generally are responsible for any non-income taxes attributable to our respective businesses for all periods.

We are primarily responsible for preparing and filing, and have control with respect to contests related to, any tax return with respect to our subsidiaries' affiliated return for U.S. federal income tax purposes and with respect to any consolidated, combined or unitary return for U.S. state or local or non-U.S. income tax purposes that includes any of our subsidiaries or any of their affiliates (and us or one of our affiliates). Under the tax sharing agreement, our subsidiaries will generally be responsible for preparing and filing, and with controlling tax contests with respect to, any tax returns that include only the respective subsidiary and its subsidiaries.

Without our prior written consent, our subsidiaries will not be able take any actions (including certain issuances of capital stock) which cause a distribution by us to fail to qualify as a tax-free distribution under Section 355 of the Internal Revenue Code. We will indemnify each of our subsidiaries for any breach by us of the tax sharing agreement (including any breach of our obligation not to cause a distribution by each of our subsidiaries to fail to be a distribution within the meaning of Section 355 of the Internal Revenue Code). Each of our subsidiaries will indemnify us for any breach by it of the tax sharing agreement. If a distribution by one of our subsidiaries fails to qualify as a tax-free distribution under Section 355 of the Internal Revenue Code and neither we nor the respective subsidiary has breached the tax sharing agreement, we and the respective subsidiary will each generally be liable for 50% of any resulting tax.

### **Limitation of Liability and Indemnification of Officers and Directors**

We plan to enter into indemnification agreements with each of our directors and executive officers, the form of which is attached as an exhibit to the registration statement of which this prospectus is a part. The indemnification agreements and our amended and restated certificate of incorporation and amended and restated bylaws require us to indemnify our directors and officers to the fullest extent permitted by Delaware law.

### **Policies and Procedures for Related Person Transactions**

Our audit committee will have the primary responsibility for reviewing and approving or disapproving "related party transactions," which are transactions between us and related persons in which the aggregate amount involved exceeds or may be expected to exceed \$120,000 and in which a related person has or will have a direct or indirect material interest. The written charter of our audit committee will provide that our audit committee shall review and approve in advance any related party transaction.

Prior to the completion of this offering, we intend to adopt a formal written policy providing that we are not permitted to enter into any transaction that exceeds \$120,000 and in which any related person has a direct or indirect material interest without the consent of our audit committee. In approving or rejecting any such transaction, our audit committee is to consider the relevant facts and circumstances available and deemed relevant to our audit committee, including whether the transaction is on terms no less favorable than terms generally available to an unaffiliated third party under the same or similar circumstances and the extent of the related person's interest in the transaction.

## PRINCIPAL STOCKHOLDERS

The following table sets forth information with respect to the beneficial ownership of our common stock, as of March 31, 2019 by:

- each person, or group of affiliated persons, known by us to beneficially own more than 5% of our common stock;
- each of our directors;
- each of our named executive officers; and
- all of our executive officers and directors as a group.

The column entitled “Percentage of Shares Beneficially Owned—Before Offering” is based on a total of 99,999,967 shares of our common stock outstanding as of March 31, 2019, after giving effect to the Reorganization, including the exchange of all outstanding units of BridgeBio Pharma LLC for 99,999,967 shares of common stock of BridgeBio Pharma, Inc. in accordance with the distribution provisions of the LLC Agreement immediately prior to the completion of this offering, assuming an initial public offering price of \$15.00 per share, the midpoint of the price range set forth on the cover page of this prospectus. See “Reorganization.” The column entitled “Percentage of Shares Beneficially Owned—After Offering” is based on shares of our common stock to be outstanding after this offering, including the 15,000,000 shares of our common stock that we are selling in this offering, but not including any additional shares issuable pursuant to the underwriters’ option to purchase additional shares or any additional shares issuable upon exercise of outstanding options.

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The number of shares beneficially owned by each stockholder is determined under rules issued by the SEC and includes voting or investment power with respect to securities. Under these rules, beneficial ownership includes any shares as to which the individual or entity has sole or shared voting power or investment power. In computing the number of shares beneficially owned by an individual or entity and the percentage ownership of that person, shares of common stock subject to options, warrants, or other rights held by such person that are currently exercisable or will become exercisable within 60 days after March 31, 2019 are considered outstanding, although these shares are not considered outstanding for purposes of computing the percentage ownership of any other person. Unless otherwise indicated, the address of all listed stockholders is 421 Kipling Street, Palo Alto, California 94301. Each of the stockholders listed has sole voting and investment power with respect to the shares beneficially owned by the stockholder unless noted otherwise, subject to community property laws where applicable.

Name of Beneficial Owner	Shares Beneficially Owned	Percentage of Shares Beneficially Owned	
		Before Offering	After Offering
<b>5% Stockholders:</b>			
KKR Genetic Disorder L.P.(1)	34,359,664	34.36%	29.88%
Viking Global Opportunities Illiquid Investments Sub-Master LP.(2)	22,117,973	22.12%	19.23%
Entities affiliated with American International Group, Inc.(3)	6,808,321	6.81%	5.92%
<b>Directors and Named Executive Officers:</b>			
Neil Kumar, Ph.D.(4)	8,597,804	8.60%	7.48%
Brian C. Stephenson, Ph.D., CFA(5)	303,603	*	*
Uma Sinha, Ph.D.	—	*	*
Charles Homcy, M.D.(6)	1,307,409	1.31%	1.14%
Frank McCormick, Ph.D.(7)	1,307,409	1.31%	1.14%
Richard H. Scheller, Ph.D.(8)	156,795	*	*
Eric Aguiar, M.D.	—	*	*
James C. Momtazee(1)	34,359,664	34.36%	29.88%
Ali J. Satvat(1)	34,359,664	34.36%	29.88%
All directors and executive officers as a group (11 persons)(9)	46,214,009	46.21%	40.19%

\* Represents beneficial ownership of less than one percent of the shares of common stock.

(1) Consists of 34,359,664 shares of common stock directly owned by KKR Genetic Disorder L.P. KKR Genetic Disorder GP LLC, as the general partner of KKR Genetic Disorder L.P., KKR Management Holdings L.P., as the sole member of KKR Genetic Disorder GP LLC, KKR Management Holdings Corp., as the general partner of KKR Management Holdings L.P., KKR Group Holdings Corp., as the sole shareholder of KKR Management Holdings Corp., KKR & Co. Inc., as the sole shareholder of KKR Group Holdings Corp., KKR Management LLC, as the Class B common stockholder of KKR & Co. Inc., and Messrs. Henry R. Kravis and George R. Roberts, as the designated members of KKR Management LLC, may be deemed to be the beneficial owners having shared voting and investment power with respect to the shares described in this footnote. The principal business address of each of the entities and persons identified in the immediately preceding sentence, except Mr. Roberts, is c/o Kohlberg Kravis Roberts & Co. L.P., 9 West 57th Street, Suite 4200, New York, NY 10019. The principal business address for Mr. Roberts is c/o Kohlberg Kravis Roberts & Co. L.P., 2800 Sand Hill Road, Suite 200, Menlo Park, CA 94025. Each of Messrs. Momtazee and Satvat is a member of our Board of Directors and serves as an executive of Kohlberg Kravis Roberts & Co. L.P. and/or one or more of its affiliates. Each of Messrs. Kravis, Roberts, Momtazee and Satvat disclaims beneficial ownership of the shares held by KKR Genetic Disorder L.P. The principal business address of each of Messrs. Momtazee and Satvat is c/o Kohlberg Kravis Roberts & Co. L.P., 2800 Sand Hill Road, Suite 200, Menlo Park, CA 94025.

(2) Consists of 22,117,973 shares of common stock directly owned by Viking Global Opportunities Illiquid Investments Sub-Master LP (“Opportunities Fund”). Opportunities Fund has the authority to dispose of and vote the shares directly owned by it, which power may be exercised by its general partner, Viking Global Opportunities Portfolio GP LLC (“Opportunities GP”), and by Viking Global Investors LP (“VGI”), which provides managerial services to Opportunities Fund. O. Andreas Halvorsen, David C. Ott and Rose Shabet, as Executive Committee members of Viking Global Partners LLC (the general partner of VGI) and Opportunities GP, have shared authority to direct the voting and disposition of investments beneficially owned by the Opportunities Fund and Opportunities GP. The business address of each of the entities is c/o Viking Global Investors LP, 55 Railroad Avenue, Greenwich, Connecticut 06830.

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- (3) Consists of (a) 4,769,454 shares of common stock owned directly by The United States Life Insurance Company in the City of New York ("USLIC"); and (b) 2,038,867 shares of common stock owned directly by American General Life Insurance Company ("AGLIC"). USLIC and AGLIC are each indirect, wholly owned subsidiaries of American International Group, Inc.
- (4) Consists of (a) 7,293,559 shares of common stock held by Dr. Kumar, of which 4,531,540 shares are subject to our right of repurchase as of March 31, 2019; (b) 1,228,325 shares of common stock held by Neil Kumar LLC; and (c) 904,853 shares of common stock held by Kumar Haldea Family Irrevocable Trust.
- (5) Consists of 303,603 shares of common stock held by Dr. Stephenson, of which 279,380 shares are subject to our right of repurchase as of March 31, 2019.
- (6) Consists of 1,307,409 shares of common stock held by Dr. Homcy, of which 690,210 shares are subject to our right of repurchase as of March 31, 2019.
- (7) Consists of 1,307,409 shares of common stock held by Dr. McCormick, of which 690,210 shares are subject to our right of repurchase as of March 31, 2019.
- (8) Consists of 156,795 shares of common stock held by Dr. Scheller, of which 86,455 shares are subject to our right of repurchase as of March 31, 2019.
- (9) Consists of the number of shares beneficially owned by the named executive officers and directors listed in the table above, as well as (i) 169,006 shares of common stock held by Michael Henderson, of which 148,122 shares are subject to our right of repurchase as of March 31, 2019; and (ii) 12,319 shares of common stock held by Cameron Turtle, of which 10,197 shares are subject to our right of repurchase as of March 31, 2019.



## PRICING SENSITIVITY ANALYSIS

Throughout this prospectus we provide information assuming that the initial public offering price per share of common stock is \$15.00, which is the midpoint of the estimated price range set forth on the cover of this prospectus. However, some of the information that we provide will be affected if the initial public offering price per share of common stock in this offering is different from the midpoint of the estimated price range set forth on the cover of this prospectus. The following table presents how some of the information set forth in this prospectus would be affected by an initial public offering price per share of common stock at the low-, mid- and high-points of the estimated price range set forth on the cover of this prospectus, assuming that the underwriters' option to purchase additional shares of our common stock is not exercised. See "Reorganization" for additional information.

	Price per share		
	\$14.00	\$15.00	\$16.00
(in thousands, except percentages, share and per share data)			
<b>Shares issued in Reorganization</b>			
Common stock issuable for units outstanding immediately prior to the Reorganization:			
Series D Preferred Units	30,533,399	30,505,452	30,481,000
Series C Preferred Units	32,814,160	32,503,831	32,232,297
Series B Preferred Units	17,721,375	17,748,982	17,773,143
Series A Preferred Units	4,906,991	4,911,479	4,915,411
Founder Units	2,247,475	2,249,535	2,251,334
Vested Common Units	1,482,506	1,483,861	1,485,041
Vested Management Incentive Units	3,520,309	3,577,518	3,627,577
Total	<u>93,226,215</u>	<u>92,980,658</u>	<u>92,765,803</u>
Restricted common stock issuable for units outstanding immediately prior to the Reorganization:			
Unvested Common Units	307,981	308,270	308,518
Unvested Management Incentive Units	6,465,768	6,711,039	6,925,650
Total	<u>6,773,749</u>	<u>7,019,309</u>	<u>7,234,168</u>
Total shares issued in Reorganization	<u>99,999,964</u>	<u>99,999,967</u>	<u>99,999,971</u>
<b>Net proceeds</b>			
Net proceeds from this offering (approximate)	\$ <u>189,747</u>	\$ <u>203,697</u>	\$ <u>217,647</u>
<b>Pro forma as adjusted capitalization</b>			
Cash and cash equivalents	\$ <u>563,711</u>	\$ <u>577,661</u>	\$ <u>591,611</u>
Total long-term debt obligations	\$ <u>54,861</u>	\$ <u>54,861</u>	\$ <u>54,861</u>
Stockholders' equity (deficit):			
Undesignated preferred stock, \$0.001 par value per share	—	—	—
Common stock, \$0.001 par value per share	115	115	115
Additional paid-in capital	676,506	690,456	704,406
Accumulated deficit	(234,733)	(234,733)	(234,733)
Noncontrolling interests	58,318	58,318	58,318
Total stockholders' equity (deficit)	<u>500,206</u>	<u>514,156</u>	<u>528,106</u>
Total capitalization	<u>\$ 500,408</u>	<u>\$ 514,358</u>	<u>\$ 528,308</u>
<b>Dilution</b>			
Pro forma as adjusted net tangible book deficit per share after giving effect to this offering	\$ <u>4.35</u>	\$ <u>4.47</u>	\$ <u>4.59</u>
Dilution per share to new investors in this offering	\$ <u>9.65</u>	\$ <u>10.53</u>	\$ <u>11.41</u>
<b>Equity ownership percentages following this offering</b>			
Existing owners in this offering	87.0%	87.0%	87.0%
New investors in this offering	13.0%	13.0%	13.0%
	<u>100%</u>	<u>100%</u>	<u>100%</u>

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In addition, throughout this prospectus we provide information assuming that the underwriters' option to purchase additional shares of common stock from us is not exercised. However, some of the information that we provide will be affected if the underwriters' option to purchase additional shares of common stock is exercised. The following table presents how some of the information set forth in this prospectus would be affected if the underwriters exercise in full their option to purchase additional shares of common stock where the initial public offering price per share of common stock is at the low-, mid- and high-points of the estimated price range set forth on the cover of this prospectus.

	Price per share		
	\$14.00	\$15.00	\$16.00
(in thousands, except percentages, share and per share data)			
<b>Shares issued in Reorganization</b>			
Common stock issuable for units outstanding immediately prior to the Reorganization:			
Series D Preferred Units	30,533,399	30,505,452	30,481,000
Series C Preferred Units	32,814,160	32,503,831	32,232,297
Series B Preferred Units	17,721,375	17,748,982	17,773,143
Series A Preferred Units	4,906,991	4,911,479	4,915,411
Founder Units	2,247,475	2,249,535	2,251,334
Vested Common Units	1,482,506	1,483,861	1,485,041
Vested Management Incentive Units	3,520,309	3,577,518	3,627,577
Total	93,226,215	92,980,658	92,765,803
Restricted common stock issuable for units outstanding immediately prior to the Reorganization:			
Unvested Common Units	307,981	308,270	308,518
Unvested Management Incentive Units	6,465,768	6,711,039	6,925,650
Total	6,773,749	7,019,309	7,234,168
Total shares issued in Reorganization	99,999,964	99,999,967	99,999,971
<b>Net proceeds</b>			
Net proceeds from this offering (approximate)	\$ 219,041	\$ 235,084	\$ 251,127
<b>Pro forma as adjusted capitalization</b>			
Cash and cash equivalents	\$ 593,006	\$ 609,049	\$ 625,092
Total long-term debt obligations	\$ 54,861	\$ 54,861	\$ 54,861
Stockholders' equity (deficit):			
Undesignated preferred stock, \$0.001 par value per share	—	—	—
Common stock, \$0.001 par value per share	117	117	117
Additional paid-in capital	705,800	721,843	737,886
Accumulated deficit	(234,733)	(234,733)	(234,733)
Noncontrolling interests	58,318	58,318	58,318
Total stockholders' equity (deficit)	529,502	545,545	561,588
Total capitalization	\$ 529,704	\$ 545,747	\$ 561,790
<b>Dilution</b>			
Pro forma as adjusted net tangible book deficit per share after giving effect to this offering	\$ 4.52	\$ 4.65	\$ 4.79
Dilution per share to new investors in this offering	\$ 9.48	\$ 10.35	\$ 11.21
<b>Equity ownership percentages following this offering</b>			
Existing owners in this offering	85.3%	85.3%	85.3%
New investors in this offering	14.7%	14.7%	14.7%
	100%	100%	100%

## DESCRIPTION OF CAPITAL STOCK

*The following description of our capital stock and provisions of our amended and restated certificate of incorporation and amended and restated bylaws as they will be in effect immediately prior to the completion of this offering are summaries and are qualified in their entirety by reference to our amended and restated certificate of incorporation and amended and restated bylaws that will be in effect immediately prior to the completion of this offering, the forms of which are filed as exhibits to the registration statement of which this prospectus forms a part. The description of our common stock reflects the completion of the Reorganization, which will occur immediately prior to the completion of this offering. See “Reorganization” for more information concerning the Reorganization.*

### **Common Stock**

The holders of our common stock are entitled to one vote for each share held on all matters submitted to a vote of the stockholders. The holders of our common stock do not have any cumulative voting rights. Holders of our common stock are entitled to receive ratably any dividends declared by our board of directors out of funds legally available for that purpose, subject to any preferential dividend rights of any outstanding preferred stock. Our common stock has no preemptive rights, conversion rights or other subscription rights or redemption or sinking fund provisions.

In the event of our liquidation, dissolution or winding up, holders of our common stock will be entitled to share ratably in all assets remaining after payment of all debts and other liabilities and any liquidation preference of any outstanding preferred stock. The shares to be issued by us in this offering will be, when issued and paid for, validly issued, fully paid and non-assessable.

### **Preferred Stock**

Upon the consummation of this offering, our board of directors will have the authority, without further action by our stockholders, to issue up to 25,000,000 shares of convertible preferred stock in one or more series and to fix the rights, preferences, privileges and restrictions thereof. These rights, preferences and privileges could include dividend rights, conversion rights, voting rights, terms of redemption, liquidation preferences, sinking fund terms and the number of shares constituting, or the designation of, such series, any or all of which may be greater than the rights of common stock. The issuance of our convertible preferred stock could adversely affect the voting power of holders of common stock and the likelihood that such holders will receive dividend payments and payments upon our liquidation. In addition, the issuance of convertible preferred stock could have the effect of delaying, deferring or preventing a change in control of our company or other corporate action. Immediately after consummation of this offering, no shares of convertible preferred stock will be outstanding, and we have no present plan to issue any shares of convertible preferred stock.

### **Registration Rights**

Upon the completion of this offering, the holders of 99,999,967 shares of our common stock are entitled to rights with respect to the registration of these securities under the Securities Act. These rights are provided under the terms of the registration rights agreement. The registration rights agreement includes demand registration rights, short-form registration rights and piggyback registration rights. All fees, costs and expenses incurred in connection with registrations under the registration rights agreement will be borne by us, and all selling expenses, including underwriting discounts and selling commissions, will be borne by the holders of the shares being registered.

### ***Demand Registration Rights***

180 days after the effective date of the registration statement for this offering, the holders of our registrable securities are entitled to demand registration rights. Under the terms of our registration rights agreement, we will

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be required, upon the request of a holder of at least 10% of our outstanding registrable securities, to file a registration statement and use reasonable best efforts to effect the registration for public resale of these shares and any additional registrable securities requested to be included in such registration by any other holders of our registrable securities.

### ***Short-form Registration Rights***

Upon the completion of this offering, the holders of our registrable securities are also entitled to short-form registration rights. Pursuant to our registration rights agreement, if we are eligible to file a registration statement on Form S-3, upon the request of a holder of at least 10% of our outstanding registrable securities, we will be required to use our reasonable best efforts to effect a registration of such shares. We are required to effect up to two registrations in any six-month period pursuant to this provision of the registration rights agreement.

### ***Piggyback Registration Rights***

The holders of our registrable securities are entitled to piggyback registration rights. If we register any of our securities either for our own account or for the account of other security holders, the holders of our outstanding registrable securities are entitled to include their shares in the registration. Subject to certain exceptions contained in the registration rights agreement, we and the underwriters may limit the number of shares included in the underwritten offering if the underwriters determine that marketing factors require a limitation of the number of shares to be underwritten.

### ***Indemnification***

Our registration rights agreement contains customary cross-indemnification provisions, under which we are obligated to indemnify holders of registrable securities in the event of material misstatements or omissions in the registration statement attributable to us, and they are obligated to indemnify us for material misstatements or omissions attributable to them.

### ***Expenses of Registration***

We will pay the registration expenses, subject to certain limited exceptions contained in the registration rights agreement, of the holders of the shares registered pursuant to the demand, short-form and piggyback registration rights described above, including the expenses of one counsel for the selling holders.

### ***Expiration of Registration Rights***

The registration rights granted under the registration rights agreement will terminate upon the earliest to occur of: (i) such time after our initial public offering when all registrable securities could be sold under Rule 144 of the Securities Act or a similar exemption without limitation during a three-month period without registration; (ii) the consummation of a transaction or series of transactions in which a person, or a group of persons, acquires from our stockholders, shares representing more than 50% of our outstanding voting stock; and (iii) the consummation of a transaction or series of transactions in which a person, or group of persons, acquires the right to receive the majority of the proceeds in a final liquidation, dissolution or termination, voluntary or involuntary, of the company.

### ***Authorized but Unissued Capital Stock***

The Delaware General Corporation Law does not require stockholder approval for any issuance of authorized shares. However, the listing requirements of Nasdaq, which would apply so long as our common stock remains listed on Nasdaq, require stockholder approval of certain issuances equal to or exceeding 20% of the then outstanding voting power or then outstanding number of shares of common stock. These additional shares may be used for a variety of corporate purposes, including future public offerings, to raise additional capital or to facilitate acquisitions.

One of the effects of the existence of unissued and unreserved common stock or preferred stock may be to enable our board of directors to issue shares to persons friendly to current management, which issuance could render more difficult or discourage an attempt to obtain control of our company by means of a merger, tender offer, proxy contest or otherwise, and thereby protect the continuity of our management and possibly deprive our stockholders of opportunities to sell their shares of common stock at prices higher than prevailing market prices.

### **Anti-Takeover Effects of Provisions of Delaware Law and Our Amended and Restated Certificate of Incorporation and Amended and Restated Bylaws**

Certain provisions of the Delaware General Corporation Law and of our amended and restated certificate of incorporation and amended and restated bylaws that will become effective upon the completion of this offering could have the effect of delaying, deferring or discouraging another party from acquiring control of us. These provisions, which are summarized below, are expected to discourage certain types of coercive takeover practices and inadequate takeover bids and, as a consequence, they might also inhibit temporary fluctuations in the market price of our common stock that often result from actual or rumored hostile takeover attempts. These provisions are also designed in part to encourage anyone seeking to acquire control of us to first negotiate with our board of directors. These provisions might also have the effect of preventing changes in our management. It is possible that these provisions could make it more difficult to accomplish transactions that stockholders might otherwise deem to be in their best interests. However, we believe that the advantages gained by protecting our ability to negotiate with any unsolicited and potentially unfriendly acquirer outweigh the disadvantages of discouraging such proposals, including those priced above the then-current market value of our common stock, because, among other reasons, the negotiation of such proposals could improve their terms.

#### ***Delaware Takeover Statute***

Upon completion of this offering, we will be subject to the provisions of Section 203 of the Delaware General Corporation Law. In general, Section 203 prohibits a publicly held Delaware corporation from engaging in a “business combination” with an “interested stockholder” for a three-year period following the time that this stockholder becomes an interested stockholder, unless the business combination is approved in a prescribed manner. Under Section 203, a business combination between a corporation and an interested stockholder is prohibited unless it satisfies one of the following conditions:

- before the stockholder became interested, our board of directors approved either the business combination or the transaction which resulted in the stockholder becoming an interested stockholder;
- upon consummation of the transaction which resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction commenced, excluding for purposes of determining the voting stock outstanding, shares owned by persons who are directors and also officers, and employee stock plans, in some instances, but not the outstanding voting stock owned by the interested stockholder; or
- at or after the time the stockholder became interested, the business combination was approved by our board of directors and authorized at an annual or special meeting of the stockholders by the affirmative vote of at least two-thirds of the outstanding voting stock which is not owned by the interested stockholder.

Section 203 defines a business combination to include:

- any merger or consolidation involving the corporation and the interested stockholder;
- any sale, transfer, lease, pledge, exchange, mortgage or other disposition involving the interested stockholder of 10% or more of the assets of the corporation;
- subject to exceptions, any transaction that results in the issuance or transfer by the corporation of any stock of the corporation to the interested stockholder;

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- subject to exceptions, any transaction involving the corporation that has the effect of increasing the proportionate share of the stock of any class or series of the corporation beneficially owned by the interested stockholder; or
- the receipt by the interested stockholder of the benefit of any loans, advances, guarantees, pledges or other financial benefits provided by or through the corporation.

In general, Section 203 defines an interested stockholder as any entity or person beneficially owning 15% or more of the outstanding voting stock of the corporation and any entity or person affiliated with or controlling or controlled by the entity or person.

### ***Provisions of Our Amended and Restated Certificate of Incorporation and Amended and Restated Bylaws***

Our amended and restated certificate of incorporation and amended and restated bylaws to be in effect upon completion of this offering will include a number of provisions that may have the effect of delaying, deferring or discouraging another party from acquiring control of us and encouraging persons considering unsolicited tender offers or other unilateral takeover proposals to negotiate with our board of directors rather than pursue non- negotiated takeover attempts. These provisions include the items described below.

*Board composition and filling vacancies.* Our amended and restated certificate of incorporation provides that directors may be removed only for cause by the affirmative vote of the holders of at least 75% of the shares then entitled to vote at an annual election of directors. Furthermore, any vacancy on our board of directors, however occurring, including a vacancy resulting from an increase in the size of our board, may be filled by the affirmative vote of a majority of our directors then in office even if less than a quorum.

*No written consent of stockholders.* Our amended and restated certificate of incorporation provides that all stockholder actions are required to be taken by a vote of the stockholders at an annual or special meeting, and that stockholders may not take any action by written consent in lieu of a meeting. This limit may lengthen the amount of time required to take stockholder actions and would prevent the amendment of our bylaws or removal of directors by our stockholder without holding a meeting of stockholders.

*Meetings of stockholders.* Our bylaws provide that only a majority of the members of our board of directors then in office may call special meetings of stockholders and only those matters set forth in the notice of the special meeting may be considered or acted upon at a special meeting of stockholders. Our bylaws limit the business that may be conducted at an annual meeting of stockholders to those matters properly brought before the meeting.

*Advance notice requirements.* Our bylaws establish advance notice procedures with regard to stockholder proposals relating to the nomination of candidates for election as directors or new business to be brought before meetings of our stockholders. These procedures provide that notice of stockholder proposals must be timely given in writing to our corporate secretary prior to the meeting at which the action is to be taken. Generally, to be timely, notice must be received at our principal executive offices not less than 90 days or more than 120 days prior to the first anniversary date of the annual meeting for the preceding year. The notice must contain certain information specified in our bylaws.

*Amendment to certificate of incorporation and bylaws.* As required by the Delaware General Corporation Law, any amendment of our amended and restated certificate of incorporation must first be approved by a majority of our board of directors, and if required by law or our amended and restated certificate of incorporation, must thereafter be approved by a majority of the outstanding shares entitled to vote on the amendment, and a majority of the outstanding shares of each class entitled to vote thereon as a class, except that the amendment of the provisions relating to stockholder action, directors, limitation of liability and the amendment of our amended and restated certificate of incorporation must be approved by not less than 75% of the outstanding shares entitled to vote on the amendment, and not less than 75% of the outstanding shares of each class entitled to vote thereon as a

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class. Our bylaws may be amended by the affirmative vote of a majority vote of the directors then in office, subject to any limitations set forth in the bylaws; and may also be amended by the affirmative vote of at least 75% of the outstanding shares entitled to vote on the amendment, or, if the board of directors recommends that the stockholders approve the amendment, by the affirmative vote of the majority of the outstanding shares entitled to vote on the amendment, in each case voting together as a single class.

*Undesignated preferred stock.* Our amended and restated certificate of incorporation provides for authorized shares of preferred stock. The existence of authorized but unissued shares of preferred stock may enable our board of directors to render more difficult or to discourage an attempt to obtain control of us by means of a merger, tender offer, proxy contest or otherwise. For example, if in the due exercise of its fiduciary obligations, our board of directors were to determine that a takeover proposal is not in the best interests of us or our stockholders, our board of directors could cause shares of preferred stock to be issued without stockholder approval in one or more private offerings or other transactions that might dilute the voting or other rights of the proposed acquirer or insurgent stockholder or stockholder group. In this regard, our amended and restated certificate of incorporation grants our board of directors' broad power to establish the rights and preferences of authorized and unissued shares of preferred stock. The issuance of shares of preferred stock could decrease the amount of earnings and assets available for distribution to holders of shares of common stock. The issuance may also adversely affect the rights and powers, including voting rights, of these holders and may have the effect of delaying, deterring or preventing a change in control of us.

### **Choice of Forum**

Our amended and restated bylaws that will become effective upon the closing of this offering will provide that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for any state law claim for: (1) any derivative action or proceeding brought on our behalf; (2) any action asserting a claim of breach of a fiduciary duty or other wrongdoing by any of our directors, officers, employees or agents to us or our stockholders; (3) any action asserting a claim against us arising pursuant to any provision of the Delaware General Corporation Law or our certificate of incorporation or bylaws; (4) any action to interpret, apply, enforce or determine the validity of our certificate of incorporation or bylaws or (5) any action asserting a claim governed by the internal affairs doctrine. The choice of forum provision does not apply to any actions arising under the Securities Act or the Exchange Act.

### **Limitations of Liability and Indemnification**

See "Executive and Director Compensation—Limitation on Liability and Indemnification Matters."

### **Market Listing**

We have applied to list our common stock on the Nasdaq Global Market under the symbol "BBIO."

### **Transfer Agent and Registrar**

Upon the completion of this offering, the transfer agent and registrar for our common stock will be American Stock Transfer & Trust Company, LLC.

## MATERIAL U.S. FEDERAL INCOME TAX CONSIDERATIONS FOR NON-U.S. HOLDERS OF COMMON STOCK

The following discussion is a summary of the material U.S. federal income tax considerations applicable to non-U.S. holders (as defined below) with respect to their ownership and disposition of shares of our common stock issued pursuant to this offering. For purposes of this discussion, a non-U.S. holder means a beneficial owner of our common stock that is for U.S. federal income tax purposes:

- a non-resident alien individual;
- a foreign corporation or any other foreign organization taxable as a corporation for U.S. federal income tax purposes; or
- a foreign estate or trust, the income of which is not subject to U.S. federal income tax on a net income basis.

This discussion does not address the tax treatment of partnerships or other entities that are pass-through entities for U.S. federal income tax purposes or persons that hold their common stock through partnerships or other pass-through entities. A partner in a partnership or other pass-through entity that will hold our common stock should consult his, her or its own tax advisor regarding the tax consequences of acquiring, holding and disposing of our common stock through a partnership or other pass-through entity, as applicable.

This discussion is based on current provisions of the Code, U.S. Treasury Regulations promulgated thereunder, current administrative rulings and judicial decisions, all as in effect as of the date of this prospectus and, all of which are subject to change or to differing interpretation, possibly with retroactive effect. Any such change or differing interpretation could alter the tax consequences to non-U.S. holders described in this prospectus. There can be no assurance that the Internal Revenue Service, which we refer to as the IRS, will not challenge one or more of the tax consequences described herein and we have not obtained, nor do we intend to obtain, a ruling from the IRS with respect to the U.S. federal income tax consequences to a non-U.S. holder of the ownership, or disposition, of our common stock. We assume in this discussion that a non-U.S. holder holds shares of our common stock as a capital asset, which generally consists of property held for investment.

This discussion does not address all aspects of U.S. federal income that may be relevant to a particular non-U.S. holder in light of that non-U.S. holder's individual circumstances nor does it address any aspects of any U.S. federal tax other than the income tax, U.S. state, local or non-U.S. taxes, the alternative minimum tax, any tax considerations resulting from a non-U.S. holder having a functional currency other than the U.S. dollar, or the Medicare tax on net investment income. This discussion also does not consider any specific facts or circumstances that may apply to a non-U.S. holder and does not address the special tax rules applicable to particular non-U.S. holders, such as:

- insurance companies;
- tax-exempt or governmental organizations;
- financial institutions;
- brokers or dealers in securities;
- regulated investment companies;
- pension plans;
- "controlled foreign corporations," "passive foreign investment companies," and corporations that accumulate earnings to avoid U.S. federal income tax;
- "qualified foreign pension funds," or entities wholly owned by a "qualified foreign pension fund";
- persons deemed to sell our common stock under the constructive sale provisions of the Code;



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- persons that hold our common stock as part of a straddle, hedge, conversion transaction, synthetic security or other integrated investment;
- persons who hold or receive our common stock pursuant to the exercise of any employee stock option or otherwise as compensation; and
- certain U.S. expatriates.

This discussion is for general information only and is not tax advice. Accordingly, all prospective non-U.S. holders of our common stock should consult their own tax advisors with respect to the U.S. federal, state, local and non-U.S. tax consequences of the purchase, ownership and disposition of our common stock in each non-U.S. holder's individual circumstances.

### **Distributions on Our Common Stock**

Distributions, if any, on our common stock generally will constitute dividends for U.S. federal income tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles. If a distribution exceeds our current and accumulated earnings and profits, the excess will be treated as a tax-free return of capital, up to the non-U.S. holder's tax basis in the common stock. Any distributions in excess of the holder's tax basis will be treated as capital gain, subject to the tax treatment described below in "Gain on Sale or Other Taxable Disposition of Our Common Stock."

Subject to the discussion in the following two paragraphs in this section and the discussions below under "Backup Withholding and Information Reporting" and "Withholding and Information Reporting Requirements—FATCA", dividends paid to a non-U.S. holder generally will be subject to withholding of U.S. federal income tax at a 30% rate or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence.

Dividends that are treated as effectively connected with a trade or business conducted by a non-U.S. holder within the United States and, if an applicable income tax treaty so provides, that are attributable to a permanent establishment or a fixed base maintained by the non-U.S. holder within the United States, are generally exempt from the 30% withholding tax described above if the non-U.S. holder satisfies applicable certification and disclosure requirements. However, such U.S. effectively connected income, net of specified deductions and credits, is taxed at the same graduated U.S. federal income tax rates applicable to United States persons (as defined in the Code). Any U.S. effectively connected income received by a non-U.S. holder that is a corporation may also, under certain circumstances, be subject to an additional "branch profits tax" at a 30% rate or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence.

A non-U.S. holder of our common stock who claims the benefit of an applicable income tax treaty between the United States and such holder's country of residence generally will be required to provide a properly executed IRS Form W-8BEN or W-8BEN-E (or successor form) and satisfy applicable certification and other requirements. Non-U.S. holders are urged to consult their tax advisors regarding their entitlement to benefits under a relevant income tax treaty. A non-U.S. holder that is eligible for a reduced rate of U.S. withholding tax under an income tax treaty may obtain a refund or credit of any excess amounts withheld by timely filing a U.S. tax return with the IRS.

### **Gain on Sale or Other Taxable Disposition of Our Common Stock**

Subject to the discussion below under "Backup Withholding and Information Reporting" and "Withholding and Information Reporting Requirements—FATCA," a non-U.S. holder generally will not be subject to any U.S.

federal income or withholding tax on any gain realized upon such holder's sale or other taxable disposition of shares of our common stock unless:

- the gain is effectively connected with the non-U.S. holder's conduct of a U.S. trade or business and, if an applicable income tax treaty so provides, is attributable to a permanent establishment or a fixed-base maintained by such non-U.S. holder in the United States, in which case the non-U.S. holder generally will be taxed on a net income basis at the graduated U.S. federal income tax rates applicable to United States persons (as defined in the Code) and, if the non-U.S. holder is a corporation, the branch profits tax described above in "Distributions on Our Common Stock" also may apply;
- the non-U.S. holder is a nonresident alien individual who is present in the United States for 183 days or more in the taxable year of the disposition and certain other conditions are met, in which case the non-U.S. holder will be subject to a 30% tax (or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence) on the gain derived from the disposition, which may be offset by certain U.S. source capital losses of the non-U.S. holder, if any (even though the individual is not considered a resident of the United States), provided that the non-U.S. holder has timely filed U.S. federal income tax returns with respect to such losses; or
- we are, or have been, at any time during the five-year period preceding such sale or other taxable disposition (or the non-U.S. holder's holding period, if shorter) a "U.S. real property holding corporation," unless our common stock is "regularly traded" (as defined by applicable U.S. Treasury regulations) on an established securities market and the non-U.S. holder holds no more than 5% of our outstanding common stock, actually or constructively, during the shorter of the 5-year period ending on the date of the disposition or the period that the non-U.S. holder held our common stock. If we are determined to be a U.S. real property holding corporation and the foregoing exception does not apply, then a purchaser may be required to withhold 15% of the proceeds payable to a non-U.S. holder from a sale of our common stock and the non-U.S. holder generally will be taxed on its gain derived from the disposition at the graduated U.S. federal income tax rates applicable to United States persons (as defined in the Code). Generally, a corporation is a U.S. real property holding corporation if the fair market value of its U.S. real property interests equals or exceeds 50% of the sum of the fair market value of its worldwide real property interests plus its other assets used or held for use in a trade or business. Although there can be no assurance, we do not believe that we are, or have been, a U.S. real property holding corporation, and we do not anticipate becoming one in the future. No assurance can be provided that our common stock will be regularly traded on an established securities market for purposes of the rules described above.

### **Backup Withholding and Information Reporting**

We must report annually to the IRS and to each non-U.S. holder the gross amount of the distributions on our common stock paid to such holder and the tax withheld, if any, with respect to such distributions. Non-U.S. holders may have to comply with specific certification procedures to establish that the holder is not a United States person (as defined in the Code) in order to avoid backup withholding at the applicable rate, currently 24%, with respect to dividends on our common stock, generally by providing an applicable IRS Form W-8. Dividends paid to non-U.S. holders subject to withholding of U.S. federal income tax, as described above in "Distributions on Our Common Stock," generally will be exempt from U.S. backup withholding.

Information reporting and backup withholding will generally apply to the proceeds of a disposition of our common stock by a non-U.S. holder effected by or through the U.S. office of any broker, U.S. or foreign, unless the holder certifies its status as a non-U.S. holder and satisfies certain other requirements, or otherwise establishes an exemption. Generally, information reporting and backup withholding will not apply to a payment of disposition proceeds to a non-U.S. holder where the transaction is effected outside the United States through a non-U.S. office of a broker. However, for information reporting purposes, dispositions effected through a non-U.S. office of a broker with substantial U.S. ownership or operations generally will be treated in a manner similar to dispositions effected through a U.S. office of a broker. Non-U.S. holders should consult their own tax

advisors regarding the application of the information reporting and backup withholding rules to them. Copies of information returns may be made available to the tax authorities of the country in which the non-U.S. holder resides or is incorporated under the provisions of a specific treaty or agreement. Backup withholding is not an additional tax. Any amounts withheld under the backup withholding rules from a payment to a non-U.S. holder can be refunded or credited against the non-U.S. holder's U.S. federal income tax liability, if any, provided that an appropriate claim is filed with the IRS in a timely manner.

#### **Withholding and Information Reporting Requirements—FATCA**

The Foreign Account Tax Compliance Act, or FATCA, generally imposes a U.S. federal withholding tax at a rate of 30% on payments of dividends on, or (subject to the proposed U.S. Treasury regulations discussed below) gross proceeds from the sale or other disposition of, our common stock paid to a foreign entity unless (i) if the foreign entity is a "foreign financial institution," such foreign entity undertakes certain due diligence, reporting, withholding, and certification obligations, (ii) if the foreign entity is not a "foreign financial institution," such foreign entity identifies certain of its U.S. investors, if any, or (iii) the foreign entity is otherwise exempt under FATCA. Under applicable U.S. Treasury regulations, withholding under FATCA currently applies to payments of dividends on our common stock. While withholding under FATCA would have applied also to payments of gross proceeds from the sale or other disposition of stock on or after January 1, 2019, recently proposed U.S. Treasury regulations eliminate FATCA withholding on payments of gross proceeds entirely. Taxpayers generally may rely on these proposed U.S. Treasury regulations until final U.S. Treasury regulations are issued. Under certain circumstances, a non-U.S. holder may be eligible for refunds or credits of this withholding tax. An intergovernmental agreement between the United States and an applicable foreign country may modify the requirements described in this paragraph. Non-U.S. holders should consult their tax advisors regarding the possible application of withholding under FATCA to their investment in our common stock and the entities through which they hold our common stock, including, without limitation, the process and deadlines for meeting the applicable requirements to prevent the imposition of the 30% withholding tax under FATCA.

## SHARES ELIGIBLE FOR FUTURE SALE

Prior to this offering, there has been no public market for shares of our common stock. We cannot predict the effect, if any, that future sales of shares of common stock, or the availability for future sale of shares of common stock, will have on the market price of shares of our common stock prevailing from time to time. The sale of substantial amounts of shares of our common stock in the public market, or the perception that such sales could occur, could harm the prevailing market price of shares of our common stock.

Currently, no shares of our common stock are outstanding. Upon the completion of this offering, we will have a total of 114,999,967 shares of our common stock outstanding (or 117,249,967 shares of common stock if the underwriters exercise in full their option to purchase additional shares of common stock). Of the outstanding shares, all of the shares sold in this offering will be freely tradable (excluding any shares sold to our directors and officers in the directed share program), except that any shares, including shares sold to an entity affiliated with an existing shareholder that may purchase shares in this offering, held by our affiliates, as that term is defined in Rule 144 under the Securities Act, may only be sold in compliance with the limitations described below.

### Rule 144

In general, a person who has beneficially owned restricted stock for at least six months would be entitled to sell their securities provided that (i) such person is not deemed to have been one of our affiliates at the time of, or at any time during the 90 days preceding, a sale and (ii) we are subject to the Exchange Act, periodic reporting requirements for at least 90 days before the sale. Persons who have beneficially owned restricted shares for at least six months but who are our affiliates at the time of, or any time during the 90 days preceding, a sale, would be subject to additional restrictions, by which such person would be entitled to sell within any three-month period only a number of securities that does not exceed the greater of either of the following:

- 1% of the number of shares then outstanding, which will equal approximately 1,150,000 shares immediately after this offering assuming no exercise of the underwriters' option to purchase additional shares, based on the number of shares outstanding as of March 31, 2019; or
- the average weekly trading volume of our common stock on the Nasdaq Global Market during the four calendar weeks preceding the filing of a notice on Form 144 with respect to the sale;

provided, in each case, that we are subject to the Exchange Act periodic reporting requirements for at least 90 days before the sale. Such sales both by affiliates and by non-affiliates must also comply with the current public information and, with respect to sales by affiliates, the manner of sale, current public information and notice provisions, of Rule 144.

### Rule 701

Rule 701 under the Securities Act, as in effect on the date of this prospectus, permits resales of shares in reliance upon Rule 144 but without compliance with certain restrictions of Rule 144, including the holding period requirement. Most of our employees, executive officers or directors who purchased shares under a written compensatory plan or contract may be entitled to rely on the resale provisions of Rule 701, but all holders of Rule 701 shares are required to wait until 90 days after the date of this prospectus before selling their shares. However, substantially all Rule 701 shares are subject to lock-up agreements as described below and under "Underwriting (Conflicts of Interest)" included elsewhere in this prospectus and will become eligible for sale upon the expiration of the restrictions set forth in those agreements.

Our amended and restated certificate of incorporation authorizes us to issue additional shares of common stock for the consideration and on the terms and conditions established by our board of directors in its sole discretion. In accordance with the Delaware General Corporation Law and the provisions of our amended and restated certificate of incorporation, we may also issue preferred stock that has designations, preferences, rights, powers and duties that are different from, and may be senior to, those applicable to shares of common stock. See "Description of Capital Stock."

### **Lock-Up Agreements**

We, along with our directors, executive officers and substantially all of our other stockholders have agreed with the underwriters that for a period of 180 days (the restricted period), after the date of this prospectus, subject to specified exceptions, we or they will not offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, lend or otherwise transfer or dispose of, directly or indirectly, any shares of common stock or any securities convertible into or exercisable or exchangeable for shares of common stock, or enter into any swap or other arrangement that transfers to another, in whole or in part, any of the economic consequences of ownership of the common stock. Upon expiration of the “restricted” period, certain of our stockholders will have the right to require us to register their shares under the Securities Act of 1933, as amended, or the Securities Act. See “—Registration Rights” below and “Description of Capital Stock—Registration Rights.”

After this offering, certain of our employees, including our executive officers and/or directors, may enter into written trading plans that are intended to comply with Rule 10b5-1 under the Exchange Act. Sales under these trading plans would not be permitted until the expiration of the lock-up agreements relating to the offering described above.

### **Registration Rights**

Upon completion of this offering, certain holders of our securities will be entitled to various rights with respect to registration of their shares under the Securities Act. Registration of these shares under the Securities Act would result in these shares becoming fully tradable without restriction under the Securities Act immediately upon the effectiveness of the registration. See the section entitled “Description of Capital Stock—Registration Rights” appearing elsewhere in this prospectus for more information.

### **Stock Options and Restricted Stock**

As soon as practicable after the completion of this offering, we intend to file a Form S-8 registration statement under the Securities Act to register shares of our common stock subject to options and restricted stock outstanding or reserved for issuance under our 2019 Stock Option and Incentive Plan. This registration statement will become effective immediately upon filing, and shares covered by this registration statement will thereupon be eligible for sale in the public markets, subject to vesting restrictions, the lock-up agreements described above and Rule 144 limitations applicable to affiliates. For a more complete discussion of our stock plans, see “Executive and Director Compensation—Employee Benefit and Stock Plans.”

## UNDERWRITING (CONFLICTS OF INTEREST)

We and the underwriters named below have entered into an underwriting agreement with respect to the shares being offered. Subject to certain conditions, each underwriter has severally agreed to purchase the number of shares indicated in the following table. J.P. Morgan Securities LLC, Goldman Sachs & Co. LLC, Jefferies LLC and SVB Leerink LLC are the representatives of the underwriters.

<u>Underwriters</u>	<u>Number of Shares</u>
J.P. Morgan Securities LLC	
Goldman Sachs & Co. LLC	
Jefferies LLC	
SVB Leerink LLC	
KKR Capital Markets LLC	
Piper Jaffray & Co.	
Mizuho Securities USA LLC	
BMO Capital Markets Corp.	
Raymond James & Associates, Inc.	
Total	<u>15,000,000</u>

The underwriters are committed to take and pay for all of the shares being offered, if any are taken, other than the shares covered by the option described below unless and until this option is exercised.

The underwriters have an option to buy up to an additional 2,250,000 shares from the company to cover sales by the underwriters of a greater number of shares than the total number set forth in the table above. They may exercise that option for 30 days. If any shares are purchased pursuant to this option, the underwriters will severally purchase shares in approximately the same proportion as set forth in the table above.

The following tables show the per share and total underwriting discounts and commissions to be paid to the underwriters by the company. Such amounts are shown assuming both no exercise and full exercise of the underwriters' option to purchase 2,250,000 additional shares.

<u>Paid by Us</u>	<u>No Exercise</u>	<u>Full Exercise</u>
<u>Per Share</u>	\$	\$
Total	\$	\$

Shares sold by the underwriters to the public will initially be offered at the initial public offering price set forth on the cover of this prospectus. Any shares sold by the underwriters to securities dealers may be sold at a discount of up to \$            per share from the initial public offering price. After the initial offering of the shares, the representatives may change the offering price and the other selling terms. The offering of the shares by the underwriters is subject to receipt and acceptance and subject to the underwriters' right to reject any order in whole or in part.

The company and its officers, directors, and holders of substantially all of the company's common stock have agreed with the underwriters, subject to certain exceptions, not to (i) offer, sell, contract to sell, pledge, grant any option to purchase, make any short sale or otherwise dispose of any shares of common stock, or any options or warrants to purchase any shares of common stock, or any securities convertible into, exchangeable for or that represent the right to receive shares of common stock, whether now owned or hereinafter acquired, (ii) publicly disclose the intention to make any such offer, sale, pledge or disposition, (iii) engage in any hedging or other transaction that is designed to or that reasonably could be expected to lead to or result in a sale or disposition of such securities or (iv) make any demand for the registration of such securities during the period through the date 180 days after the date of this prospectus, except with the prior written consent of J.P. Morgan Securities LLC and Goldman Sachs & Co. LLC.

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The restrictions described in the immediately preceding paragraph do not apply to, among other items, the transfer or disposition of shares of our common stock:

- (i) acquired in this offering by holders that are not an officer or director;
- (ii) in transactions relating to common stock or other securities acquired in open market transactions after the date of the final prospectus;
- (iii) as a *bona fide* gift or gifts;
- (iv) to any trust for the direct or indirect benefit of the holder of such common stock;
- (v) by will or intestacy;
- (vi) to any immediate family member;
- (vii) to satisfy tax withholding obligations upon exercise or vesting or the exercise price upon a cashless net exercise, in each case, of share options, equity awards, warrants or other rights to acquire our common stock pursuant to our equity incentive plans described in this prospectus, provided that any filing made pursuant to Section 16(a) of the Exchange Act, shall include a footnote noting the circumstances described in this clause and no other public announcement shall be required or voluntarily made in connection with such transfer;
- (viii) if the holder is a corporation, partnership, limited liability company, trust or other business entity, pursuant to a distribution to partners, members or stockholders, subsidiaries or affiliates (as defined in Rule 405 promulgated under the Securities Act) of the holder or to any investment fund or other entity that controls or manages such holder (or is under common control or management with the undersigned);
- (ix) by operation of law or pursuant to a court order or settlement agreement related to the distribution of assets in connection with the dissolution of a marriage or civil union;
- (x) pursuant to a bona fide third party tender offer, merger, consolidation or other similar transaction made to all holders of our common stock involving a change of control after the completion of this offering that, in each case, has been approved by our board of directors, provided that all of the holders common stock subject to the restrictions in the lock-up that are not so transferred, sold, tendered or otherwise disposed of remain subject to the lock-up, and, provided further that in the event that the tender offer, merger, consolidation or other such transaction is not completed, the common stock owned by such holder shall remain subject to the lock-up agreement;
- (xi) in connection with the issuance of shares of our common stock upon the Reorganization; or
- (xii) with the prior written consent of J.P. Morgan Securities LLC and Goldman Sachs & Co. LLC on behalf of the underwriters;

provided that, in the case of any transfer or distribution pursuant to clauses (ii) through (v) or (vii), each transferee shall execute a lock-up agreement and in the case of any transfer or distribution pursuant to clauses (iii) through (vi), (viii) and (ix), no filing by any party under the Exchange Act or other public announcement shall be required or shall be made voluntarily in connection with such transfer or distribution (other than a filing on a Form 5 made after the expiration of the restricted period).

Prior to the offering, there has been no public market for the shares. The initial public offering price has been negotiated among the company and the representatives. Among the factors to be considered in determining the initial public offering price of the shares, in addition to prevailing market conditions, will be the company's historical performance, estimates of the business potential and earnings prospects of the company, an assessment of the company's management and the consideration of the above factors in relation to market valuation of companies in related businesses.

An application has been made to quote the common stock on Nasdaq under the symbol "BBIO".

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In connection with the offering, the underwriters may purchase and sell shares of common stock in the open market. These transactions may include short sales, stabilizing transactions and purchases to cover positions created by short sales. Short sales involve the sale by the underwriters of a greater number of shares than they are required to purchase in the offering, and a short position represents the amount of such sales that have not been covered by subsequent purchases. A “covered short position” is a short position that is not greater than the amount of additional shares for which the underwriters’ option described above may be exercised. The underwriters may cover any covered short position by either exercising their option to purchase additional shares or purchasing shares in the open market. In determining the source of shares to cover the covered short position, the underwriters will consider, among other things, the price of shares available for purchase in the open market as compared to the price at which they may purchase additional shares pursuant to the option described above. “Naked” short sales are any short sales that create a short position greater than the amount of additional shares for which the option described above may be exercised. The underwriters must cover any such naked short position by purchasing shares in the open market. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the common stock in the open market after pricing that could adversely affect investors who purchase in the offering. Stabilizing transactions consist of various bids for or purchases of common stock made by the underwriters in the open market prior to the completion of the offering.

The underwriters may also impose a penalty bid. This occurs when a particular underwriter repays to the underwriters a portion of the underwriting discount received by it because the representatives have repurchased shares sold by or for the account of such underwriter in stabilizing or short covering transactions.

Purchases to cover a short position and stabilizing transactions, as well as other purchases by the underwriters for their own accounts, may have the effect of preventing or retarding a decline in the market price of the company’s stock, and together with the imposition of the penalty bid, may stabilize, maintain or otherwise affect the market price of the common stock. As a result, the price of the common stock may be higher than the price that otherwise might exist in the open market. The underwriters are not required to engage in these activities and may end any of these activities at any time. These transactions may be effected on NYSE, NASDAQ NMS or relevant exchange, in the over-the-counter market or otherwise.

### *European Economic Area*

In relation to each Member State of the European Economic Area which has implemented the Prospectus Directive (each, a “Relative Member State”) an offer to the public of our common shares may not be made in that Relevant Member State, except that an offer to the public in that Relevant Member State of our common shares may be made at any time under the following exemptions under the Prospectus Directive:

- To any legal entity which is a qualified investor as defined in the Prospectus Directive;
- To fewer than 150 natural or legal persons (other than qualified investors as defined in the Prospectus Directive), subject to obtaining the prior consent of the Representatives for any such offer; or
- In any other circumstances falling within Article 3(2) of the Prospectus Directive;

provided that no such offer or shares of our common stock shall result in a requirement for the publication by us or any Brazilian placement agent of a prospectus pursuant to Article 3 of the Prospectus Directive.

For the purposes of this provision, the expression an “offer to public” in relation to our common shares in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and our common shares to be offered so as to enable an investor to decide to purchase our common shares, as the same may be varied in that Member State by any measure implementing the Prospectus Directive in that Member State, the expression “Prospectus Directive” means Directive 2003/71/EC (as



amended), including by Directive 2010/73/EU and includes any relevant implementing measure in the Relevant Member State.

This European Economic Area selling restriction is in addition to any other selling restrictions set out below.

#### *United Kingdom*

In the United Kingdom, this prospectus is only addressed to and directed as qualified investors who are (i) investment professionals falling within Article 19(5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005 (the Order); or (ii) high net worth entities and other persons to whom it may lawfully be communicated, falling within Article 49(2)(a) to (d) of the Order (all such persons together being referred to as “relevant persons”). Any investment or investment activity to which this prospectus relates is available only to relevant persons and will only be engaged with relevant persons. Any person who is not a relevant person should not act or rely on this prospectus or any of its contents.

#### *Canada*

The securities may be sold in Canada only to purchasers purchasing, or deemed to be purchasing, as principal that are accredited investors, as defined in National Instrument 45-106 Prospectus Exemptions or subsection 73.3(1) of the Securities Act (Ontario), and are permitted clients, as defined in National Instrument 31-103 Registration Requirements, Exemptions, and Ongoing Registrant Obligations. Any resale of the securities must be made in accordance with an exemption form, or in a transaction not subject to, the prospectus requirements of applicable securities laws.

Securities legislation in certain provinces or territories of Canada may provide a purchaser with remedies for rescission or damages if this offering memorandum (including any amendment thereto) contains a misrepresentation, provided that the remedies for rescission or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser’s province or territory. The purchaser should refer to any applicable provisions of the securities legislation of the purchaser’s province or territory of these rights or consult with a legal advisor.

Pursuant to section 3A.3 of National Instrument 33-105 Underwriting Conflicts (NI 33-105), the underwriters are not required to comply with the disclosure requirements of NI 33-105 regarding underwriter conflicts of interest in connection with this offering.

#### *Hong Kong*

The shares may not be offered or sold in Hong Kong by means of any document other than (i) in circumstances which do not constitute an offer to the public within the meaning of the Companies (Winding Up and Miscellaneous Provisions) Ordinance (Cap. 32 of the Laws of Hong Kong) (“Companies (Winding Up and Miscellaneous Provisions) Ordinance”) or which do not constitute an invitation to the public within the meaning of the Securities and Futures Ordinance (Cap. 571 of the Laws of Hong Kong) (“Securities and Futures Ordinance”), or (ii) to “professional investors” as defined in the Securities and Futures Ordinance and any rules made thereunder, or (iii) in other circumstances which do not result in the document being a “prospectus” as defined in the Companies (Winding Up and Miscellaneous Provisions) Ordinance, and no advertisement, invitation or document relating to the shares may be issued or may be in the possession of any person for the purpose of issue (in each case whether in Hong Kong or elsewhere), which is directed at, or the contents of which are likely to be accessed or read by, the public in Hong Kong (except if permitted to do so under the securities laws of Hong Kong) other than with respect to shares which are or are intended to be disposed of only to persons outside Hong Kong or only to “professional investors” in Hong Kong as defined in the Securities and Futures Ordinance and any rules made thereunder.

*Singapore*

This prospectus has not been registered as a prospectus with the Monetary Authority of Singapore. Accordingly, this prospectus and any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of the shares may not be circulated or distributed, nor may the shares be offered or sold, or be made the subject of an invitation for subscription or purchase, whether directly or indirectly, to persons in Singapore other than (i) to an institutional investor (as defined under Section 4A of the Securities and Futures Act, Chapter 289 of Singapore (the “SFA”)) under Section 274 of the SFA, (ii) to a relevant person (as defined in Section 275(2) of the SFA) pursuant to Section 275(1) of the SFA, or any person pursuant to Section 275(1A) of the SFA, and in accordance with the conditions specified in Section 275 of the SFA or (iii) otherwise pursuant to, and in accordance with the conditions of, any other applicable provision of the SFA, in each case subject to conditions set forth in the SFA.

Where the shares are subscribed or purchased under Section 275 of the SFA by a relevant person which is a corporation (which is not an accredited investor (as defined in Section 4A of the SFA)) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor, the securities (as defined in Section 239(1) of the SFA) of that corporation shall not be transferable for 6 months after that corporation has acquired the shares under Section 275 of the SFA except: (1) to an institutional investor under Section 274 of the SFA or to a relevant person (as defined in Section 275(2) of the SFA), (2) where such transfer arises from an offer in that corporation’s securities pursuant to Section 275(1A) of the SFA, (3) where no consideration is or will be given for the transfer, (4) where the transfer is by operation of law, (5) as specified in Section 276(7) of the SFA, or (6) as specified in Regulation 32 of the Securities and Futures (Offers of Investments) (Shares and Debentures) Regulations 2005 of Singapore (“Regulation 32”)

Where the shares are subscribed or purchased under Section 275 of the SFA by a relevant person which is a trust (where the trustee is not an accredited investor (as defined in Section 4A of the SFA)) whose sole purpose is to hold investments and each beneficiary of the trust is an accredited investor, the beneficiaries’ rights and interest (howsoever described) in that trust shall not be transferable for 6 months after that trust has acquired the shares under Section 275 of the SFA except: (1) to an institutional investor under Section 274 of the SFA or to a relevant person (as defined in Section 275(2) of the SFA), (2) where such transfer arises from an offer that is made on terms that such rights or interest are acquired at a consideration of not less than S\$200,000 (or its equivalent in a foreign currency) for each transaction (whether such amount is to be paid for in cash or by exchange of securities or other assets), (3) where no consideration is or will be given for the transfer, (4) where the transfer is by operation of law, (5) as specified in Section 276(7) of the SFA, or (6) as specified in Regulation 32.

*Japan*

The securities have not been and will not be registered under the Financial Instruments and Exchange Act of Japan (Act No. 25 of 1948, as amended), or the FIEA. The securities may not be offered or sold, directly or indirectly, in Japan or to or for the benefit of any resident of Japan (including any person resident in Japan or any corporation or other entity organized under the laws of Japan) or to others for reoffering or resale, directly or indirectly, in Japan or to or for the benefit of any resident of Japan, except pursuant to an exemption from the registration requirements of the FIEA and otherwise in compliance with any relevant laws and regulations of Japan.

The company estimates that its share of the total expenses of the offering, excluding underwriting discounts and commissions, will be approximately \$5.6 million.

The company has agreed to indemnify the several underwriters against certain liabilities, including liabilities under the Securities Act of 1933.

The underwriters and their respective affiliates are full service financial institutions engaged in various activities, which may include sales and trading, commercial and investment banking, advisory, investment management,

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investment research, principal investment, hedging, market making, brokerage and other financial and non-financial activities and services. Certain of the underwriters and their respective affiliates have provided, and may in the future provide, a variety of these services to the issuer and to persons and entities with relationships with the issuer, for which they received or will receive customary fees and expenses.

In the ordinary course of their various business activities, the underwriters and their respective affiliates, officers, directors and employees may purchase, sell or hold a broad array of investments and actively trade securities, derivatives, loans, commodities, currencies, credit default swaps and other financial instruments for their own account and for the accounts of their customers, and such investment and trading activities may involve or relate to assets, securities and/or instruments of the issuer (directly, as collateral securing other obligations or otherwise) and/or persons and entities with relationships with the issuer. The underwriters and their respective affiliates may also communicate independent investment recommendations, market color or trading ideas and/or publish or express independent research views in respect of such assets, securities or instruments and may at any time hold, or recommend to clients that they should acquire, long and/or short positions in such assets, securities and instruments.

### *Conflicts of Interest*

Affiliates of KKR Capital Markets LLC own more than 10% of our common stock. Because KKR Capital Markets LLC is an underwriter for this offering, it is deemed to have a “conflict of interest” within the meaning of FINRA Rule 5121(f)(5)(B). Accordingly, this offering is being made in compliance with the requirements of FINRA Rule 5121. Since KKR Capital Markets LLC is not primarily responsible for managing this offering, pursuant to FINRA Rule 5121, the appointment of a qualified independent underwriter is not necessary. KKR Capital Markets LLC will not confirm sales to discretionary accounts without the prior written approval of the account holder.

## LEGAL MATTERS

The validity of the common stock offered hereby will be passed upon for us by Goodwin Procter LLP, San Francisco, California and for the underwriters by Latham & Watkins LLP, Menlo Park, California.

## EXPERTS

### **BridgeBio Pharma LLC**

The combined and consolidated financial statements as of and for the years ended December 31, 2017 and December 31, 2018 included in this Prospectus, have been audited by Deloitte & Touche LLP, an independent registered public accounting firm, as stated in their report appearing herein. Such combined and consolidated financial statements are included in reliance upon the report of such firm given upon their authority as experts in accounting and auditing.

### **BridgeBio Pharma, Inc.**

The financial statement of BridgeBio Pharma, Inc. as of May 17, 2019 included in this Prospectus, has been audited by Deloitte & Touche LLP, an independent registered public accounting firm, as stated in their report appearing herein. Such financial statement is included in reliance upon the report of such firm given upon their authority as experts in accounting and auditing.

## WHERE YOU CAN FIND MORE INFORMATION

We have filed with the SEC a registration statement on Form S-1 (File Number 333-231759) under the Securities Act with respect to the common stock we are offering by this prospectus. This prospectus does not contain all of the information included in the registration statement. For further information pertaining to us and our common stock, you should refer to the registration statement and to its exhibits. Whenever we make reference in this prospectus to any of our contracts, agreements or other documents, the references are not necessarily complete, and you should refer to the exhibits attached to the registration statement for copies of the actual contract, agreement or other document.

Upon the closing of the offering, we will be subject to the informational requirements of the Exchange Act and will file annual, quarterly and current reports, proxy statements and other information with the SEC. You can read our SEC filings, including the registration statement, at the SEC's website at [www.sec.gov](http://www.sec.gov). We also maintain a website at <https://bridgebio.com>. Upon completion of the offering, you may access, free of charge, our annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K and amendment to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act as soon as reasonably practicable after such material is electronically filed with, or furnished to, the SEC.

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**REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM**

To the board of directors of BridgeBio Pharma, Inc.

**Opinion on the Financial Statement**

We have audited the accompanying balance sheet of BridgeBio Pharma, Inc. (the “Company”) as of May 17, 2019, and the related notes (collectively referred to as the “financial statement”). In our opinion, the financial statement presents fairly, in all material respects, the financial position of the Company as of May 17, 2019, in conformity with accounting principles generally accepted in the United States of America.

**Basis for Opinion**

This financial statement is the responsibility of the Company’s management. Our responsibility is to express an opinion on this financial statement based on our audit. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statement is free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audit, we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company’s internal control over financial reporting. Accordingly, we express no such opinion.

Our audit included performing procedures to assess the risks of material misstatement of the financial statement, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statement. Our audit also included assessing the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statement. We believe that our audit of the financial statement provides a reasonable basis for our opinion.

/s/ Deloitte & Touche LLP

San Francisco, California

May 24, 2019

We have served as the Company’s auditor since 2019.

**BRIDGEBIO PHARMA, INC.****Balance Sheet**  
*(in thousands, except share and per share amounts)*

	<u>May 17, 2019</u>
<b>Assets</b>	
Total assets	\$ —
<b>Liabilities</b>	
Total liabilities	\$ —
Commitments and contingencies	
<b>Stockholders' equity</b>	
Stockholders' equity:	
Common stock, \$0.001 par value, 1,000 shares authorized, none issued and outstanding	\$ —
Total liabilities and stockholders' equity	\$ —

*The accompanying notes are an integral part of this balance sheet.*

**BRIDGEBIO PHARMA, INC.**

**Notes to Balance Sheet**

**1. Organization and Background**

BridgeBio Pharma, Inc. (the “Corporation”) was formed as a Delaware corporation on May 17, 2019. The Corporation was formed for the purpose of completing an initial public offering and related transactions in order to carry on the business of BridgeBio Pharma LLC. The Corporation will be the sole managing member of BridgeBio Pharma LLC and will operate and control all of the businesses and affairs of BridgeBio Pharma LLC. The Corporation does not intend to be an operating entity and incur any expenses prior to, and independent of, its initial public offering.

**2. Summary of Significant Accounting Policies**

*Basis of Presentation*

The balance sheet is presented in accordance with generally accepted accounting principles in the United States of America (“GAAP”). Statements of operations, stockholders’ equity and cash flows have not been presented because there have been no activities in this entity.

*Underwriting Commissions and Offering Costs*

Underwriting commissions and offering costs to be incurred in connection with the Corporation’s common share offerings will be reflected as a reduction of additional paid-in capital. Underwriting commissions and offering costs are not recorded in the Corporation’s balance sheet because such costs are not the Corporation’s liability until the Corporation completes a successful initial public offering.

*Organizational Costs*

Organizational costs are not recorded in the Corporation’s balance sheet because such costs are not the Corporation’s liability until the Corporation completes a successful initial public offering. Thereafter, costs incurred to organize the Corporation will be expensed as incurred.

**3. Stockholders’ Equity**

The Corporation is authorized to issue 1,000 shares of common stock, par value \$0.001 per share, none of which have been issued or are outstanding as of May 24, 2019.

**4. Subsequent Events**

Subsequent events through May 24, 2019, the date on which the balance sheet was available to be issued, were evaluated by the Corporation to determine the need, if any, for recognition or disclosure in this balance sheet. The Corporation did not note any subsequent events requiring disclosure or adjustments to the balance sheet.



**REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM**

To the members and the Board of Managers of BridgeBio Pharma LLC

**Opinion on the Financial Statements**

We have audited the accompanying combined and consolidated balance sheets of BridgeBio Pharma LLC, its subsidiaries and controlled entities (the “Company”) as of December 31, 2017 and 2018, the related combined and consolidated statements of operations and comprehensive loss, redeemable convertible preferred units, redeemable founder units, redeemable common units, management incentive units, redeemable convertible noncontrolling interests and members’ deficit, and cash flows, for each of the two years in the period ended December 31, 2018, and the related notes (collectively referred to as the “financial statements”). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2017 and 2018, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2018, in conformity with accounting principles generally accepted in the United States of America.

**Basis for Opinion**

These financial statements are the responsibility of the Company’s management. Our responsibility is to express an opinion on the Company’s financial statements based on our audit. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB and in accordance with auditing standards generally accepted in the United States of America. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits, we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company’s internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provides a reasonable basis for our opinion.

/s/ Deloitte & Touche LLP

San Francisco, California

April 15, 2019 (May 16, 2019 as to the effects of the restatements discussed in Note 2 and Note 7)

We have served as the Company’s auditor since 2018.

**BRIDGEBIO PHARMA LLC**
**Combined and Consolidated Balance Sheets**  
*(in thousands, except units and per unit amounts)*

	<b>December 31,</b>	
	<b>2017</b>	<b>2018</b>
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 91,995	\$ 436,086
Prepaid expenses and other current assets	5,136	9,137
Total current assets	97,131	445,223
Property and equipment, net	440	1,575
PellePharm investment	—	17,050
Other assets	473	1,093
Total assets	<u>\$ 98,044</u>	<u>\$ 464,941</u>
<b>Liabilities, Redeemable Convertible Preferred Units, Redeemable Founder Units, Redeemable Common Units, Management Incentive Units, Redeemable Convertible Noncontrolling Interests and Members' Deficit</b>		
Current liabilities:		
Accounts payable	\$ 1,885	\$ 13,509
Accrued compensation and benefits	1,426	4,047
Accrued research and development liabilities	3,129	8,915
Accrued distributions to unitholders	997	997
LEO call option liability	—	3,009
Other accrued liabilities	1,113	2,100
Total current liabilities	8,550	32,577
Term loans, noncurrent	—	54,507
Other liabilities	312	495
Total liabilities	<u>8,862</u>	<u>87,579</u>
Commitments and contingencies (Note 9)		
Redeemable convertible preferred units (Series A, Series B, Series C and Series D); no par value; 257,000,129 and 407,955,726 units authorized as of December 31, 2017 and 2018; 219,406,923 and 407,955,726 units issued and outstanding as of December 31, 2017 and 2018; aggregate liquidation value of \$507,032 as of December 31, 2018	143,867	478,865
Redeemable founder units; no par value; 11,420,741 units authorized, issued and outstanding as of December 31, 2017 and 2018; aggregate liquidation value of \$6,202 as of December 31, 2018	1,754	1,754
Redeemable common units; no par value; 9,098,522 units authorized as of December 31, 2017 and 2018; 5,856,075 and 7,197,783 units issued and outstanding as of December 31, 2017 and 2018; aggregate liquidation value of \$3,910 as of December 31, 2018	1,431	1,619
Management incentive units; no par value; 45,428,102 and 48,695,602 units authorized as of December 31, 2017 and 2018; 9,835,925 and 19,117,628 issued and outstanding as of December 31, 2017 and 2018	226	3,221
Redeemable convertible noncontrolling interests	833	122
Members' deficit:		
Accumulated deficit	(61,427)	(170,580)
Total BridgeBio members' deficit	(61,427)	(170,580)
Noncontrolling interests	2,498	62,361
Total members' deficit	<u>(58,929)</u>	<u>(108,219)</u>
Total liabilities, redeemable convertible preferred units, redeemable founder units, redeemable common units, management incentive units, redeemable convertible noncontrolling interests and members' deficit	<u>\$ 98,044</u>	<u>\$ 464,941</u>

*The accompanying notes are an integral part of these combined and consolidated financial statements.*

## BRIDGEBIO PHARMA LLC

Combined and Consolidated Statements of Operations and Comprehensive Loss  
(in thousands, except units and per unit amounts)

	Year Ended December 31,	
	2017	2018
Operating expenses:		
Research and development	\$ 30,556	\$ 140,073
General and administrative	13,302	43,587
Total operating expenses	43,858	183,660
Loss from operations	(43,858)	(183,660)
Other income (expense), net:		
Interest income	39	2,004
Interest expense	(13)	(2,547)
Gain on deconsolidation of PellePharm	—	19,327
Loss from PellePharm	—	(275)
LEO call option expense	—	(3,009)
Other expense	—	(1,291)
Total other income (expense), net	26	14,209
Net loss and comprehensive loss	(43,832)	(169,451)
Net loss attributable to redeemable convertible noncontrolling interests and noncontrolling interests	13,267	38,702
Net loss and comprehensive loss attributable to BridgeBio	(30,565)	(130,749)
Cumulative returns on redeemable convertible preferred units (Series A, Series B and Series C)	(5,672)	(13,287)
Net loss attributable to redeemable founder units and redeemable common units	\$ (36,237)	\$ (144,036)
Net loss per unit attributable to redeemable founder unitholders and redeemable common unitholders, basic and diluted	\$ (2.18)	\$ (8.01)
Total weighted-average redeemable founder units and redeemable common units used in computing net loss per unit, basic and diluted	16,650,073	17,991,781
Pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited)		\$ (2.32)
Total weighted-average shares used in computing pro forma net loss per share, basic and diluted (unaudited)		62,087,997

The accompanying notes are an integral part of these combined and consolidated financial statements.

BRIDGEBIO PHARMA LLC

Combined and Consolidated Statements of Redeemable Convertible Preferred Units, Redeemable Founder Units, Redeemable Common Units, Management Incentive Units, Redeemable Convertible Noncontrolling Interests and Members' Deficit  
(in thousands, except units and per unit amounts)

	Redeemable Convertible Preferred Units		Redeemable Founder Units		Redeemable Common Units		Management Incentive Units		Redeemable Convertible Noncontrolling Interests	Accumulated Deficit	Noncontrolling Interests	Total Members' Deficit
	Units	Amount	Units	Amount	Units	Amount	Units	Amount				
<b>Balances as of January 1, 2017</b>	88,943,092	\$ 31,280	11,420,741	\$ 1,124	4,514,367	\$ 589	3,588,901	\$ 26	\$ 1,520	\$ (18,130)	\$ 2,595	\$ (15,535)
MyoKardia distributions (Note 13)	—	(1,727)	—	(234)	—	(187)	—	—	—	—	—	—
Issuance and vesting of redeemable common units and associated equity-based compensation	—	—	—	—	1,341,708	341	—	—	—	—	—	—
Issuance and vesting of management incentive units and associated equity-based compensation	—	—	—	—	—	—	6,247,024	200	—	—	—	—
Issuance of Series B redeemable convertible preferred units at \$0.44 per unit, net of issuance costs of \$0	26,901,279	11,837	—	—	—	—	—	—	—	—	—	—
Settlement of Series B redeemable convertible preferred unit tranche liability on issuance of Series B redeemable convertible preferred units (Note 13)	—	183	—	—	—	—	—	—	—	—	—	—
Issuance of Series C redeemable convertible preferred units at \$0.9656 per unit, net of issuance costs of \$818	99,420,050	95,182	—	—	—	—	—	—	—	—	—	—
Conversion of promissory notes into Series C redeemable convertible preferred units at \$0.9656 per unit	4,142,502	4,000	—	—	—	—	—	—	—	—	—	—
Capital transaction upon Merger (Note 2)	—	2,980	—	864	—	688	—	—	—	(4,532)	—	(4,532)
Repayment of nonrecourse notes	—	132	—	—	—	—	—	—	—	—	—	—
Issuance of noncontrolling interest	—	—	—	—	—	—	—	—	2,839	—	1,444	1,444
Transfers to (from) noncontrolling interest	—	—	—	—	—	—	—	—	(769)	(8,200)	8,969	769
Net loss and comprehensive loss	—	—	—	—	—	—	—	—	(2,757)	(30,565)	(10,510)	(41,075)
<b>Balances as of December 31, 2017</b>	<b>219,406,923</b>	<b>143,867</b>	<b>11,420,741</b>	<b>1,754</b>	<b>5,856,075</b>	<b>1,431</b>	<b>9,835,925</b>	<b>226</b>	<b>833</b>	<b>(61,427)</b>	<b>2,498</b>	<b>(58,929)</b>
Issuance and vesting of redeemable common units and associated equity-based compensation	—	—	—	—	1,341,708	188	—	—	—	—	—	—
Issuance and vesting of management incentive units and associated equity-based compensation	—	—	—	—	—	—	9,281,703	2,995	—	—	—	—
Issuance of Series C redeemable convertible preferred units at \$0.9656 per unit, net of issuance costs of \$0	37,593,206	36,300	—	—	—	—	—	—	—	—	—	—
Issuance of Series D redeemable convertible preferred units at \$1.9823 per unit, net of issuance costs of \$541	150,955,597	298,698	—	—	—	—	—	—	—	—	—	—
Issuance of noncontrolling interest	—	—	—	—	—	—	—	—	62,363	—	99,479	99,479
Repurchase of noncontrolling interest	—	—	—	—	—	—	—	—	—	—	(44,234)	(44,234)
Transfers to (from) and conversion of noncontrolling interest	—	—	—	—	—	—	—	—	(51,698)	21,596	30,102	51,698
Deconsolidation of PellePharm	—	—	—	—	—	—	—	—	1,154	—	688	688
Net loss and comprehensive loss	—	—	—	—	—	—	—	—	(12,530)	(130,749)	(26,172)	(156,921)
<b>Balances as of December 31, 2018</b>	<b>407,955,726</b>	<b>\$478,865</b>	<b>11,420,741</b>	<b>\$ 1,754</b>	<b>7,197,783</b>	<b>\$ 1,619</b>	<b>19,117,628</b>	<b>\$ 3,221</b>	<b>\$ 122</b>	<b>\$ (170,580)</b>	<b>\$ 62,361</b>	<b>\$(108,219)</b>

The accompanying notes are an integral part of these combined and consolidated financial statements.

## BRIDGEBIO PHARMA LLC

Combined and Consolidated Statements of Cash Flows  
(in thousands)

	Year Ended December 31,	
	2017	2018
<b>Operating activities</b>		
Net loss	\$(43,832)	\$(169,451)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	260	245
Equity-based compensation	1,841	6,067
Loss on disposal of property and equipment, net	—	7
Gain on deconsolidation of PellePharm	—	(19,327)
Loss from PellePharm	—	275
Accretion of term loans and convertible promissory notes	—	783
Acquired in-process research and development assets	—	17,922
Shares issued under license agreements	—	190
LEO call option expense	—	3,009
Change in fair value of Eidos financial instruments	—	1,146
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(4,301)	(6,100)
Other assets	(92)	(843)
Accounts payable	1,577	16,700
Accrued compensation and benefits	1,131	3,396
Accrued research and development liabilities	2,650	5,785
Other accrued liabilities	10	3,371
Other liabilities	268	182
Net cash used in operating activities	(40,488)	(136,643)
<b>Investing activities</b>		
Decrease in cash and cash equivalents resulting from deconsolidation of PellePharm	—	(2,858)
Cash paid for in-process research and development assets acquired	—	(16,000)
Purchases of property and equipment	(464)	(2,178)
Net cash used in investing activities	(464)	(21,036)
<b>Financing activities</b>		
Proceeds from the issuance of Series B redeemable convertible preferred units, net of issuance costs	11,837	—
Proceeds from the issuance of Series C redeemable convertible preferred units, net of issuance costs	95,182	36,300
Proceeds from the issuance of Series D redeemable convertible preferred units, net of issuance costs	—	298,698
Proceeds from issuance of common stock in connection with the initial public offering of Eidos, net of underwriting discounts and commissions	—	95,536
Proceeds from issuance of promissory notes	4,000	1,000
Proceeds from repayment of nonrecourse notes	132	—
Proceeds from term loans, net of issuance costs	—	56,438
Repayment of term loans	—	(1,097)
Proceeds from third-party investors in redeemable convertible noncontrolling interests	2,839	58,430
MyoKardia distributions (Note 13)	(1,151)	—

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	Year Ended December 31,	
	2017	2018
Proceeds from repayment of the loans received by noncontrolling interest shareholder	—	37
Proceeds from subsidiary stock option exercises	144	440
Repurchase of noncontrolling interest	—	(44,234)
Net cash provided by financing activities	112,983	501,548
Net increase in cash, cash equivalents and restricted cash	72,031	343,869
Cash, cash equivalents and restricted cash at beginning of year	20,345	92,376
Cash, cash equivalents and restricted cash at end of year	<u>\$ 92,376</u>	<u>\$436,245</u>
<b>Supplemental Disclosures of Cash Flow Information:</b>		
Cash paid for interest	<u>\$ —</u>	<u>\$ 1,574</u>
<b>Supplemental Disclosures of Non-Cash Investing and Financing Information:</b>		
Conversion of promissory note upon issuance of Series C redeemable convertible preferred units	<u>\$ 4,000</u>	<u>\$ —</u>
Transfers to (from) noncontrolling interest (Note 7)	<u>\$ 8,200</u>	<u>\$ 21,596</u>
Conversion of promissory note into redeemable convertible noncontrolling interest	<u>\$ —</u>	<u>\$ 1,005</u>
Settlement of Series B redeemable convertible preferred unit tranche liability	<u>\$ 183</u>	<u>\$ —</u>
Accrued MyoKardia distributions (Note 13)	<u>\$ 997</u>	<u>\$ —</u>
Capital transaction upon Merger (Note 2)	<u>\$ 4,532</u>	<u>\$ —</u>
Conversion of redeemable noncontrolling interest into noncontrolling interest	<u>\$ —</u>	<u>\$ 12,252</u>
Fair value of redeemable convertible noncontrolling interest issued for acquired in-process research and development assets	<u>\$ —</u>	<u>\$ 1,922</u>

*The accompanying notes are an integral part of these combined and consolidated financial statements.*

## BRIDGEBIO PHARMA LLC

### Notes to Combined and Consolidated Financial Statements

#### 1. Organization and Description of Business

BridgeBio Pharma LLC (“BridgeBio”) was established to identify and advance transformative medicines to treat patients who suffer from Mendelian diseases, which are diseases that arise from defects in a single gene, and cancers with clear genetic drivers. BridgeBio’s pipeline of programs spans early discovery to late stage development. Since inception, BridgeBio has either created wholly-owned subsidiaries or has made investments in certain controlled entities, including partially-owned subsidiaries for which BridgeBio has a majority voting interest and variable interest entities (“VIEs”) for which BridgeBio is the primary beneficiary (collectively, the “Company”). BridgeBio is headquartered in Palo Alto, California.

#### *Liquidity and Going Concern*

The Company has incurred significant losses and negative cash flows from operations since its inception and has an accumulated deficit of \$170.6 million as of December 31, 2018. The Company has cash and cash equivalents of \$436.1 million as of December 31, 2018, of which \$238.7 million was held by BridgeBio. The remaining cash and cash equivalents were held by the Company’s wholly-owned subsidiaries and controlled entities and these funds are designated for specific entity usage, except in limited circumstances.

The Company has historically financed its operations primarily through the sale of its equity securities and, to a lesser extent, debt borrowings. To date, none of the Company’s product candidates have been approved for sale and therefore the Company has not generated any revenue from product sales. Management expects operating losses to continue for the foreseeable future. The Company believes that its existing cash and cash equivalents will be sufficient for the Company to continue as a going concern for at least one year from the issuance date of its December 31, 2018 combined and consolidated financial statements.

#### 2. Summary of Significant Accounting Policies

##### *Basis of Presentation and Consolidation*

Prior to June 2017, the Company consisted of two separate legal entities, BridgeBio and BridgeBio LLC. Historically, the Company’s members have provided funding to both entities, and in June 2017, to consolidate the investments made in both entities, BridgeBio and BridgeBio LLC merged through a transaction under common control (the “Merger”). As part of the Merger, BridgeBio LLC’s redeemable convertible Series A preferred units, redeemable convertible Series B preferred units, redeemable founder units and redeemable common units were cancelled and holders of such units were issued the same number of BridgeBio’s redeemable convertible Series A preferred units, redeemable convertible Series B preferred units, redeemable founder units and redeemable common units. As a result of the Merger, the Company recorded a capital transaction of \$4.5 million to accumulated deficit in June 2017, which represents the difference between the carrying amounts of the cancelled and newly issued units. As a result of the Merger, there was no gain or loss recognized at BridgeBio LLC for tax purposes.

The combined and consolidated financial statements represent the combined balances of BridgeBio and BridgeBio LLC since inception. The combined and consolidated financial statements include the accounts of BridgeBio as well as wholly-owned subsidiaries and controlled entities including partially-owned subsidiaries for which BridgeBio has a majority voting interest under the voting interest model (“VOE”) and VIEs for which BridgeBio is the primary beneficiary under the VIE model (collectively, the “consolidated entities”). Ownership interests in entities over which the Company has significant influence, but not a controlling financial interest, are accounted for as equity method investments. The accompanying combined and consolidated financial statements are presented in accordance with generally accepted accounting principles in the United States of America (“GAAP”). All intercompany transactions and balances have been eliminated upon consolidation.

**BRIDGEBIO PHARMA LLC**

**Notes to Combined and Consolidated Financial Statements**

Ownership interests in consolidated entities that are held by entities other than BridgeBio are reported as redeemable convertible noncontrolling interests and noncontrolling interests in the combined and consolidated balance sheets. Losses attributed to redeemable convertible noncontrolling interests and noncontrolling interests are reported separately in the combined and consolidated statements of operations and comprehensive loss.

***Variable Interest Entities and Voting Interest Entities***

BridgeBio consolidates those entities in which it has a direct or indirect controlling financial interest based on either the VIE model or the VOE model.

VIEs are entities that, by design, either (i) lack sufficient equity to permit the entity to finance its activities without additional subordinated financial support from other parties; or (ii) have equity investors that do not have the ability to make significant decisions relating to the entity's operations through voting rights, or do not have the obligation to absorb the expected losses, or do not have the right to receive the residual returns of the entity.

The primary beneficiary of a VIE is required to consolidate the assets and liabilities of the VIE. The primary beneficiary is the party that has both (i) the power to direct the activities of the VIE that most significantly impact the VIE's economic performance; and (ii) the obligation to absorb losses or the right to receive benefits from the VIE that could potentially be significant to the VIE through its interest in the VIE.

To assess whether BridgeBio has the power to direct the activities of a VIE that most significantly impact the VIE's economic performance, BridgeBio considers all the facts and circumstances, including its role in establishing the VIE and its ongoing rights and responsibilities. This assessment includes identifying the activities that most significantly impact the VIE's economic performance and identifying which party, if any, has power over those activities. In general, the parties that make the most significant decisions affecting the VIE (management and representation on the Board of Directors) and have the right to unilaterally remove those decision-makers are deemed to have the power to direct the activities of a VIE.

To assess whether BridgeBio has the obligation to absorb losses of the VIE or the right to receive benefits from the VIE that could potentially be significant to the VIE, BridgeBio considers all of its economic interests, which primarily include equity investments in preferred and common stock and issuance of notes that are convertible into preferred stock, that are deemed to be variable interests in the VIE. This assessment requires BridgeBio to apply judgment in determining whether these interests, in the aggregate, are considered potentially significant to the VIE. Factors considered in assessing the significance include: the design of the VIE, including its capitalization structure; subordination of interests; payment priority; relative share of interests held across various classes within the VIE's capital structure; and the reasons why the interests are held by BridgeBio.

At the VIE's inception, BridgeBio determines whether it is the primary beneficiary and if the VIE should be consolidated based on the facts and circumstances. BridgeBio then performs on-going reassessments of the VIE based on reconsideration events and reevaluates whether a change to the consolidation conclusion is required each reporting period. Refer to Note 6.

Entities that do not qualify as a VIE are assessed for consolidation under the VOE model. Under the VOE model, BridgeBio consolidates the entity if it determines that it, directly or indirectly, has greater than 50% of the voting shares and that other equity holders do not have substantive voting, participating or liquidation rights. Refer to Note 6.



**BRIDGEBIO PHARMA LLC**

**Notes to Combined and Consolidated Financial Statements**

BridgeBio has either created or made investments in the following entities:

<b>Consolidated Entities</b>	<b>Relationship as of December 31, 2018</b>	<b>Date Control First Acquired</b>	<b>Ownership % as of January 1, 2017</b>	<b>Ownership % as of December 31, 2017</b>	<b>Ownership % as of December 31, 2018</b>
TheRas, Inc.	Wholly-owned subsidiary	August 2016	100%	100%	100%
BridgeBio Services, Inc.	Wholly-owned subsidiary	April 2017	—	100%	100%
Origin Biosciences, Inc.	Wholly-owned subsidiary	April 2018	—	—	100%
Fortify Therapeutics, Inc.	Wholly-owned subsidiary	June 2018	—	—	100%
Sub20, Inc.	Wholly-owned subsidiary	June 2018	—	—	100%
Unnamed Entity	Wholly-owned subsidiary	December 2018	—	—	100%
Eidos Therapeutics, Inc.(1)	Partially-owned subsidiary	April 2016	53.8%	79.9%	62.5%
Molecular Skin Therapeutics, Inc.	Controlled VIE	July 2016	80%	56.5%	61.7%
Quartz Therapeutics, Inc.	Controlled VIE	October 2016	100%	89.0%	89.0%
PellePharm, Inc.(2)	VIE	December 2016	52.6%	54.7%	43.3%
Navire Pharma, Inc.	Controlled VIE	February 2017	—	80.0%	78.8%
CoA Therapeutics, Inc.	Controlled VIE	February 2017	—	100%	99.5%
Dermeccular Therapeutics, Inc.	Controlled VIE	April 2017	—	86.0%	87.6%
Phoenix Tissue Repair, Inc.	Controlled VIE	July 2017	—	23.0%	56.7%
QED Therapeutics, Inc.	Controlled VIE	January 2018	—	—	94.4%
Adrenas Therapeutics, Inc.	Controlled VIE	January 2018	—	—	90.1%
Orfan Biotech, Inc.	Controlled VIE	January 2018	—	—	85.1%
Ferro Therapeutics, Inc.	Controlled VIE	March 2018	—	—	89.4%
Venthera, Inc.	Controlled VIE	April 2018	—	—	82.0%
Aspa Therapeutics, Inc.	Controlled VIE	June 2018	—	—	92.5%

(1) BridgeBio determined that Eidos Therapeutics, Inc. (“Eidos”) was a controlled VIE as of December 31, 2017 and through its initial public offering in June 2018, at which time it determined that Eidos is no longer a VIE. Subsequent to the Eidos initial public offering and through December 31, 2018, BridgeBio has a majority voting interest in Eidos and consolidates Eidos under the VOE model. Refer to Note 6.

(2) BridgeBio determined that it was the primary beneficiary of PellePharm, Inc. (“PellePharm”) as of December 31, 2017 and through the execution of a series of agreements (the “Leo Agreement”) with LEO Pharma A/S and LEO Spiny Merger Sub, Inc. (“LEO”) in November 2018, at which time BridgeBio determined that it is no longer the primary beneficiary and deconsolidated PellePharm. Refer to Note 6 and Note 8. The ownership percentage as of December 31, 2018 of 43.3% in the above table has been revised to correct the previously reported percentage of 52.1%.

**Equity Method and Cost Method Investments**

The Company utilizes the equity method to account for investments when it possesses the ability to exercise significant influence, but not control, over the operating and financial decisions of the investee. Generally, the ability to exercise significant influence is presumed when the investor possesses more than 20% of the voting interests of the investee. This presumption may be overcome based on specific facts and circumstances that demonstrate that the ability to exercise significant influence is restricted. The Company applies the equity method to investments in common stock and to other investments in entities that have risk and reward characteristics that are substantially similar to an investment in the investee’s common stock.

In applying the equity method, the Company records the investment at cost unless the initial recognition is the result of the deconsolidation of a subsidiary, in which case it is recorded at fair value. The Company subsequently increases or decreases the carrying amount of the investment by its proportionate share of the net earnings or losses and other comprehensive income of the investee based on the Company’s percentage of common stock ownership during the respective reporting period. Payments to investees such as additional investments, loans and expenses incurred on behalf of investees, as well as payments from investees such as

## BRIDGEBIO PHARMA LLC

### Notes to Combined and Consolidated Financial Statements

dividends, distributions and repayments of loans are recorded as adjustments in the carrying value of the investment. In the event that net losses of the investee reduce the carrying amount to zero, additional net losses may be recorded if the Company has a cost method investment in the investee, has guaranteed obligations of the investee, or is otherwise committed to provide further financial support for the investee.

The Company utilizes the cost method to account for investments when it does not have significant influence over the investee. Under the cost method, the Company records the investment at cost unless the initial recognition is the result of the deconsolidation of a subsidiary, in which case it is recorded at fair value. The Company recognizes income for any dividends declared from the distribution of the investee's earnings.

As of December 31, 2018, the Company has an equity and cost method investment in PellePharm, which is presented in the combined and consolidated financial statements in a single line item titled "PellePharm Investment." Refer to Note 8 for further discussion on the PellePharm investment.

Under the equity and cost method of accounting, the Company's equity and cost method investments in PellePharm are reviewed for indicators of impairment at each reporting period and are written down to fair value if there is evidence of a loss in value that is other-than-temporary. Factors that may be indicative of an impairment include a series of operating losses of an investee, the absence of an ability to recover the carrying amount of the investment, the inability of the investee to sustain an earnings capacity and a current fair value of an investment that is less than its carrying amount. Indicators that a decline in value may be other-than-temporary include the length of time and the extent to which the estimated fair value or market value has been below the carrying value, the financial condition and the near-term prospects of the investee, the intent and ability of the Company to retain its investment in the investee for a period of time sufficient to allow for any anticipated recovery in market value and general market conditions. The estimation of fair value and whether an other-than-temporary impairment has occurred requires the application of significant judgment and future results may vary from current assumptions. No impairment charge was recognized during the years ended December 31, 2017 and 2018 related to the PellePharm investment.

#### ***Use of Estimates***

The preparation of combined and consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities and disclosure of contingent assets and liabilities at the date of the combined and consolidated financial statements, and the reported amounts of expenses during the reporting period. Significant estimates and assumptions made in the accompanying combined and consolidated financial statements include, but are not limited to, the fair value of the redeemable convertible preferred units, the fair value of the redeemable founder units, the fair value of the redeemable common units, the fair value of the LEO Call Option liability, the fair value of the redeemable convertible preferred unit tranche liability, the valuation of equity-based awards, the valuation of intangible assets, the valuation of equity and cost method investments, income tax uncertainties and accruals for research and development activities. The Company bases its estimates on historical experience and on various other assumptions that are believed to be reasonable. Actual results may differ from those estimates or assumptions.

#### ***Segments***

The Company has determined that its chief executive officer is the chief operating decision maker ("CODM"). The Company operates and manages the business as one reporting and one operating segment, which is the business of identifying and advancing transformative medicines to treat patients. The Company's CODM reviews financial information on an aggregate basis for purposes of allocating resources and evaluating financial performance. All of the Company's assets are located in the United States.

**BRIDGEBIO PHARMA LLC****Notes to Combined and Consolidated Financial Statements*****Concentration of Credit Risk and Other Risks and Uncertainties***

Financial instruments that subject the Company to significant concentrations of credit risk consist primarily of cash, cash equivalents and restricted cash. The Company's cash, cash equivalents and restricted cash are held in financial institutions in the United States. Amounts on deposit may at times exceed federally insured limits. Management believes that the financial institutions are financially sound and, accordingly, minimal credit risk exists with respect to the financial institutions.

The Company is subject to certain risks and uncertainties and believes that changes in any of the following areas could have a material adverse effect on future financial position or results of operations: ability to obtain future financing; regulatory approval and market acceptance of, and reimbursement for, product candidates; performance of third-party clinical research organizations and manufacturers upon which the Company relies; development of sales channels; protection of the Company's intellectual property; litigation or claims against the Company based on intellectual property, patent, product, regulatory or other factors; and the Company's ability to attract and retain employees necessary to support its growth.

The Company is dependent on third-party manufacturers to supply products for research and development activities in its programs. In particular, the Company relies and expects to continue to rely on a small number of manufacturers to supply it with its requirements for the active pharmaceutical ingredients and formulated drugs related to these programs. These programs could be adversely affected by a significant interruption in the supply of active pharmaceutical ingredients and formulated drugs.

***Cash, Cash Equivalents and Restricted Cash***

The Company considers all highly liquid investments purchased with original maturities of three months or less from the purchase date to be cash equivalents. Cash equivalents consist of amounts invested in money market accounts.

As of December 31, 2017 and 2018, the Company had restricted cash of \$0.4 million and \$0.2 million, of which \$0.2 million at each period end relates to collateral for an operating lease entered into in 2017. Restricted cash is classified in other assets in the accompanying combined and consolidated balance sheets.

The following table provides a reconciliation of cash, cash equivalents and restricted cash reported within the combined and consolidated balance sheets that sum to the total of the amounts shown in the combined and consolidated statements of cash flows:

	<u>December 31,</u>	
	<u>2017</u>	<u>2018</u>
	(in thousands)	
Cash and cash equivalents	\$91,995	\$436,086
Restricted cash	381	159
Total cash, cash equivalents and restricted cash shown in the combined and consolidated statements of cash flows	<u>\$92,376</u>	<u>\$436,245</u>

As of December 31, 2017 and 2018, total cash and cash equivalents held by BridgeBio is \$74.5 million and \$238.7 million. The remaining cash and cash equivalents were held by the Company's wholly-owned subsidiaries and controlled entities and these funds are designated for specific entity usage, except in limited circumstances.

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**Notes to Combined and Consolidated Financial Statements**

***Fair Value Measurements***

Assets and liabilities recorded at fair value on a recurring basis in the balance sheets are categorized based upon the level of judgment associated with the inputs used to measure their fair values. Fair value is defined as the exchange price that would be received for an asset or an exit price that would be paid to transfer a liability in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. The authoritative guidance on fair value measurements establishes a three-tier fair value hierarchy for disclosure of fair value measurements as follows:

Level 1—Observable inputs such as unadjusted, quoted prices in active markets for identical assets or liabilities at the measurement date;

Level 2—Inputs (other than quoted prices included in Level 1) are either directly or indirectly observable for the asset or liability. These include quoted prices for similar assets or liabilities in active markets and quoted prices for identical or similar assets or liabilities in markets that are not active; and

Level 3—Unobservable inputs that are supported by little or no market activity and that are significant to the fair value of the assets or liabilities.

To the extent that the valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized in Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement.

The carrying amounts reflected in the accompanying combined and consolidated balance sheets for cash and cash equivalents, restricted cash, prepaid expenses and other current assets, accounts payable and accrued expenses approximate their fair values, due to their short-term nature.

The fair value of the Company's outstanding term loans with Hercules, Capital Inc. (see Note 10) is estimated using the net present value of the payments, discounted at an interest rate that is consistent with a market interest rate, which is a Level 2 input. The estimated fair value of the Company's outstanding term loans approximates the carrying amount, as the term loan bears a floating rate that approximates the market interest rate.

***Property and Equipment, net***

Property and equipment are stated at cost, net of accumulated depreciation and amortization. Depreciation and amortization of property and equipment is calculated using the straight-line method over the estimated useful lives of the respective assets. Maintenance and repairs that do not improve or extend the life of the assets are expensed when incurred. Upon sale or retirement of assets, the cost and accumulated depreciation and amortization are removed from the combined and consolidated balance sheet and any resulting gain or loss is reflected in the combined and consolidated statement of operations in the period realized.

The Company's estimated useful lives of its property and equipment are as follows:

Furniture and office equipment	3 - 5 years
Lab equipment	3 - 5 years
Leasehold improvements	Shorter of remaining lease term or estimated useful life

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**Notes to Combined and Consolidated Financial Statements**

***Intangible Asset***

The identifiable intangible asset consists of an assembled workforce acquired in an asset acquisition in 2016. This intangible asset was amortized on a straight-line basis over its estimated useful life of one year. The straight-line method of amortization represents the Company's best estimate of the distribution of the economic value of the identifiable intangible asset. The intangible asset is carried at cost less accumulated amortization. Amortization is included in research and development expenses. As of December 31, 2017, the assembled workforce intangible asset was fully amortized.

***Asset Acquisitions***

The Company measures and recognizes asset acquisitions that are not deemed to be business combinations based on the cost to acquire the assets, which includes transaction costs. Goodwill is not recognized in asset acquisitions. In an asset acquisition, the cost allocated to acquire in-process research and development ("IPR&D") with no alternative future use is charged to research and development expense at the acquisition date.

***Impairment of Long-Lived Assets***

Long-lived assets are reviewed for impairment annually or whenever events or changes in circumstances indicate that the carrying amount of an asset may not be recoverable. Recoverability is measured by comparison of the carrying amount of an asset group to the future net undiscounted cash flows that the assets are expected to generate. If the carrying amount of an asset group exceeds its estimated future cash flows, an impairment charge is recognized in the amount by which the carrying amount of the asset group exceeds the fair value of the asset group. There was no impairment of long-lived assets for any of the periods presented.

***Redeemable Convertible Series B Preferred Unit Tranche Liability***

The Company has accounted for the freestanding right that its Series B redeemable convertible preferred unit investors have to purchase additional shares of the Company's Series B redeemable convertible preferred units at a fixed price in subsequent closings as a tranche liability, which was recognized at fair value upon issuance. The tranche liability is subject to remeasurement to fair value at each combined and consolidated balance sheet date. Any change in the fair value of the tranche liability is recognized as a component of other income (expense) in the combined and consolidated statements of operations. In April 2017, the tranche liability was settled upon the closing of the Company's Series B redeemable convertible preferred unit financing.

***LEO Call Option Liability***

On November 19, 2018, PellePharm entered into the LEO Agreement with LEO pursuant to which LEO was granted an exclusive, irrevocable option to acquire PellePharm. The Company has accounted for this option held by LEO to acquire its PellePharm ownership interest (the "LEO Call Option") as a current liability as BridgeBio has the obligation to sell its PellePharm shares to LEO at a pre-determined price if the option is exercised. The LEO Call Option was recorded at fair value upon execution of the LEO Agreement. The LEO Call Option is subject to remeasurement to fair value at each combined and consolidated balance sheet date until the LEO Call Option is either exercised or expires as it does not qualify for equity classification. Any change in the fair value of the LEO Call Option is recognized as a component of other income (expense) in the combined and consolidated statements of operations. Refer to Note 3 and Note 6 for further discussion.

***Eidos Embedded Derivative Liability***

Eidos determined that the automatic redemption of a convertible promissory note issued in March 2018 into new shares of Eidos preferred stock at 70% of the issuance price upon the closing of a qualified financing is an

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**Notes to Combined and Consolidated Financial Statements**

embedded derivative liability. The embedded derivative liability balance was settled upon the redemption of the convertible promissory notes into redeemable convertible preferred stock of Eidos in March 2018. Refer to Note 3 and Note 6 for further discussion.

***Eidos Redeemable Convertible Preferred Stock Tranche Liability***

Eidos determined that its obligation to issue additional shares of Eidos redeemable convertible preferred stock upon the achievement of certain milestones or at the option of the holders represents a freestanding financial instrument (the “Eidos Tranche Liability”). The instrument was subject to remeasurement at each combined and consolidated balance sheet date with changes in fair value being recognized as a component of other income (expense) in the statements of operations. The Eidos Tranche Liability balance was reclassified to redeemable convertible preferred stock upon the issuance of the additional shares in May 2018. Refer to Note 3 and Note 6 for further discussion.

***Eidos Redeemable Convertible Preferred Stock Warrant Liability***

Eidos’ redeemable convertible preferred stock warrants (the “Eidos Warrants”) required liability classification (the “Eidos Warrant Liability”) as the underlying Eidos preferred stock was deemed redeemable. Upon initial recognition, the Eidos Warrants were recorded at the estimated fair value. The Eidos Warrants were subject to remeasurement at each combined and consolidated balance sheet date, with changes in fair value being recognized as a component of other income (expense). The Eidos Warrant Liability was adjusted for changes in fair value until the completion of the Eidos initial public offering (the “Eidos IPO”), at which time the Eidos Warrants were net exercised into shares of Eidos common stock and the related Eidos Warrant Liability was reclassified to Eidos common stock and additional paid-in capital. Refer to Note 3 and Note 6 for further discussion.

***Classification of the Redeemable Units and Management Incentive Units***

The Company has classified all of its outstanding redeemable convertible Series D preferred units (the “Series D Preferred Units”), redeemable convertible Series C preferred units (the “Series C Preferred Units”), redeemable convertible Series B preferred units (the “Series B Preferred Units”) and redeemable convertible Series A preferred units (the “Series A Preferred Units” or, collectively, the “Preferred Units”), as well as its redeemable founder units (the “Founder Units”), redeemable common units (the “Common Units”) and management incentive units (the “Management Incentive Units”) outside of members’ deficit in the accompanying combined and consolidated balance sheets because these units contain certain redemption features that are not solely within the control of the Company.

***Research and Development Expenses***

Research and development costs are expensed as incurred. Research and development expenses consist of salaries, benefits and other personnel related costs including equity-based compensation expense, laboratory supplies, preclinical studies, clinical trials and related clinical manufacturing costs, costs related to manufacturing preparations, fees paid to other entities to conduct certain research and development activities on the Company’s behalf and allocated facility and other related costs. Non-refundable advance payments for goods or services that will be used or rendered for future research and development activities are deferred and capitalized as prepaid expenses until the related goods are delivered or services are performed.

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***Accrued Research and Development Liabilities***

The Company records accrued liabilities for estimated costs of research and development activities conducted by third-party service providers, which include the conduct of preclinical studies and clinical trials, and contract manufacturing activities. The Company records the estimated costs of research and development activities based upon the estimated amount of services provided but not yet invoiced and includes these costs in accrued research and development liabilities in the combined and consolidated balance sheets and within research and development expense in the combined and consolidated statements of operations. These costs are a significant component of the Company's research and development expenses.

The Company accrues for these costs based on factors such as estimates of the amount of work completed through discussions with internal personnel and external service providers as to the progress or stage of completion of the services and in accordance with agreements established with its third-party service providers for such services. The Company makes significant judgments and estimates in determining the accrued research and development liabilities balance in each reporting period. As actual costs become known, the Company adjusts its accrued estimates. Although the Company does not expect its estimates to be materially different from amounts actually incurred, the status and timing of services performed, the number of patients enrolled in clinical trials and the rate of patient enrollment may vary from its estimates and could result in its reporting amounts that are too high or too low in any particular period. The Company's accrued expenses are dependent, in part, upon the receipt of timely and accurate reporting from clinical research organizations and other third-party service providers. The Company records advance payments to service providers as prepaid assets, which are expensed as the contracted services are performed. To date, there have been no material differences between the Company's accrued costs and actual costs.

***Equity-Based Compensation***

Equity-based compensation is measured at the grant date for all equity-based awards made to employees and non-employees based on the fair value of the awards and is recognized as expense on a straight-line basis over the requisite service period, which is generally the vesting period. The Company has elected to recognize the actual forfeitures by reducing the equity-based compensation in the same period as the forfeitures occur. The method for how fair value is determined for the awards is described in Note 14. Equity-based compensation for awards made to non-employees was measured as per ASC 505-50 until the Company early adopted Accounting Standards Update ("ASU") 2018-07 *Compensation-Stock Compensation (Topic 718)* on January 1, 2017. The Company remeasured its equity-classified non-employee awards for which a measurement date had not been established at their adoption date fair-value based measurement (January 1, 2017) and determined there was no cumulative-effect adjustment to opening accumulated deficit. Subsequent to the adoption of ASU 2018-07, the Company accounts for non-employee awards similar to employee awards.

BridgeBio has granted Management Incentive Units and Common Units to employees and non-employees. These awards generally have only a service condition and vest over a period of up to five years. The awards have accelerated vesting upon a fundamental transaction (a "Fundamental Transaction") which is defined as (i) a merger, recapitalization or other business combination, (ii) a sale, transfer, exclusive license or disposition of the Company or (iii) a final liquidation, dissolution, winding-up or termination of the Company. BridgeBio's consolidated entities have granted stock options that are exercisable in the underlying entity's equity and have issued restricted stock awards in the underlying entity's equity to employees and non-employees. None of the awards issued by the consolidated entities are issued for BridgeBio members' capital. These awards generally have only a service condition and generally vest over a period of up to four years.

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The Company classifies equity-based compensation in its combined and consolidated statements of operations in the same manner in which the award recipients' payroll costs are classified or in which the award recipients' service payments are classified.

***Income Taxes***

Since BridgeBio is a "pass-through" entity under the United States Internal Revenue Code of 1986, as amended ("the Internal Revenue Code"), the members of the Company are taxed directly on their respective ownership interests in consolidated income and, therefore, no provision or liability for federal income tax has been included in the accompanying combined and consolidated financial statements.

For BridgeBio's consolidated entities, income taxes are accounted for under the asset and liability method. Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax base and net operating loss and tax credit carryforwards. Deferred tax assets and liabilities are determined based upon the difference between the consolidated financial statement carrying amounts and the tax basis of assets and liabilities and are measured using the enacted tax rate expected to apply to taxable income in the years in which the differences are expected to be reversed. Deferred tax assets are reduced by a valuation allowance if it is more likely than not that some portion or all of the deferred tax asset will not be realized.

BridgeBio's consolidated entities recognize uncertain income tax positions at the largest amount that is more likely than not to be sustained upon audit by the relevant taxing authority. Changes in recognition or measurement are reflected in the period in which judgment occurs. BridgeBio's consolidated entities' policy is to recognize interest and penalties related to the underpayment of income taxes as a component of the provision for income taxes. To date, there have been no interest or penalties recorded in relation to unrecognized tax benefits.

***Defined Contribution Plan***

BridgeBio has a defined contribution retirement savings plan under Section 401(k) of the Internal Revenue Code. This plan allows eligible employees to defer a portion of their annual compensation on a pre-tax basis. BridgeBio is authorized to make matching contributions, but has not made such contributions for the years ended December 31, 2017 and 2018.

***Comprehensive Loss***

Comprehensive loss is defined as a change in equity of a business enterprise during a period, resulting from transactions from non-owner sources. To date, the Company has not had any transactions that are required to be reported in comprehensive loss other than the net loss incurred from operations. Thus, comprehensive loss is the same as net loss for the periods presented.

***Emerging Growth Company Status***

The Company is an emerging growth company, as defined in the Jumpstart Our Business Startups Act of 2012 (the "JOBS Act"). Under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards issued subsequent to the enactment of the JOBS Act until such time as those standards apply to private companies. The Company has elected to use this extended transition period for complying with new or revised accounting standards that have different effective dates for public and private companies until the earlier of the date that it (i) is no longer an emerging growth company or (ii) affirmatively and irrevocably opts



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out of the extended transition period provided in the JOBS Act. As a result, these combined and consolidated financial statements may not be comparable to companies that comply with the new or revised accounting pronouncements as of public company effective dates.

As described in “Recently Adopted Accounting Pronouncements” below, the Company early adopted multiple accounting standards, as the JOBS Act does not preclude an emerging growth company from adopting a new or revised accounting standard earlier than the time that such standard applies to private companies. The Company expects to use the extended transition period for any other new or revised accounting standards during the period in which it remains an emerging growth company.

***Net Loss per Unit***

The holders of the Company’s Preferred Units are entitled to receive distributions, including cumulative returns on their units outstanding, prior and in preference to any distributions on any of the Company’s Founder Units and Common Units, which are also entitled to cumulative returns. Cumulative returns for Preferred Units, Common Units and Founder Units no longer accumulate subsequent to the Series D Preferred Unit financing described in Note 13. For the years ended December 31, 2017 and 2018, the Company determined that its Founder Units and Common Units are common stock equivalents.

The numerator for basic net loss per unit is calculated as the net loss adjusted for cumulative returns for Preferred Units for the year ended December 31, 2017 and through the November 2018 Series D issuance date for the year ended December 31, 2018. The denominator for basic net loss per unit is determined as the weighted-average number of Founder Units and Common Units outstanding during the years ended December 31, 2017 and 2018.

In accordance with the two-class method, undistributed earnings are allocated to all participating securities. For the years ended December 31, 2017 and 2018, the Company considers its Preferred Units to be participating securities as they are entitled to participate in undistributed earnings along with Founder Unit and Common Unit members. Management Incentive Units are not participating securities.

Basic net loss per unit is the same as diluted net loss per unit as the inclusion of all potentially dilutive Preferred Units, unvested Common Units, and Management Incentive Units would have been anti-dilutive.

***Pro Forma Net Loss per Share***

Prior to the completion of its initial public offering (“IPO”), the Company will complete a series of transactions (the “Reorganization”) pursuant to which BridgeBio will become a wholly-owned subsidiary of BridgeBio Pharma, Inc. (the “Corporation”), a newly formed Delaware corporation.

The unaudited pro forma basic and diluted net loss per share attributable to common stockholders for the year ended December 31, 2018 has been prepared to give effect to the exchange of all Preferred Units, Founder Units, vested Common Units and vested Management Incentive Units (collectively, the “Outstanding Units”) of BridgeBio into shares of common stock of the Corporation upon the Reorganization as if the Reorganization had occurred on the later of beginning of period or the issuance date of the Outstanding Units.

***Recently Adopted Accounting Pronouncements***

***ASU 2015-17 Income Taxes (Topic 740)***. In November 2015, the FASB issued *ASU 2015-17 Income Taxes (Topic 740): Balance Sheet Classification of Deferred Taxes (“ASU 2015-17”)*, which simplifies the presentation

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of deferred taxes in a classified balance sheet by eliminating the requirement to separate deferred income tax liabilities and assets into current and noncurrent amounts. Instead, ASU 2015-17 requires that all deferred tax liabilities and assets be shown as noncurrent in a classified balance sheet. ASU 2015-17 is effective for fiscal years beginning after December 15, 2017 and may be applied either prospectively or retrospectively to all periods presented. The Company adopted this guidance on January 1, 2018 retrospective to all periods presented, which did not result in a restatement of its combined and consolidated balance sheet as of December 31, 2017.

**ASU 2016-18 Statement of Cash Flows (Topic 230).** In November 2016, the FASB issued *ASU 2016-18 Statement of Cash Flows (Topic 230) Restricted Cash—a consensus of the FASB Emerging Issues Task Force (“ASU 2016-18”)*, which requires that amounts generally described as restricted cash and restricted cash equivalents should be included with cash and cash equivalents when reconciling the beginning-of-period and end-of-period total amounts shown in the statement of cash flows. ASU 2016-18 is effective for fiscal years beginning after December 15, 2018 and should be applied using a retrospective transition method to each period presented. Early adoption is permitted. The Company early adopted this guidance on January 1, 2017. The adoption of this guidance did not materially impact the Company’s combined and consolidated financial statements.

**ASU 2016-09 Stock Compensation—Improvements to Employee Share-Based Payment Accounting.** In March 2016, the FASB issued *ASU 2016-09, Stock Compensation—Improvements to Employee Share-Based Payment Accounting (“ASU 2016-09”)*. ASU 2016-09 was issued to simplify accounting guidance by identifying, evaluating, and improving areas for which cost and complexity can be reduced while maintaining or improving the usefulness of the information provided to users of financial statements. The areas affected by ASU 2016-09 include accounting for income taxes, classification of excess tax benefits in the statement of cash flows, minimum statutory tax withholding requirements, and classification of employee taxes paid in the statement of cash flows when an employer withholds shares for tax-withholding purposes. In addition, under this guidance, an entity can make an accounting policy election to either estimate the number of awards that are expected to vest or account for forfeitures when they occur. ASU 2016-09 is effective for fiscal years beginning after December 15, 2017 and should be applied using a retrospective transition method to each period presented. Early adoption is permitted. Upon early adoption of this guidance on January 1, 2016, the Company changed its policy to account for forfeitures as they occur. The adoption of this guidance did not materially impact the Company’s combined and consolidated financial statements.

**ASU 2017-01 Business Combinations (Topic 805).** In January 2017, the FASB issued *ASU 2017-01, Business Combinations (Topic 805): Clarifying the Definition of a Business (“ASU 2017-01”)*. This ASU provides guidance to evaluate whether transactions should be accounted for as acquisitions (or disposals) of assets or businesses. If substantially all of the fair value of the gross assets acquired (or disposed of) is concentrated in a single asset or a group of similar assets, the assets acquired (or disposed of) are not considered a business. This guidance is effective for annual periods beginning after December 15, 2018, with early adoption permitted. The Company early adopted this guidance. As a result of applying this guidance, the Company accounted for its acquisition of PellePharm, Inc. in 2016 as an asset acquisition (see Note 6) and other asset acquisitions (see Note 5).

**ASU 2017-09 Compensation—Stock Compensation (Topic 718).** In May 2017, the FASB issued *ASU 2017-09, Compensation—Stock Compensation (Topic 718): Scope of Modification Accounting (“ASU 2017-09”)*. This ASU provides guidance about which changes to the terms or conditions of a share-based payment award require an entity to apply modification accounting in Topic 718. The guidance is effective for annual and interim periods beginning after December 15, 2017, with early adoption permitted. The Company early adopted this guidance on January 1, 2016. The adoption of this guidance did not have a material impact on the Company’s combined and consolidated financial position, results of operations and disclosures.

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**ASU 2017-11 Earnings Per Share (Topic 260) Distinguishing Liabilities from Equity (Topic 480) Derivatives and Hedging (Topic 815).** In July 2017, FASB issued a two-part ASU 2017-11, I. *Accounting for Certain Financial Instruments with Down Round Features*, and II. *Replacement of the Indefinite Deferral for Mandatorily Redeemable Financial Instruments of Certain Nonpublic Entities and Certain Mandatorily Redeemable Noncontrolling Interests with a Scope Exception* (“ASU 2017-11”). ASU 2017-11 amends guidance in ASC 260, *Earnings Per Share*, ASC 480, *Distinguishing Liabilities from Equity*, and ASC 815, *Derivatives and Hedging*. Part I of this ASU changes the classification analysis of certain equity-linked financial instruments (or embedded features) with down round features and clarifies existing disclosure requirements. Part II does not have an accounting effect. The standard is effective for fiscal years beginning after December 15, 2019 with early adoption permitted. The Company early adopted this guidance effective January 1, 2016. The adoption of this guidance did not have a material impact on the Company’s combined and consolidated financial position, results of operations and disclosures.

**ASU 2018-07 Compensation-Stock Compensation (Topic 718).** In June 2018, the FASB issued ASU No. 2018-07, *Compensation-Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting* (“ASU 2018-07”). ASU 2018-07 is intended to reduce the cost and complexity and to improve financial reporting for nonemployee share-based payments. The ASU expands the scope of Topic 718, (which currently only includes share-based payments to employees) to include share-based payments issued to non-employees for goods or services. Consequently, the accounting for share-based payments to non-employees and employees will be substantially aligned. The standard is effective for fiscal years beginning after December 15, 2019. Early adoption is permitted, but no earlier than a company’s adoption date of Topic 606. The Company early adopted this guidance effective January 1, 2017. The adoption of this guidance did not have a material impact on the Company’s financial position, results of operations and disclosures.

**Recently Issued Accounting Pronouncements Not Yet Adopted**

**ASU 2016-02 Leases (Topic 842).** In February 2016, the FASB issued ASU 2016-02, *Leases* (“ASU 2016-02”), which for operating leases requires the lessee to recognize a right-of-use asset and a lease liability, initially measured at the present value of the lease payments, in its balance sheet. The guidance also requires a lessee to recognize single lease costs, calculated so that the cost of the lease is allocated over the lease term, on a generally straight-line basis. A modified retrospective transition approach is required for leases existing at, or entered into after, the beginning of the earliest comparative period presented in the financial statements, including a number of optional practical expedients that entities may elect to apply. ASU 2016-02 is effective for fiscal years beginning after December 15, 2019 and interim periods within fiscal years beginning after December 15, 2020, with early adoption permitted. The Company has not determined the potential effects of this ASU on its combined and consolidated financial statements.

**ASU 2016-15 Statement of Cash Flows (Topic 230) Classification of Certain Cash Receipts and Cash Payments.** In August 2016, the FASB issued ASU 2016-15, *Statement of Cash Flows (Topic 230): Classification of Certain Cash Receipts and Cash Payments* (“ASU 2016-15”). The areas affected by ASU 2016-15 are debt prepayment and debt extinguishment costs, settlement of zero-coupon debt instruments or other debt instruments with coupon interest rates that are insignificant in relation to the effective interest rate of the borrowing, contingent consideration payments made after a business combination, proceeds from the settlement of insurance claims, proceeds from the settlement of corporate-owned life insurance policies (including bank-owned life insurance policies), distributions received from equity method investees, beneficial interests in securitization transactions and separately identifiable cash flows and application of the predominance principle. Specifically, under this guidance, cash payments for debt prepayment or debt extinguishment costs will be classified as cash outflows for financing activities. The amendments in ASU 2016-15 are effective for fiscal years beginning after

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December 15, 2018 and interim periods within fiscal years beginning after December 15, 2019. Early adoption is permitted, including adoption in an interim period. If an entity early adopts the amendments in an interim period, any adjustments should be reflected as of the beginning of the fiscal year that includes that interim period. The amendments in ASU 2016-15 will be applied using a retrospective transition method to each period presented. The adoption of ASU 2016-15 is not expected to materially impact the Company's combined and consolidated financial statements.

**ASU 2018-13, Fair Value Measurement – Disclosure Framework (Topic 820).** In August 2018, the FASB issued *ASU 2018-13, Fair Value Measurement - Disclosure Framework (Topic 820)* ("ASU 2018-13"). The updated guidance improves the disclosure requirements on fair value measurements and is effective for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2019. Early adoption is permitted upon issuance of the standard for disclosures modified or removed with a delay of adoption of the additional disclosures until their effective date. The adoption of ASU 2018-13 is not expected to materially impact the Company's combined and consolidated financial statements.

### 3. Fair Value Measurement

The following table presents information about the Company's financial assets and liabilities that are measured at fair value on a recurring basis and indicates the fair value hierarchy of the valuation:

	December 31, 2017			
	Total	Level 1	Level 2	Level 3
(in thousands)				
Assets:				
Money market funds	\$ 200	\$ 200	\$ —	\$ —
Total assets	<u>\$ 200</u>	<u>\$ 200</u>	<u>\$ —</u>	<u>\$ —</u>
December 31, 2018				
	Total	Level 1	Level 2	Level 3
(in thousands)				
Assets:				
Money market funds	\$395,780	\$395,780	\$ —	\$ —
Total assets	<u>\$395,780</u>	<u>\$395,780</u>	<u>\$ —</u>	<u>\$ —</u>
Liabilities:				
LEO Call Option liability	\$ 3,009	\$ —	\$ —	\$3,009
Total liabilities	<u>\$ 3,009</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$3,009</u>

There were no transfers between Level 1, Level 2 or Level 3 during the years ended December 31, 2017 and 2018.

**BRIDGEBIO PHARMA LLC****Notes to Combined and Consolidated Financial Statements****Series B Preferred Unit Tranche Liability**

The Company's Series B Preferred Unit tranche liability (see Note 13) contains unobservable inputs that reflect management's own assumptions in which there is little, if any, market activity for at the measurement date. Accordingly, the Company estimated the fair value of the tranche liability using a Black-Scholes option pricing model with the following assumptions:

	<u>Year Ended December 31, 2017</u>
Expected term (in years)	0.3
Expected volatility	43.1%
Risk-free interest rate	0.51%
Dividend yield	—

The following table sets forth a summary of the changes in the estimated fair value of the Company's Series B Preferred Units tranche liability:

	<u>Total (in thousands)</u>
Balance as of January 1, 2017	\$ 183
Settlement of the Series B Preferred Units tranche liability in 2017	(183)
Balance as of December 31, 2017	<u>\$ —</u>

**LEO Call Option Liability**

The valuation of the LEO Call Option (see Note 6) contains unobservable inputs that reflect the management's own assumptions for which there is little, if any, market activity at the measurement date. Accordingly, the LEO Call Option liability is remeasured to fair value on a recurring basis using unobservable inputs that are classified as Level 3 inputs.

The Company estimated the fair value of the LEO Call Option by estimating the fair value of various clinical, regulatory, and sales milestones based on the estimated risk and probability of achievement of each milestone, and allocated the value using a Black-Scholes option pricing model with the following assumptions:

	<u>Year Ended December 31, 2018</u>
Probability of milestone achievement	12.0%-84.0%
Discount rate	2.7%-11.0%
Expected term (in years)	0.58-4.38
Expected volatility	67.0%-79.0%
Risk-free interest rate	2.51%-2.78%
Dividend yield	—

**BRIDGEBIO PHARMA LLC****Notes to Combined and Consolidated Financial Statements**

The following table sets forth a summary of the changes in the estimated fair value of the LEO Call Option:

	<b>Total (in thousands)</b>
Balance as of January 1, 2018	\$ —
Initial fair value upon execution of the Leo Agreement in November 2018	1,879
Change in fair value upon remeasurement recognized in other income (expense)	1,130
Balance as of December 31, 2018	<u>\$ 3,009</u>

**Eidos Embedded Derivative Liability**

The convertible promissory notes issued by the Company to a minority stockholder of Eidos in March 2018 for \$1.0 million contain a redemption feature that was determined to be an embedded derivative requiring bifurcation and separate accounting on issuance (see Note 6). The fair value of the derivative was determined using an income approach that identified the cash flows utilizing a “with-and-without” valuation methodology. The inputs used to determine the estimated fair value of the derivative instrument were based primarily on the probability and timing of an underlying event triggering the redemption.

The following table sets forth a summary of the changes in the fair value of the Eidos embedded derivative liability:

	<b>Total (in thousands)</b>
Balance as of January 1, 2018	\$ —
Initial fair value of the Eidos embedded derivative liability issued with the convertible promissory notes	642
Change in fair value upon remeasurement recognized in other income (expense)	—
Settlement of the Eidos embedded derivative liability	(642)
Balance as of December 31, 2018	<u>\$ —</u>

The embedded derivative liability balance was settled upon the redemption of convertible promissory notes into redeemable convertible preferred stock of Eidos in March 2018.

**Eidos Redeemable Convertible Preferred Stock Tranche Liability**

In March 2018, Eidos entered into the Eidos Series B Preferred Stock Purchase Agreement for issuance of shares of Eidos Series B redeemable convertible preferred stock in two closings (see Note 6). The Eidos Tranche Liability related to the obligation of Eidos to issue additional shares. The Eidos Tranche Liability was recorded at fair value and the change in fair value upon remeasurement is recognized as a component of other income (expense) in the combined and consolidated statements of operations. Eidos estimated the initial fair value using a Black-Scholes option pricing model using the following assumptions: a term of 0.08 years, a risk-free rate of 1.63%, a volatility of 36.4%, and a dividend yield of 0.0%.

Upon the second closing in May 2018, the related Eidos Tranche Liability was remeasured using a probability-weighted expected return method (PWERM). The PWERM included probabilities of three scenarios, including an Eidos initial public offering occurring in June 2018. The scenarios were weighted based on Eidos’ estimate of each event occurring in deriving the estimated fair value.

**BRIDGEBIO PHARMA LLC****Notes to Combined and Consolidated Financial Statements**

The following table sets forth a summary of the changes in the fair value of the Eidos Tranche Liability:

	<b>Total</b> <b>(in thousands)</b>
Balance as of January 1, 2018	\$ —
Initial fair value of the Eidos Tranche Liability on issuance of Eidos Series B redeemable convertible preferred stock	49
Change in fair value upon remeasurement recognized in other income (expense)	467
Settlement of the Eidos Tranche Liability	(516)
Balance as of December 31, 2018	<u>\$ —</u>

The Eidos Tranche Liability balance was reclassified to redeemable convertible preferred stock upon the second closing in May 2018.

**Eidos Redeemable Convertible Preferred Stock Warrant Liability**

The fair value of the Eidos Warrant Liability (see Note 6) is based on significant inputs not observed in the market and thus represents a Level 3 measurement. Eidos estimated the fair value of the Eidos Warrant Liability using a PWERM that included probabilities of three scenarios, including an Eidos initial public offering occurring in June 2018. The scenarios were weighted based on Eidos' estimate of each event occurring in deriving the estimated fair value. The Eidos Warrant Liability was remeasured upon the Eidos IPO using the value of the underlying share based on the Eidos IPO price less the warrant strike price.

The following table sets forth a summary of the changes in the fair value of the Eidos Warrant Liability:

	<b>Total</b> <b>(in thousands)</b>
Balance as of January 1, 2018	\$ —
Initial fair value of the Eidos Warrant Liability	88
Change in fair value upon remeasurement recognized in other income (expense)	263
Settlement of the Eidos Warrant Liability on Eidos IPO	(351)
Balance as of December 31, 2018	<u>\$ —</u>

In the combined and consolidated financial statements, the issuance of redeemable convertible preferred stock of Eidos to third parties is presented as the issuance of redeemable noncontrolling interest. Upon the Eidos IPO in June 2018, all outstanding shares of Eidos' redeemable convertible preferred stock were converted into shares of common stock of Eidos. This transaction is reflected as conversion of redeemable noncontrolling interest into noncontrolling interest. The net exercise of the Eidos Warrants upon the Eidos IPO is presented as the issuance of noncontrolling interest. See Note 7 for details.

## BRIDGEBIO PHARMA LLC

## Notes to Combined and Consolidated Financial Statements

## 4. Balance Sheet Components

*Prepaid Expenses and Other Current Assets*

Prepaid expenses and other current assets consist of the following:

	December 31,	
	2017	2018
	(in thousands)	
Prepaid clinical and research related expenses	\$4,247	\$7,087
Other current assets	889	2,050
Total prepaid expenses and other current assets	<u>\$5,136</u>	<u>\$9,137</u>

*Property and Equipment, Net*

Property and equipment, net, consists of the following:

	December 31,	
	2017	2018
	(in thousands)	
Furniture and office equipment	\$ 89	\$ 635
Lab equipment	298	1,020
Leasehold improvements	80	145
Construction in progress	—	6
Total property and equipment, gross	467	1,806
Less: accumulated depreciation and amortization	(27)	(231)
Total property and equipment, net	<u>\$440</u>	<u>\$1,575</u>

Depreciation and amortization expense for property and equipment was \$27,000 and \$0.2 million for the years ended December 31, 2017 and 2018.

*Other Accrued Liabilities*

Other accrued liabilities consist of the following:

	December 31,	
	2017	2018
	(in thousands)	
Accrued professional services	\$ 318	\$ 772
Accrued other liabilities	795	1,328
Total other accrued liabilities	<u>\$1,113</u>	<u>\$2,100</u>

## 5. Asset Acquisitions

*Origin Biosciences, Inc. ("Origin") Asset Acquisition*

In June 2018, Origin entered into an Asset Purchase Agreement with Alexion Pharma Holding Unlimited Company ("Alexion") to acquire intellectually property rights, including patent rights, know-how, and contracts, related to the ALXN1101 molecule. As consideration, Origin made an upfront cash payment of \$1.0 million. There were no material direct transaction costs related to the transaction.



**BRIDGEBIO PHARMA LLC****Notes to Combined and Consolidated Financial Statements**

Origin accounted for the transaction as an asset acquisition as substantially all of the estimated fair value of the gross assets acquired was concentrated in a single identified asset, IPR&D, thus satisfying the requirements of the screen test in ASU 2017-01. The assets acquired and liabilities assumed in the transaction were measured based on their fair values. The fair value of the IPR&D acquired was \$1.0 million and was charged to research and development expense as it had no alternative future use at the time of the acquisition. If certain substantive milestones are met in the future, Origin could be required to pay up to \$18.8 million if Origin receives a priority review voucher from the Food and Drug Administration, \$3.0 million in regulatory milestone payments, \$17.0 million in sales milestone payments, and pay royalties of up to low double-digit percentages on future net sales, if any.

***QED Therapeutics, Inc. (“QED”) Asset Acquisition***

In January 2018, QED entered into a License Agreement with Novartis International Pharmaceutical, Inc. (“Novartis”), pursuant to which QED acquired certain intellectual property rights, including patents and know-how, related to BBP-831 for the treatment of patients with FGFR-driven diseases. As consideration for the License Agreement, QED made an upfront cash payment of \$15.0 million and issued 2,941,176 shares of QED Series A Preferred Stock to Novartis. There were no material direct transaction costs related to the transaction. The fair value of the QED Series A Preferred Stock was valued by a third-party specialist at \$0.59 per share or a total fair value of shares issued of \$1.7 million.

QED accounted for the transaction as an asset acquisition as substantially all of the estimated fair value of the gross assets acquired was concentrated in a single identified asset, IPR&D, thus satisfying the requirements of the screen test in ASU 2017-01. The assets acquired and liabilities assumed in the transaction were measured based on their fair values. The fair value of the IPR&D acquired was \$16.7 million and was charged to research and development expense as it had no alternative future use at the time of the acquisition. If certain substantive milestones are met in the future, QED could be required to pay up to \$60.0 million in regulatory milestone payments, \$35.0 million in sales milestone payments, and pay royalties of up to low double-digit percentages on future net sales, if any.

***Phoenix Tissue Repair, Inc. (“PTR”) Asset Acquisition***

In July 2017, PTR entered into the Contribution Agreement and Asset Purchase Agreement with Shire Human Genetic Therapies, Inc. and its subsidiary Lotus Tissue Repair, Inc. to acquire the right, title, and interest in certain intellectual property, research program assets, and contracts relating to recombinant human collagen type VII. As consideration, PTR made an upfront cash payment of \$1.5 million and issued 10,019,900 shares of PTR common stock valued at a nominal fair value at issuance. There were no material direct transaction costs related to the transaction.

PTR accounted for the transaction as an asset acquisition as substantially all of the estimated fair value of the gross assets acquired was concentrated in a single identified asset, IPR&D, thus satisfying the requirements of the screen test in ASU 2017-01. The assets acquired and liabilities assumed in the transaction were measured based on their fair values. The fair value of the IPR&D acquired was \$1.5 million and was charged to research and development expense as it had no alternative future use at the time of the acquisition. If certain substantive milestones are met in the future, PTR could be required to pay up to \$27.0 million in regulatory milestone payments, \$60.0 million in sales milestone payments, and pay royalties of up to low single-digit percentages on future net sales, if any.

**BRIDGEBIO PHARMA LLC**

**Notes to Combined and Consolidated Financial Statements**

**6. Variable Interest Entities and Voting Interest Model**

The entities consolidated by BridgeBio are comprised of wholly-owned subsidiaries and partially-owned entities consolidated under the VOE model and VIEs for which BridgeBio is the primary beneficiary under the VIE model. The results of operations of the consolidated entities are included within the BridgeBio combined and consolidated financial statements for the years ended December 31, 2017 and 2018. As of December 31, 2017 and 2018, there were no significant restrictions on the VIE assets or liabilities. For VIEs, BridgeBio calculates the maximum exposure to loss to be equal to the amount invested in the equity of the VIE and the amount of outstanding convertible notes.

The below listed partially-owned entities were determined to be under BridgeBio's control as of December 31, 2018, with the exception of PellePharm, Inc. as discussed in Note 8. At each reporting period, the Company reassesses whether it has a majority voting interest for entities consolidated under the VOE model and whether it remains the primary beneficiary for VIEs consolidated under the VIE model.

***Eidos***

Eidos is a clinical stage biopharmaceutical company focused on the development of BBP-265 to address the large and growing unmet need in diseases caused by transthyretin amyloidosis. In April 2016, the Company initially invested \$1.0 million and determined that its investment in Eidos represented a variable interest. At that time, Eidos did not have sufficient resources to carry out its principal activities without additional financial support. BridgeBio was determined to be the primary beneficiary of Eidos as it controlled the activities that most significantly impacted Eidos' economic performance, controlled the most significant decisions affecting Eidos through its representation within management and Eidos' Board of Directors, and BridgeBio had a majority ownership interest.

In February 2018, BridgeBio entered into a note and warrant purchase agreement with Eidos, pursuant to which Eidos issued a convertible promissory note (the "Eidos Note") with the principal amount of \$10.0 million and a warrant to purchase a number of shares of preferred stock equal to \$4.0 million at the price paid by investors in the next equity financing (the "Eidos Warrant"). In March 2018, BridgeBio transferred 10% or \$1.0 million of its interests in the Eidos Note and the Eidos Warrant to the minority stockholder of Eidos. In March 2018, the Eidos Note was redeemed into shares of Series B redeemable convertible preferred stock of Eidos at a 30% discount to the price paid by other investors.

In March 2018, Eidos entered into the Eidos Series B Preferred Stock Purchase Agreement for issuance of shares of Eidos Series B redeemable convertible preferred stock in two closings. As part of the March 2018 closing, Eidos also issued a freestanding tranche liability related to the obligation of Eidos to issue additional shares and the right to request investors to purchase additional shares. The tranche liability was recorded at fair value and remeasured through the settlement date in May 2018. In May 2018, BridgeBio contributed \$11.2 million into Eidos in exchange for shares of Series B redeemable convertible preferred stock.

In June 2018, Eidos completed its initial public offering. All redeemable convertible preferred stock of Eidos was converted into common stock at the closing of the Eidos IPO. As part of the Eidos IPO, BridgeBio purchased common stock of \$17.0 million. The Eidos Warrant was also net exercised upon the completion of the Eidos IPO.

In December 2018, BridgeBio purchased common stock of Eidos for \$44.2 million from certain investors and Eidos founders, which resulted in BridgeBio owning 62.5% of Eidos as of December 31, 2018.

**BRIDGEBIO PHARMA LLC**

**Notes to Combined and Consolidated Financial Statements**

From the date of BridgeBio's initial investment until the Eidos IPO on June 22, 2018, Eidos was determined to be a VIE and BridgeBio consolidated Eidos as the primary beneficiary. Subsequent to the Eidos IPO, BridgeBio determined that Eidos was no longer a VIE due to it having sufficient equity at risk to finance its activities without additional subordinated financial support. From June 22, 2018 through December 31, 2018, BridgeBio determined that it held greater than 50% of the voting shares of Eidos and there were no other parties with substantive participating, liquidation or kick-out rights. BridgeBio consolidated Eidos under the VOE model as of December 31, 2018.

***PellePharm, Inc. ("PellePharm")***

PellePharm is a clinical stage biopharmaceutical company developing BBP-009, a topical gel formulation of patidegib, a hedgehog inhibitor, for the treatment of Gorlin Syndrome and High-Frequency Basal Cell Carcinoma. In July 2015, BridgeBio made an initial investment of \$4.5 million in PellePharm and in a series of transactions through December 2016, the Company increased its ownership interest to greater than 50%. BridgeBio determined that its initial investment in PellePharm represented a variable interest, but that BridgeBio was not the primary beneficiary until December 2016.

In December 2016, upon BridgeBio's additional investment in PellePharm, BridgeBio determined that consolidation of PellePharm was appropriate. At that time, BridgeBio was determined to be the primary beneficiary as it controlled the activities that most significantly impacted PellePharm's economic performance, controlled the most significant decisions affecting PellePharm through its representation within management and on PellePharm's Board of Directors, and BridgeBio had a majority ownership interest. The Company accounted for the transaction as an asset acquisition as substantially all of the estimated fair value of the gross assets acquired was concentrated in a single identified asset, IPR&D, thus satisfying the requirements of the screen test in ASU 2017-01. The assets acquired and liabilities assumed in the transaction were measured based on their fair values. The fair value of the IPR&D acquired was charged to research and development expense as it had no alternative future use at the time of the acquisition. The assembled workforce acquired was amortized over its estimated economic life of one year. During the year ended December 31, 2017, the Company recorded amortization expense in research and development expense related to the assembled workforce of \$0.2 million and the asset was fully amortized as of December 31, 2017.

In May 2018, BridgeBio increased its ownership of PellePharm through the purchase of additional Series B shares for \$4.0 million. In June 2018, BridgeBio contributed an additional \$1.5 million through the purchase of Series C redeemable convertible preferred stock of PellePharm.

On November 19, 2018, PellePharm entered into the LEO Agreement with LEO, pursuant to which LEO was granted an exclusive, irrevocable option to acquire PellePharm. The LEO Call Option is exercisable by LEO on or before the occurrence of certain events relating to PellePharm's clinical development programs and no later than July 30, 2021. As consideration for the option, LEO agreed to pay PellePharm exclusivity payments totaling \$27.9 million. LEO also purchased PellePharm common stock in the amount of \$5.1 million. In addition, LEO may pay additional exclusivity payments to PellePharm in an amount not to exceed \$37.0 million under certain circumstances. LEO is not a related party to or de facto agent of BridgeBio.

The Company accounts for the LEO Call Option as a current liability in its combined and consolidated financial statements because BridgeBio is obligated to sell its shares in PellePharm to LEO at a pre-determined price, if the option is exercised. As discussed in Note 3, the fair value of the LEO Call Option on issuance was \$1.9 million. The Company will remeasure the LEO Call Option to fair value at each subsequent combined and consolidated balance sheet date until the LEO Call Option is either exercised or expires.

**BRIDGEBIO PHARMA LLC**

**Notes to Combined and Consolidated Financial Statements**

The date the LEO Agreement was entered into was determined to be a VIE reconsideration event. Based on the Company's assessment, BridgeBio concluded that PellePharm remains a VIE after the reconsideration event as it does not have sufficient equity at risk to finance its activities without additional subordinated financial support. However, based on changes to PellePharm's governance structure and Board of Directors composition as a result of the LEO Agreement, BridgeBio is no longer the primary beneficiary as it no longer has the power over the key decisions that most significantly impact PellePharm's economic performance. Accordingly, BridgeBio deconsolidated PellePharm on November 19, 2018 (see Note 8). Through December 31, 2017 and 2018, BridgeBio has contributed \$19.4 million and \$25.0 million, respectively, to PellePharm in exchange for shares of redeemable convertible preferred stock. After the deconsolidation in November 2018, PellePharm is considered a related party of BridgeBio.

***Consolidated VIEs***

The following entities are VIEs for which BridgeBio was determined to be the primary beneficiary at the time of its initial investment in the VIE and through December 31, 2018. For each entity, the initial investment was determined to represent a variable interest as, at that time, the entity did not have sufficient resources to carry out its principal activities without additional financial support. BridgeBio was determined to be the primary beneficiary of each entity as it controlled the activities that most significantly impact the entity's economic performance and controlled the most significant decisions affecting the entity through its representation within management and the entity's board of directors. BridgeBio also had a majority ownership interest in the following entities as of December 31, 2017 and 2018 with exception of a 23% ownership of Phoenix Tissue Repair, Inc. as of December 31, 2017.

Molecular Skin Therapeutics, Inc. ("MoST") is a biopharmaceutical company focused on developing BBP-561, a series of topical KLK5/7 inhibitors, for the treatment of Netherton Syndrome. BridgeBio made investments in MoST of \$1.5 million in 2017 and \$1.2 million in 2018 in exchange for shares of redeemable convertible preferred stock.

Quartz Therapeutics, Inc. ("Quartz") is a biopharmaceutical company focused on the development of effective therapies for patients suffering from RAS-driven cancers. BridgeBio made investments in Quartz of \$4.0 million in 2017 in exchange for shares of redeemable convertible preferred stock. BridgeBio issued convertible notes to Quartz in 2018 totaling \$1.1 million that are outstanding as of December 31, 2018.

Navire Pharma, Inc. ("Navire") is a biopharmaceutical company advancing the Company's BBP-398 discovery program for small molecule inhibitors of SHP2 for the potential treatment of cancers driven by hyperactive receptor tyrosine kinase, or MAPK signaling. BridgeBio made investments in Navire of \$3.2 million in 2017 and \$6.8 million in 2018 in exchange for shares of redeemable convertible preferred stock.

CoA Therapeutics, Inc. ("CoA") is a biopharmaceutical company focused on the development of BBP-671, an oral small molecule, for the treatment of Pantothenate Kinase Associated Neurodegeneration, or PKAN. BridgeBio made investments in CoA of \$1.5 million in 2017 and \$7.0 million in 2018 in exchange for shares of redeemable convertible preferred stock.

Dermecular Therapeutics, Inc. ("Dermecular") is a biopharmaceutical company focused on the development of BBP-321, an oral S1P lyase inhibitor, for the treatment of Darier Disease and Hailey-Hailey Disease. BridgeBio made investments in Dermecular of \$4.5 million in 2017 and \$0.7 million in 2018 in exchange for shares of redeemable convertible preferred stock.

**BRIDGEBIO PHARMA LLC**

**Notes to Combined and Consolidated Financial Statements**

PTR is a biopharmaceutical company focused on developing BBP-589, an IV-administered recombinant collagen type VII, protein replacement therapy, for the treatment of recessive dystrophic epidermolysis bullosa. BridgeBio made investments in PTR of \$3.0 million in 2017 and \$10.5 million in 2018 in exchange for shares of redeemable convertible preferred stock.

Adrenas Therapeutics, Inc. (“Adrenas”) is a biopharmaceutical company focused on developing BBP-631, an adeno-associated virus, gene transfer product candidate, for the treatment of congenital adrenal hyperplasia, caused by 21-hydroxylase deficiency. BridgeBio made investments in Adrenas of \$13.4 million in 2018 in exchange for shares of redeemable convertible preferred stock.

QED is a biopharmaceutical company focused on developing infigratinib, an oral FGFR1-3 selective tyrosine kinase inhibitor, for the treatment of FGFR-driven cancers. BridgeBio made investments in QED of \$50.0 million in 2018 in exchange for shares of redeemable convertible preferred stock.

Orfan Biotech, Inc. (“Orfan”) is a biopharmaceutical company focused on developing BBP-711, a series of oral small molecule inhibitors of glycolate oxidase, for the treatment of primary hyperoxaluria and recurrent kidney stone disease. BridgeBio made investments in Orfan of \$3.0 million in 2018 in exchange for shares of redeemable convertible preferred stock.

Ferro Therapeutics, Inc. (“Ferro”) is a biopharmaceutical company focused on developing BBP-954 for irreversible inhibitors of glutathione peroxidase 4, for the treatment of solid and hematological cancers. BridgeBio made investments in Ferro of \$3.0 million in 2018 in exchange for shares of redeemable convertible preferred stock.

Venthera, Inc. (“Venthera”) is a biopharmaceutical company focused on developing BBP-681, a transdermal PI3K inhibitor, for the treatment of cutaneous venous and lymphatic malformations. BridgeBio made investments in Venthera of \$5.5 million in 2018 in exchange for shares of redeemable convertible preferred stock.

Aspa Therapeutics, Inc. (“Aspa”) is a biopharmaceutical company focused on developing BBP-812, an adeno-associated virus, gene transfer therapy, for the treatment of Canavan Disease. BridgeBio made investments in Aspa of \$8.0 million in 2018 in exchange for shares of redeemable convertible preferred stock.

**BRIDGEBIO PHARMA LLC**
**Notes to Combined and Consolidated Financial Statements**

The following table provides the assets and liabilities for all consolidated VIEs as of December 31, 2017:

	<u>Dermecular</u>	<u>Eidos</u>	<u>MoST</u>	<u>Navire</u>	<u>PellePharm</u>	<u>PTR</u>	<u>Quartz</u>	<u>Total</u>
	(in thousands)							
<b>Assets:</b>								
Current assets:								
Cash and cash equivalents	\$ 3,986	\$ 5,497	\$ 211	\$ 512	\$ 2,238	\$ 342	\$ 994	\$ 13,780
Prepaid expenses and other current assets	2	484	113	7	4,058	1	38	4,703
<b>Total current assets</b>	<b>3,988</b>	<b>5,981</b>	<b>324</b>	<b>519</b>	<b>6,296</b>	<b>343</b>	<b>1,032</b>	<b>18,483</b>
Property and equipment, net	—	114	—	3	—	—	287	404
Other assets	—	180	—	—	200	1	—	381
<b>Total assets</b>	<b>\$ 3,988</b>	<b>\$ 6,275</b>	<b>\$ 324</b>	<b>\$ 522</b>	<b>\$ 6,496</b>	<b>\$ 344</b>	<b>\$ 1,319</b>	<b>\$ 19,268</b>
<b>Liabilities:</b>								
Current liabilities:								
Accounts payable	\$ 56	\$ 564	\$ 14	\$ 2	\$ 514	\$ 40	\$ 118	\$ 1,308
Accrued compensation and benefits	—	433	—	80	288	—	66	867
Accrued research and development liabilities	118	563	—	833	1,570	—	—	3,084
Other accrued liabilities	127	305	8	14	351	9	18	832
<b>Total current liabilities</b>	<b>301</b>	<b>1,865</b>	<b>22</b>	<b>929</b>	<b>2,723</b>	<b>49</b>	<b>202</b>	<b>6,091</b>
Other liabilities	—	273	—	24	—	—	—	297
<b>Total liabilities</b>	<b>\$ 301</b>	<b>\$ 2,138</b>	<b>\$ 22</b>	<b>\$ 953</b>	<b>\$ 2,723</b>	<b>\$ 49</b>	<b>\$ 202</b>	<b>\$ 6,388</b>

The following table provides the assets and liabilities for all consolidated VIEs as of December 31, 2018:

	<u>Adrenas</u>	<u>Aspa</u>	<u>PTR</u>	<u>QED</u>	<u>Venthera</u>	<u>All Other</u>	<u>Total</u>
	(in thousands)						
<b>Assets:</b>							
Current assets:							
Cash and cash equivalents	\$ 3,046	\$ 4,259	\$ 6,934	\$ 8,630	\$ 2,913	\$ 6,713	\$ 32,495
Prepaid expenses and other current assets	665	1,722	28	3,240	—	321	5,976
<b>Total current assets</b>	<b>3,711</b>	<b>5,981</b>	<b>6,962</b>	<b>11,870</b>	<b>2,913</b>	<b>7,034</b>	<b>38,471</b>
Property and equipment, net	584	129	88	181	—	277	1,259
Other assets	7	—	41	—	—	28	76
<b>Total assets</b>	<b>\$ 4,302</b>	<b>\$ 6,110</b>	<b>\$ 7,091</b>	<b>\$ 12,051</b>	<b>\$ 2,913</b>	<b>\$ 7,339</b>	<b>\$ 39,806</b>
<b>Liabilities:</b>							
Current liabilities:							
Accounts payable	\$ 1,876	\$ 1,187	\$ 621	\$ 3,537	\$ 333	\$ 1,737	\$ 9,291
Accrued compensation and benefits	377	30	287	1,392	—	467	2,553
Accrued research and development liabilities	227	728	—	4,390	—	1,251	6,596
Other accrued liabilities	28	32	8	229	9	82	388
<b>Total current liabilities</b>	<b>2,508</b>	<b>1,977</b>	<b>916</b>	<b>9,548</b>	<b>342</b>	<b>3,537</b>	<b>18,828</b>
Other liabilities	—	—	—	150	—	29	179
<b>Total liabilities</b>	<b>\$ 2,508</b>	<b>\$ 1,977</b>	<b>\$ 916</b>	<b>\$ 9,698</b>	<b>\$ 342</b>	<b>\$ 3,566</b>	<b>\$ 19,007</b>

**BRIDGEBIO PHARMA LLC**

**Notes to Combined and Consolidated Financial Statements**

VIEs included in the “All Other” category of the above table are not significant individually for separate presentation. Going forward, BridgeBio may not provide any further investment in certain of these VIEs.

**7. Noncontrolling Interests**

As of December 31, 2017 and 2018, the Company has both redeemable convertible noncontrolling interests and noncontrolling interests in consolidated partially-owned entities, for which BridgeBio has a majority voting interest under the VOE model and for which BridgeBio is the primary beneficiary under the VIE model. These balances are reported as separate components outside members’ deficit and as part of members’ deficit, respectively, in “Redeemable convertible noncontrolling interests” and “Noncontrolling interests” in the combined and consolidated balance sheets.

The Company adjusts the carrying value of noncontrolling interest to reflect the book value attributable to noncontrolling shareholders of consolidated partially-owned entities when there is a change in the ownership during the respective reporting period. Such adjustments in the aggregate amounts of \$8.2 million and \$21.6 million for the years ended December 31, 2017 and 2018, respectively, are recorded to accumulated deficit in the line item “Transfers to (from) noncontrolling interest” for the year ended December 31, 2017 and “Transfers to (from) and conversion of noncontrolling interest” for the year ended December 31, 2018 in the Combined and Consolidated Statements of Redeemable Convertible Preferred Units, Redeemable Founder Units, Redeemable Common Units, Management Incentive Units, Redeemable Convertible Noncontrolling Interests and Members’ Deficit.

Upon the Eidos IPO in June 2018, all outstanding shares of Eidos’ redeemable convertible preferred stock were converted into shares of common stock of Eidos. This transaction is reflected as conversion of redeemable noncontrolling interest into noncontrolling interest in the table below. The net exercise of the Eidos Warrants upon the Eidos IPO is presented as the issuance of noncontrolling interest in the table below.

## BRIDGEBIO PHARMA LLC

## Notes to Combined and Consolidated Financial Statements

The following table provides a rollforward of the redeemable convertible noncontrolling interests balance, as follows:

	<u>Eidos</u>	<u>Orfan</u>	<u>PellePharm</u> <u>(in thousands)</u>	<u>QED</u>	<u>Total</u>
Balance as of January 1, 2017	\$ 6	—	\$ 1,514	—	\$ 1,520
Issuance of redeemable convertible noncontrolling interest	—	—	2,839	—	2,839
Net loss attributable to redeemable convertible noncontrolling interest	(27)	—	(2,730)	—	(2,757)
Transfers to (from) redeemable convertible noncontrolling interest	26	—	(795)	—	(769)
Balance as of December 31, 2017	5	—	828	—	833
Issuance of redeemable convertible noncontrolling interest	51,012	187	9,429	1,735	62,363
Net loss attributable to redeemable convertible noncontrolling interest	(1,411)	(263)	(6,181)	(4,675)	(12,530)
Deconsolidation of PellePharm	—	—	1,154	—	1,154
Transfers to (from) and conversion of noncontrolling interest:					
Transfers to (from) redeemable convertible noncontrolling interest	(37,354)	84	(5,230)	3,054	(39,446)
Conversion of redeemable convertible noncontrolling interest to noncontrolling interest	(12,252)	—	—	—	(12,252)
Balance as of December 31, 2018	<u>\$ —</u>	<u>\$ 8</u>	<u>\$ —</u>	<u>\$ 114</u>	<u>\$ 122</u>



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**Notes to Combined and Consolidated Financial Statements**

The following table provides a rollforward of the noncontrolling interests balance:

	<u>Adrenas</u>	<u>Aspa</u>	<u>Eidos</u>	<u>PellePharm</u>	<u>PTR</u>	<u>Venthera</u>	<u>All Other</u>	<u>Total</u>
	(in thousands)							
Balance as of January 1, 2017	\$ —	\$ —	\$ 605	\$ 1,990	\$ —	\$ —	\$ —	\$ 2,595
Issuance of noncontrolling interest	—	—	1,218	181	1	—	44	1,444
Transfers to (from) noncontrolling interest	—	—	2,157	2,272	2,242	—	2,298	8,969
Net loss attributable to noncontrolling interest	—	—	(3,214)	(3,560)	(2,016)	—	(1,720)	(10,510)
Balance as of December 31, 2017	—	—	766	883	227	—	622	2,498
Issuance of noncontrolling interest	5	7	98,765	239	7	14	442	99,479
Net loss attributable to noncontrolling interest	(1,548)	(416)	(13,457)	(4,541)	(2,716)	(612)	(2,882)	(26,172)
Deconsolidation of PellePharm	—	—	—	688	—	—	—	688
Repurchase of redeemable noncontrolling interest	—	—	(44,234)	—	—	—	—	(44,234)
Transfers to (from) and conversion of noncontrolling interest:								
Transfers to (from) noncontrolling interest	1,760	654	4,093	2,731	5,210	1,047	2,355	17,850
Conversion of redeemable convertible noncontrolling interest to noncontrolling interest	—	—	12,252	—	—	—	—	12,252
Balance as of December 31, 2018	<u>\$ 217</u>	<u>\$ 245</u>	<u>\$ 58,185</u>	<u>\$ —</u>	<u>\$ 2,728</u>	<u>\$ 449</u>	<u>\$ 537</u>	<u>\$ 62,361</u>

Previously reported amounts for the year ended December 31, 2018 have been corrected in the above tables related to the overstatement of “Conversion of redeemable convertible noncontrolling interest to noncontrolling interest” and understatement of “Transfers to (from) redeemable convertible noncontrolling interest” and “Transfers to (from) noncontrolling interest” in the amount of \$25.1 million, for both Eidos and in total. Such correction does not impact the “Redeemable convertible noncontrolling interests”, “Noncontrolling interests”, or “Total members’ deficit” ending balances for the period ended December 31, 2018. This correction is also reflected in the “Conversion of redeemable noncontrolling interest into noncontrolling interest” balance in the accompanying supplemental disclosures of non-cash investing and financing information. The Company evaluated this correction considering both quantitative and qualitative factors and concluded it was immaterial to previously issued combined and consolidated financial statements.

**BRIDGEBIO PHARMA LLC****Notes to Combined and Consolidated Financial Statements****8. PellePharm Deconsolidation**

As a result of the deconsolidation of PellePharm in November 2018 (see Note 6), BridgeBio recorded a gain of \$19.3 million primarily related to the remeasurement of its common stock and preferred stock investment in PellePharm to its estimated fair value of \$17.3 million. The gain is included in the accompanying combined and consolidated statement of operations for the year ended December 31, 2018. The Company concluded that the deconsolidation of PellePharm did not qualify for presentation as discontinued operations.

The valuation technique used to measure the fair value of the retained investment in the PellePharm's common stock and preferred stock is the PWERM, which was based on the expected proceeds from either the acquisition of PellePharm by LEO or LEO not exercising its option to acquire PellePharm during the option period. As of the deconsolidation date, BridgeBio holds 8.0% of the outstanding PellePharm common stock and 61.9% of the outstanding PellePharm preferred stock. BridgeBio also has continuing involvement and significant influence in PellePharm through its participation on the PellePharm Board of Directors. The carrying amount of BridgeBio's investment in PellePharm in the combined and consolidated balance sheets represents its maximum loss exposure related to its VIE investment in PellePharm.

As of the deconsolidation date, BridgeBio's investment in PellePharm had a fair value of \$17.3 million, which is comprised of \$0.5 million in PellePharm common stock that is accounted for as an equity method investment and \$16.8 million in PellePharm preferred stock that is accounted as a cost method investment.

The following represents the amounts related to the PellePharm deconsolidation accounting:

	<u>Amount</u> <u>(in thousands)</u>
Working capital <sup>(1)</sup> (excluding cash and cash equivalents)	\$ 6,134
Term loan	1,359
Property and equipment, net	(791)
Carrying value of noncontrolling interest	(688)
Carrying value of redeemable convertible noncontrolling interest	(1,154)
Fair value of interest retained by BridgeBio	17,325
Gain on deconsolidation of PellePharm	<u>(19,327)</u>
Decrease in cash and cash equivalents resulting from the deconsolidation of PellePharm	<u>\$ 2,858</u>

(1) Working capital is defined as current assets less current liabilities.

After the deconsolidation of PellePharm in November 2018, BridgeBio accounted for its retained common stock investment as an equity method investment. BridgeBio's common stock investment valued at \$0.5 million upon deconsolidation was compared to BridgeBio's percentage of underlying equity in net assets of PellePharm. BridgeBio concluded that there was no material basis difference.

Through December 31, 2018, BridgeBio's share of PellePharm's net losses amounted to \$0.3 million based on its percentage of common stock ownership in PellePharm. As of December 31, 2018, the aggregate carrying amount for the Company's equity method investment in PellePharm is \$0.2 million. As of December 31, 2018, the aggregate carrying amount for the Company's cost method investment in PellePharm is \$16.8 million. The aggregate carrying amount of the PellePharm investment is presented as a separate line item in the combined and consolidated balance sheet as of December 31, 2018. The Company did not recognize an impairment related to its PellePharm investment during the year ended December 31, 2018.

**BRIDGEBIO PHARMA LLC****Notes to Combined and Consolidated Financial Statements****9. Commitments and Contingencies*****Operating Lease Commitments***

The Company leases office space under noncancelable operating leases that have terms expiring through April 2023. In March 2017, BridgeBio entered into a three-year agreement to rent 3,900 square feet of office space in Palo Alto, California. The aggregate rent expense under the lease is \$1.1 million.

In November 2017, Eidos entered into a five-year agreement to rent 4,659 square feet of office space in San Francisco, California. The aggregate rent expense under the lease is \$1.7 million.

In January 2018, PellePharm entered into a five-year agreement to rent 4,484 square feet of office space in San Francisco, California. The aggregate rent expense under the lease is \$1.7 million.

In January 2018, PTR entered into a sixty-three-month agreement to rent 2,460 square feet of office space in Boston, Massachusetts. The aggregate rent expense under the lease is \$1.1 million.

In February 2018, QED entered into a thirty-seven-month agreement to rent 1,944 square feet of office space in San Francisco, California. The aggregate rent expense under the lease is \$0.6 million. In October 2018, QED entered into a thirty-four-month agreement to rent 10,000 square feet of office space in San Francisco, California. The aggregate rent expense under the lease is \$2.6 million.

The Company recognizes rent expense on a straight-line basis over the noncancelable lease period and records the difference between cash payments and the recognition of rent expense as a deferred rent liability. Where leases contain escalation clauses, rent abatements and/or concessions, such as rent holidays and landlord or tenant incentives or allowances, the Company applies them in the determination of straight-line rent expense over the lease period.

As of December 31, 2018, future minimum lease payments for all noncancelable operating leases with remaining lease terms in excess of one year, are as follows:

<b>Year Ending December 31:</b>	<b>Amount</b>
	<b>(in thousands)</b>
2019	\$ 1,924
2020	1,759
2021	1,333
2022	597
2023	98
Total future minimum lease payments	<u>\$ 5,711</u>

Total rent expense for the years ending December 31, 2017 and 2018 was \$0.4 million and \$1.5 million, respectively.

***Other Research and Development Agreements***

The Company may also enter into contracts in the normal course of business with clinical research organizations for clinical trials, with contract manufacturing organizations for clinical supplies and with other vendors for preclinical studies, supplies and other services and products for operating purposes. These contracts

**BRIDGEBIO PHARMA LLC**

**Notes to Combined and Consolidated Financial Statements**

generally provide for termination on notice, with the exception of potential termination charges related to one of the Company's contract manufacturing agreements in the event that certain minimum purchase volumes are not met. As of December 31, 2017 and 2018, there were no amounts accrued related to termination charges for minimum purchase volumes not being met.

***Indemnification***

In the ordinary course of business, the Company may provide indemnifications of varying scope and terms to vendors, lessors, business partners, board members, officers and other parties with respect to certain matters, including, but not limited to, losses arising out of breach of such agreements, services to be provided by the Company, negligence or willful misconduct of the Company, violations of law by the Company, or intellectual property infringement claims made by third parties. In addition, the Company has entered into indemnification agreements with directors and certain officers and employees that will require the Company, among other things, to indemnify them against certain liabilities that may arise by reason of their status or service as directors, officers or employees. No demands have been made upon the Company to provide indemnification under such agreements, and thus, there are no claims that the Company is aware of that could have a material effect on the Company's combined and consolidated balance sheets, statements of operations and comprehensive loss, or statements of cash flows.

The Company also maintains director and officer insurance, which may cover certain liabilities arising from its obligation to indemnify the Company's directors. To date, the Company has not incurred any material costs and has not accrued any liabilities in the combined and consolidated financial statements as a result of these provisions.

***Contingencies***

On December 31, 2018, Children Hospital Research Center at Oakland ("CHRCO") filed, and as of April 15, 2019 has not yet served, a civil complaint against Dr. Ervin Epstein, Co-Founder and Chief Medical Officer of PellePharm and PellePharm in the Northern District of California. CHRCO asserts four causes of action against Dr. Epstein (conversion, breach of contract, breach of the implied covenant of good faith and fair dealing, and specific performance), and one related cause of action against PellePharm (constructive trust). All five causes of action are generally directed to a set of accusations relating to Dr. Epstein's prior employment at CHRCO. CHRCO has since withdrawn its complaint in the Northern District of California and filed, and as of May 16, 2019, has not yet served a revised civil complaint against PellePharm and Dr. Epstein in the Superior Court of the State of California, County of San Francisco, asserting the same five causes of action. On April 11, 2019, CHRCO filed an unopposed ex parte application with the Court to extend the deadline to serve the complaint to June 11, 2019. Dr. Epstein and PellePharm dispute all of CHRCO's allegations and believe they lack merit and they intend to contest the case vigorously. No responsive pleading is required at this time, nor has Dr. Epstein or PellePharm provided one.

From time to time, the Company may become involved in legal proceedings arising in the ordinary course of business. The Company is unable to predict the outcome of these matters or the ultimate legal and financial liability, and at this time cannot reasonably estimate the possible loss or range of loss and accordingly has not accrued a related liability.

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**Notes to Combined and Consolidated Financial Statements**

**10. Hercules Term Loan**

***Hercules Loan and Security Agreement***

In June 2018, the Company executed a Loan and Security Agreement with Hercules Capital, Inc. (“Hercules”), under which the Company borrowed \$35.0 million. The term of the loan was approximately 42 months, with a maturity date of January 1, 2022 (the “Maturity Date”). No principal payments were due during an interest-only period, commencing on the initial borrowing date and continuing through July 1, 2020 (the “Amortization Date”). The outstanding balance of the loan was to be repaid monthly beginning on the Amortization Date and extending through the Maturity Date. The term loan bears interest at a floating rate equal to the greater of: (i) the prime rate as reported in the Wall Street Journal plus 4.35% and (ii) 9.35% (9.85% as of December 31, 2018 based on the prime rate as of that date), payable monthly.

In December 2018, the Company executed the First Amendment to the Loan and Security Agreement, whereby the Company borrowed an additional \$20.0 million to increase the total principal balance outstanding to \$55.0 million (the “Amended Hercules Term Loan”). Upon draw of the additional \$20.0 million, the interest-only period on the entire facility was extended until January 1, 2021 (the “Amended Amortization Date”). The outstanding balance of the original loan of \$35.0 million and the additional borrowing of \$20.0 million is to be repaid monthly beginning on the Amended Amortization Date and extending through July 1, 2022 (the “Amended Maturity Date”). The additional \$20.0 million loan bears interest at a floating rate equal to the greater of: (i) the prime rate as reported in the Wall Street Journal plus 3.35% and (ii) 9.10% (9.10% as of December 31, 2018), payable monthly.

On the earliest to occur of (i) the Amended Maturity Date, (ii) the date the Company prepays the outstanding principal amount of the Amended Hercules Term Loan or (iii) the date the outstanding principal amount of the Amended Hercules Term Loan otherwise becomes due, the Company will owe Hercules an end of term charge equal to 6.35% of the principal amount of the original \$35.0 million term loan, or \$2.2 million, and 5.75% of the principal amount of the incremental \$20.0 million term loan, or \$1.2 million. These amounts will be accrued over the term of the loan using the effective-interest method.

Effective the first fiscal quarter following the completion by the Company of an initial public offering (“IPO”) of its shares on a United States-based national exchange with total net proceeds of no less than \$175.0 million, the Company shall receive: (i) a further six month interest-only extension to July 1, 2021, (ii) a further six month maturity extension to January 1, 2023, (iii) a reduction of 0.5% on the then effective interest rate on the entire facility, and (iv) the option to pay up to 1.5% of scheduled cash pay interest on the entire facility as payment in kind (“PIK Interest”), with such cash pay interest paid as PIK Interest at a 1:1.2 ratio. All PIK Interest shall be capitalized and added to the outstanding principal balance under the Amended Hercules Term Loan, which shall then accrue further cash interest and fees pursuant to the terms of the Amended Hercules Term Loan. There are no material embedded features requiring bifurcation.

During the year ended December 31, 2018, the Company recognized interest expense related to the Amended Hercules Term Loan of \$2.4 million, of which \$0.5 million relates to amortization of debt discount.

The Amended Hercules Term Loan contains customary representations and warranties, events of default, and affirmative and negative covenants for a term loan facility of this size and type. However, Hercules imposes no liquidity covenants on the Company and Hercules cannot limit or restrict the Company’s ability to dispose of assets, make investments, or make acquisitions. As pledged collateral for its obligations under the Amended Hercules Term Loan, the Company granted Hercules a security interest in all assets or personal property of the Company, including all equity interests owned or hereafter acquired by the Company. Further, at Hercules’ sole

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discretion the Company must make a mandatory prepayment equal to 75% of net cash proceeds received from the sale or licensing of any pledged or collateral assets, including intellectual property, of a consolidated entity owned by the Company, or the repurchase or redemption of any pledged collateral by certain specified operating companies. None of the Company's consolidated entities are a party to, nor provide any credit support or other security in connection with the Amended Hercules Term Loan.

The term loans balance is as follows:

	<b>December 31, 2018</b>
	<b>(in thousands)</b>
Principal value of term loans	\$ 55,000
Net of debt issuance costs and debt accretion	(493)
Term loans, noncurrent	<u>\$ 54,507</u>

Future minimum payments of principal and estimated payments of interest on the Company's outstanding variable rate borrowings as of December 31, 2018 are as follows:

<b>Year Ending December 31:</b>	<b>(in thousands)</b>
2019	\$ 5,200
2020	5,355
2021	37,575
2022	25,292
Total future payments	73,422
Less amounts representing interest	(15,049)
Less final end of term payment	(3,373)
Total principal amount of term loan payments	<u>\$ 55,000</u>

**11. License Agreements*****Stanford License Agreement***

In April 2016, Eidos entered into a license agreement with the Board of Trustees of the Leland Stanford Junior University ("Stanford University") relating to Eidos' drug discovery and development initiatives. Under this agreement, Eidos has been granted certain worldwide exclusive licenses to make, use and sell products that are covered by licensed patent rights. In March 2017, Eidos paid a license fee of \$10,000, which was recorded as research and development expense during the year ended December 31, 2017, as the acquired assets did not have any alternative future use. In August 2016, Eidos issued 56,809 shares of common stock valued at a nominal fair value at issuance. Eidos may also be required to make future payments of up to approximately \$1.0 million to Stanford University upon achievement of specific intellectual property, clinical and regulatory milestone events, and pay royalties of up to low single-digit percentages on future net sales, if any. In addition, Eidos is obligated to pay Stanford University a percentage of non-royalty revenue received by Eidos from its sublicensees, with the amount owed decreasing annually for three years based on when the applicable sublicense agreement is executed. During the years ended December 31, 2017 and 2018, Eidos recognized research and development expense of less than \$0.1 million and \$0.3 million, respectively, in connection with this agreement.

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***The University of Texas License Agreement***

In March 2017, Navire entered into a collaboration and license agreement with The Board of Regents of The University of Texas System (“Board of Regents”) and The University of Texas M.D. Anderson Cancer Center (“MD Anderson” and collectively “University of Texas”) relating to Navire’s drug discovery and development initiatives. Under this agreement, Navire and the University of Texas will carry out the development, manufacture and commercialization of licensed product under exclusive licenses granted by the University of Texas. In partial consideration for the exclusive license grant, the Company issued the Board of Regents shares of common stock of Navire valued at \$0.3 million at issuance pursuant to a stock purchase agreement entered into simultaneously. In addition, Navire is required to make additional payments based on achievement of a milestone event by March 31, 2018 (this was not achieved) as well as obtaining certain development milestones and specific market capitalization thresholds of Navire.

If commercial sales of a licensed product commence, the Company will pay MD Anderson royalties at percentage rates ranging in the low single digits on net sales of licensed products. The Company may offset payments made to third parties to obtain rights needed for the commercialization of a licensed product against royalties payable to MD Anderson provided that such expenses in a given year may not be credited against more than a specified percentage of the royalties in such year and subject to a minimum floor in the low single digits. The Company’s obligation to pay royalties continues on a country-by-country basis until the expiration of all licensed patent rights covering licensed products in such country. During the years ended December 31, 2017 and 2018, Navire recognized research and development expense of \$2.2 million and \$4.4 million, respectively, in connection with this agreement.

***The Regents of the University of California License Agreement***

In September 2016, TheRas, Inc. (“TheRas”) entered into a license agreement with The Regents of the University of California (“UCSF”) relating to TheRas’ drug discovery and development initiatives. Under this agreement, TheRas has been granted certain worldwide exclusive licenses to use the licensed compounds (the “UCSF License”). In connection with the UCSF License and subsequent amendments, the Company paid issuance fees totaling \$0.3 million. In addition, under the terms of the UCSF License, the Company is required to pay to UCSF certain annual license maintenance fees unless the Company is selling or otherwise exploiting licensed products or services and paying royalties to UCSF on net sales for such licensed products or services. With respect to such royalty obligations, the Company agreed to pay UCSF low single-digit tiered royalties on annual net sales of licensed products and services, with a minimum royalty requirement of \$0.1 million. The Company’s obligation to pay royalties continues on a country-by-country basis until the expiration of all licensed patent rights covering licensed products in such country. In addition, the Company is obligated to make contingent milestone payments totaling up to \$22.4 million upon the achievement of certain clinical or regulatory milestones. In the event that the Company sublicenses the patent rights, UCSF is also entitled to receive a percentage of the sublicensing income received by the Company. During the years ended December 31, 2017 and 2018, TheRas recognized research and development expense of \$0.2 million and \$0.1 million, respectively, in connection with this agreement.

***St. Jude License Agreement***

In April 2017, CoA entered into a license agreement with St. Jude Children’s Research Hospital, Inc., (“St. Jude”) relating to the CoA’s drug discovery and development initiatives. Under this agreement, CoA has been granted a worldwide exclusive license to use a licensed compound. CoA paid for certain costs and an upfront license fee of \$0.1 million in April 2017, which were recorded as research and development expense. Under the license agreement, CoA may also be required to make future payments of up to approximately \$4.6 million upon

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achievement of specific intellectual property, clinical and regulatory milestone events, as well as pay royalties on future net sales, if any. During the years ended December 31, 2017 and 2018, CoA recognized research and development expense of \$0.4 million and \$0.6 million, respectively, in connection with this agreement.

***K-Gen License Agreement***

In March 2018, Ferro entered into a license agreement with K-Gen, Inc. (“K-Gen”) relating to Ferro’s drug discovery and development initiatives. Under this agreement, Ferro has been granted certain worldwide exclusive licenses to use the licensed compounds. Upon execution of the agreement, Ferro made an upfront payment of \$0.8 million to K-Gen and is required to pay mid single-digit royalties on future net sales, if any. In addition, Ferro is obligated to pay K-Gen a percentage of non-royalty revenue received by Ferro from its sublicensees. During the year ended December 31, 2018, Ferro recognized research and development expense of \$0.8 million in connection with this agreement.

***Memorial Sloan Kettering Cancer Center License Agreement***

In April 2018, Venthera entered into a license agreement with Memorial Sloan Kettering Cancer Center (“MSK”) relating to Venthera’s drug discovery and development initiatives. Under this agreement, Venthera has been granted certain worldwide exclusive licenses to use the licensed products. Upon execution of the agreement, Venthera made an upfront payment and issued 76,934 shares of Venthera’s common stock to MSK. Venthera may be required to make future payments of up to \$10.0 million upon achievement of specific intellectual property, clinical and regulatory milestone events, as well as pay low single-digit royalties on future net sales, if any, and a nominal annual license maintenance fee in each year before the first commercial sale of any licensed product. In addition, Venthera is obligated to pay MSK a percentage of non-royalty revenue received by Venthera from its sublicensees, with the amount owed decreasing annually for three years based on when the applicable sublicense agreement is executed. During the year ended December 31, 2018, Venthera recognized a nominal research and development expense and general and administrative expense in connection with this agreement.

***University of Massachusetts License Agreement***

In April 2018, Aspa entered into a license agreement with the University of Massachusetts (“UM”) relating to Aspa’s drug discovery and development initiatives. Under this agreement, Aspa has been granted certain worldwide exclusive licenses to use the licensed compounds. Aspa may be required to make future payments of up to \$4.8 million upon achievement of specific intellectual property, clinical and regulatory milestone events, as well as pay mid single-digit royalties on future net sales, if any. During the year ended December 31, 2018, Aspa recognized research and development expense of \$0.8 million in connection with this agreement.

***NeuroVive License Agreement***

In June 2018, Fortify Therapeutics, Inc. (“Fortify”) entered into a license agreement with NeuroVive Pharmaceutical AB (“NeuroVive”) relating to Fortify’s drug discovery and development initiatives. Under this agreement, Fortify has been granted certain worldwide exclusive licenses to use the licensed compounds. Upon execution of the agreement and during 2018, Fortify made payments of \$0.2 million. Fortify may be required to reimburse NeuroVive for certain expenses and to make future payments of up to approximately \$6.0 million upon achievement of specific preclinical, clinical and regulatory milestone events, as well as pay mid-to-high single-digit royalties on future net sales, if any. During the year ended December 31, 2018, Fortify recognized research and development expense of \$0.2 million in connection with this agreement.



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***Life License Agreement***

In August 2018, Adrenas entered into a license agreement with Life Technologies Corporation (“Life”) relating to Adrenas’ drug discovery and development initiatives. Under this agreement, Adrenas has been granted certain worldwide non-exclusive licenses to use the licensed compounds. Upon execution of the agreement, Adrenas made a nominal upfront payment and may be required to make future payments upon achievement of licensing of certain products. During the year ended December 31, 2018, BridgeBio recognized a nominal research and development expense in connection with this agreement.

In November 2018, BridgeBio entered into a commercial license agreement with Life relating to the Company’s drug discovery and development initiatives. Under this agreement, BridgeBio has been granted certain worldwide non-exclusive licenses to use certain cell lines to produce licensed products. Upon execution of the agreement, BridgeBio paid an initial license fee and may be required to pay additional license fees to Life upon licensing additional licensed products.

***Unnamed Entity License Agreement***

In December 2018, the Company’s Unnamed Entity entered into a license agreement relating to the Unnamed Entity’s drug discovery and development initiatives. Under this agreement, the Unnamed Entity has been granted certain worldwide exclusive licenses to use the licensed compounds. Upon execution of this agreement, the Unnamed Entity made an upfront payment of \$0.5 million. If certain substantive milestones are met in the future, the Unnamed Entity may be required to make up to \$13.3 million in clinical and regulatory milestone payments, \$80.0 million in sales milestone payments, and pay royalties of up to the mid single-digit percentages on future net sales, if any. During the year ended December 31, 2018, the Unnamed Entity recognized research and development expense of \$0.5 million in connection with this agreement.

***Other License and Collaboration Agreements***

In addition to the agreements described above, the Company has also entered into other license and collaboration agreements with various institutions and business entities on terms similar to those described above, none of which are material individually or in the aggregate.

**12. Related Party Transactions**

***Nonrecourse Notes***

In 2016 and 2017 the Company entered into nonrecourse notes (the “Notes”) with two founders. The Notes were issued to facilitate the purchase of Series B Preferred Units by two founders. The principal amount of the Notes was \$0.3 million and mature in May 2021. The Notes were accounted for as an option for which the Company recognized equity-based compensation expense on issuance. The repayment of the Notes is recorded as an addition to the Series B Preferred Units balance as payments are received. As of December 31, 2017 and 2018, the Notes had an outstanding balance of \$0.2 million and were subsequently paid in full in February 2019.

***Promissory Note***

In May 2017, the Company issued a promissory note of \$4.0 million to KKR Alternative Credit, Inc. to facilitate the purchase of Series C Preferred Units. The promissory note bore no interest and was not contractually convertible into Preferred Units, Founder Units, or Common Units. The promissory note was payable in cash upon the earlier of the six-month anniversary of issuance or the issuance by the Company of any

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units. In August 2017, upon the initial closing of the Series C Preferred Units financing, the promissory note was converted into 4,142,502 Series C Preferred Units at the same issuance price of \$0.9656 as the purchase price for the other investors in Series C Preferred Units financing.

***Consulting Agreement***

During 2017, the Company and a founder entered into a consulting agreement in which the Company agreed to pay \$0.2 million per year in exchange for consulting services. During 2017, the Company entered into a consulting agreement with its chief executive officer in which the Company paid \$0.5 million in exchange for consulting services during 2017.

**13. Redeemable Convertible Preferred Units, Founder Units, Common Units and Management Incentive Units**

***Series B Preferred Units Financing***

In March 2016, BridgeBio entered into the Series B Preferred Units Purchase Agreement, (the “Series B Agreement”), for the issuance of up to 75,340,907 Series B Preferred Units in several closings at a price of \$0.44 per unit. In March 2016, BridgeBio LLC issued and sold an aggregate of 15,568,183 Series B preferred units at a price of \$0.44 per unit, for an aggregate purchase price of approximately \$6.9 million.

According to the terms of the Series B Agreement, BridgeBio in future closings is required to issue the remaining 68,406,108 Series B Preferred Units at the same fixed price as the initial closing and the Series B investors are required to purchase the remaining Series B Preferred Units. On issuance, BridgeBio determined that the obligation to issue the remaining 68,406,108 units of its Series B Preferred Units is a tranche liability that should be accounted for as a separate freestanding financial instrument and remeasured to fair value at each subsequent reporting period until settlement or expiration. The Series B Preferred Units tranche liability was recorded on issuance in March 2016 at a fair value of \$0.9 million. During the year ended December 31, 2016, additional closings of BridgeBio’s Series B Preferred Units occurred resulting in the Series B Preferred Units tranche liability amounting to \$0.2 million as of December 31, 2016.

In April 2017, BridgeBio settled the Series B Preferred Units tranche liability when it issued the remaining 26,901,279 Series B Preferred Units for aggregate proceeds of \$11.8 million, which resulted in the Series B Preferred Units tranche liability being extinguished.

***Series C Preferred Units Financing***

In June 2017, BridgeBio entered into the Series C Preferred Units Purchase Agreement (the “Series C Agreement”) for the issuance of up to 141,155,758 Series C Preferred Units at a price of \$0.9656 per unit. Between August 2017 and December 2017, BridgeBio issued 99,420,050 Series C Preferred Units at a price of \$0.9656 per unit for net proceeds of \$95.2 million. As discussed in Note 12, BridgeBio issued 4,142,502 Series C Preferred Units upon the conversion of a \$4.0 million promissory note.

As of December 31, 2017, the purchasers of Series C Preferred Units had a non-transferable right, but not an obligation (“Capital Commitment”) to purchase up to an additional 37,593,206 Series C Preferred Units at the same terms as the initial closing. BridgeBio determined that the Capital Commitment was not a freestanding instrument and was not required to be bifurcated as an embedded derivative. In April 2018, BridgeBio issued 37,593,206 Series C Preferred Units at a price of \$0.9656 per unit for net proceeds of \$36.3 million.

**BRIDGEBIO PHARMA LLC**

**Notes to Combined and Consolidated Financial Statements**

***Series D Preferred Units Financing***

In November 2018, BridgeBio entered into the Series D Preferred Unit Purchase Agreement (the “Series D Agreement”) for the issuance of up to 150,955,597 Series D Preferred Units at a price of \$1.9823 per unit. In November 2018 and December 2018, BridgeBio issued 150,955,597 Series D Preferred Units at a price of \$1.9823 per unit for net proceeds of \$298.7 million (net of \$0.5 million in issuance costs) and executed its Fourth Amended and Restated Limited Liability Company Agreement (the “LLC Agreement”) to create such new membership interests. The terms of Series D Preferred Units are similar to those of Series C Preferred Units, with the exception of distribution provisions as discussed below. Series D Preferred Units have the first priority up to the Series D Preferred Unit value of \$1.9823 per unit. In addition, the LLC Agreement was amended such that cumulative returns for all previously issued Preferred Units, Common Units and Founder Units would no longer accumulate.

As a result of the execution of the LLC Agreement in November 2018, BridgeBio concluded that the terms and conditions related to the Series C Preferred Units, Series B Preferred Units, Series A Preferred Units, and Founder Units were amended. BridgeBio performed a qualitative assessment as to whether a modification or extinguishment of the units had occurred under the accounting literature as a result of this amendment and concluded that the terms and conditions had not substantively changed. In addition, BridgeBio also performed a supplemental assessment by performing a valuation of the units before and after the execution of the LLC Agreement. BridgeBio concluded that the fair value of the Series C Preferred Units, Series B Preferred Units, Series A Preferred Units, and Founder Units had declined by less than ten percent. Consistent with the conclusions reached under the qualitative approach and the supplemental fair value assessment, the amendment was accounted for as a modification in accordance with the accounting literature. As there was a negative incremental change in fair value for the Series C Preferred Units, Series B Preferred Units, Series A Preferred Units, and Founder Units, no adjustment to the carrying value of the units was recorded for the year ended December 31, 2018.

The pre and post Series D issuance valuations were performed by a third-party specialist utilizing a PWERM, which included a scenario-based analysis that estimated the value per unit based on the probability-weighted present value of expected future investment returns, considering each of the possible outcomes available, as well as the economic and control rights of each unit type. Refer to Note 14 for discussion of the accounting treatment for the Common Units and Management Incentive Units related to the amended terms and conditions.

***Redeemable Convertible Preferred Units, Founder Units, Common Units and Management Incentive Units***

As of December 31, 2018, the LLC Agreement provided for the issuance of Series A Preferred Units, Series B Preferred Units, Series C Preferred Units, Series D Preferred Units, Founder Units, Common Units and Management Incentive Units.

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**Notes to Combined and Consolidated Financial Statements**

Outstanding Preferred Units, Founder Units and Common Units consist of the following:

	Units Issued and Outstanding	Original Issue Price Per Unit	Carrying Value	Liquidation Preference
(in thousands, except unit and per unit amounts)				
Series A Preferred Units	24,935,281	\$ 0.2627	\$ 4,919	\$ 12,625
Series B Preferred Units	90,909,090	\$ 0.4400	39,766	43,905
Series C Preferred Units	103,562,552	\$ 0.9656	99,182	101,676
Total Preferred Units as of December 31, 2017	219,406,923		143,867	158,206
Founder Units	11,420,741	\$ —	1,754	5,783
Common Units	5,856,075	\$ —	1,431	2,966
Total outstanding units as of December 31, 2017	<u>236,683,739</u>		<u>\$147,052</u>	<u>\$ 166,955</u>

	Units Issued and Outstanding	Original Issue Price Per Unit	Carrying Value	Liquidation Preference
(in thousands, except unit and per unit amounts)				
Series A Preferred Units	24,935,281	\$ 0.2627	\$ 4,919	\$ 13,542
Series B Preferred Units	90,909,090	\$ 0.4400	39,766	47,096
Series C Preferred Units	141,155,758	\$ 0.9656	135,482	147,155
Series D Preferred Units	150,955,597	\$ 1.9823	298,698	299,239
Total Preferred Units as of December 31, 2018	407,955,726		478,865	507,032
Founder Units	11,420,741	\$ —	1,754	6,202
Common Units	7,197,783	\$ —	1,619	3,910
Total outstanding units as of December 31, 2018	<u>426,574,250</u>		<u>\$482,238</u>	<u>\$ 517,144</u>

As of December 31, 2017 and 2018, BridgeBio has classified all of its outstanding Preferred Units, Founder Units, Common Units, and Management Incentive Units outside of members' deficit in the accompanying combined and consolidated financial statements because these units contain certain redemption features that are not solely within the control of BridgeBio. Specifically, in the event an IPO does not take place by a pre-defined date, the majority preferred unitholders could force a "liquidation event" that is not solely within BridgeBio's control. As of December 31, 2017 and 2018, the Company did not adjust the carrying values of the Preferred Units, Founder Units and Common Units to their deemed liquidation values of such units since a liquidation event was not probable as of the combined and consolidated balance sheet dates. Subsequent adjustments to increase or decrease the carrying values to the ultimate liquidation values will be made if and when it becomes probable that such a liquidation event will occur.

The significant rights, preferences and privileges of the Preferred Units, Founder Units, Common Units and Management Incentive Units are as follows:

**Voting**

Preferred Unit members do not have any voting rights, other than specified consent rights as set forth in the LLC Agreement. Except as described below, management of BridgeBio is delegated to the board of BridgeBio. The board consists of seven managers: the Company's chief executive officer (the "Founder Manager"), two individuals designated by the holders of a majority of the Series B Preferred Units, two individuals designated by certain holders of the Series D Preferred Units, an individual designated by the Founder Manager, and an

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**Notes to Combined and Consolidated Financial Statements**

independent manager to be designated by any then-serving manager and approved by majority vote of the then-serving managers.

Prior written approval of holders of a majority of the outstanding Preferred Units (other than the Series A Preferred Units) is required for the Company to perform the following, with certain exceptions: issue any new series of units with rights, preferences or privileges senior to or pari passu with any of the Preferred Units, amend any existing unit in a manner that would make such units pari passu or senior to any existing Preferred Unit, amend the Certificate of Formation or the LLC Agreement in a manner adverse to the powers, preferences or rights of the Preferred Units, enter into a Fundamental Transaction, redeem or repurchase any existing Preferred Unit (other than repurchases in connection with the cessation of employment), change the number of managers constituting the board, incur indebtedness in excess of \$100,000,000, adopt or amend equity incentive plans, enter into certain transactions between the Company or its subsidiaries and any member (or affiliate of a member) of the Company, or cause the Company to have to register as an investment company or investment adviser within the meaning of the Investment Company Act and Investment Advisers Act, respectively.

Prior written approval of holders of a majority of the outstanding Series D Preferred Units is required for the Company to amend the Certification of Formation or LLC Agreement in a manner adverse to the Series D Preferred Units, issue Series D Preferred Units other than pursuant to the Series D Agreement, waive the treatment of a transaction as a Fundamental Transaction with respect to the Series D Preferred Units or consummate an underwritten IPO other than a qualifying IPO per the terms of the LLC Agreement. The approval of holders of a majority of the outstanding Series C Preferred Units is required to amend the Certification of Formation or LLC Agreement in a manner adverse to the Series C Preferred Units or to issue additional Series C Preferred Units. The approval of holders of a majority of the outstanding Series B Preferred Units is required to amend the Certification of Formation or LLC Agreement in a manner adverse to the Series B Preferred Units or to issue additional Series B Preferred Units.

***Conversion***

There are no voluntary conversion features for the Preferred Units.

The board of BridgeBio may consummate an IPO and upon the approval of the majority of preferred members (and holders of a majority of the Series D Preferred Units if such IPO is not a qualifying IPO per the terms of the LLC Agreement), each Preferred Unit will be mandatorily convertible into common stock or other securities of the corporation used to effect the IPO (the "IPO Corporation"). In connection with any such transaction, unless the Company continues as a holding company for the IPO Corporation, the members shall receive, in exchange for their respective units, either (x) shares of common stock or other equity interests of the IPO Corporation which are of the type offered and sold to the public in such IPO and have substantially the same relative economic interest in such corporate or other entity, or (y) equity interests of a holding company or companies which, together with any remaining interests in the Company and any securities received in the IPO Corporation, have substantially the same relative economic interest if each member receives in exchange for its units (of any class) an amount of the common stock or other equity securities of such IPO Corporation having a fair market value equal to the amount that would have been distributed to such member pursuant to distribution provisions of the LLC Agreement (determined by the valuation of the capital stock of the IPO Corporation based on the offering price to the public in the IPO), or, if for less than all of the units in accordance with clause (y) above, the Implied Equity Value. Implied Equity Value is defined on any date of determination as the amount of distributions that would be received by a member with respect to such units, as applicable, following (a) a hypothetical sale of all of the Company's assets at the implied gross value and (b) a distribution by the Company of all net proceeds from such hypothetical sale to the members in accordance with the distribution rights stated below.

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If, at any time following March 26, 2021, an IPO Corporation has not consummated an IPO, then the Majority Preferred Members may seek to cause the Company or an IPO Corporation to effect (a) an IPO in respect of the Company under the Securities Act by delivering written notice to the board, (b) a merger, sale, reorganization or recapitalization of the Company that would result in a Fundamental Transaction, by delivering written notice to the board or (c) in consultation with the board, a fully auctioned merger, sale, reorganization or recapitalization that would result in the sale of one or more entities in which the Company owns an equity interest.

***Distributions***

BridgeBio's board has the authority to determine the amount, if any, of proceeds available for distribution to the unitholders and such amount would be distributed in accordance with the following priorities pursuant to the LLC Agreement:

- First, to the Series D members, pro rata among them in proportion to the amounts due to each member, an amount with respect to each Series D unit, until aggregate distributions are equal to the Series D unit value of \$1.9823 per unit;
- Second, to the Series C Preferred Unit members and the Series B Preferred Unit members, pro rata among them in proportion to the amounts due to such member with respect to such distributions, until the aggregate distributions are equal to the Series C unit value and Series B unit value, respectively. The Series C unit value is \$0.9656 per unit plus an amount between \$0.0477 and \$0.1014 based on the issuance date of the unit. The Series B unit value is \$0.44 per unit plus an amount between \$0.0575 and \$0.1044 based on the issuance date of the unit.
- Third, to the Series A Preferred Unit members, pro rata among them in proportion to the amounts due to such member with respect to such distributions until the aggregate distributions are equal to the Series A unit amount of \$0.44 per unit plus an amount between \$0.1026 and \$0.1044 based on the issuance date of the unit;
- Fourth, to the Founder Unit members and the Common Unit members pro rata among them in proportion to the amounts due to such member with respect to such distributions until the aggregate distributions are equal to the unit amount of \$0.44 per Founder Unit and Common Unit plus an amount between \$0.1026 and \$0.1044 based on the issuance date of the unit.
- Fifth, to the non-Series D Preferred Unit members pro rata in proportion to their non-Series D percentage interests, provided that: (a) no Series C member will share in any subsequent distributions once the aggregate distributions made to all Series C units are equal to \$339.5 million; (b) no Series B member will share in any subsequent distributions once the aggregate distributions made to all Series B units are equal to \$170.9 million; (c) no Series A member will share in any subsequent distributions once the aggregate distributions made to all Series A units are equal to \$47.5 million; (d) no Founder Unit member will share in any distribution once the aggregate distributions made to all Founder Units are equal to \$21.8 million; (e) no Common Unit member will share in any distribution once the aggregate distributions made to all Common Units are equal to the \$17.3 million; and (f) no Management Incentive Unit member will share in any distribution once the aggregate distributions made to all Management Incentive Units are equal to \$103.0 million; provided further that no member will share in any distribution with respect to any Management Incentive Unit until after the point at which the aggregate distributions to all units exceed the amount of such Management Incentive Unit participation threshold; and
- Sixth, to the members pro rata in proportion to their percentage interests; provided that no member will share in any distribution with respect to any Management Incentive Unit until after the point at which

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**Notes to Combined and Consolidated Financial Statements**

the aggregate distributions previously and currently made to all units exceed the amount of the Management Incentive Unit participation threshold.

Unit values are based on issuance date of the underlying unit and reflect the cumulative returns for such unit that have accrued through to the date of the LLC Agreement and shall no longer accumulate subsequent to such agreement.

***Liquidation***

In the event of any liquidation, dissolution, or winding-up of BridgeBio, the assets of BridgeBio will be distributed in accordance with the same order of priority as distributions (discussed above), but first to the creditors of BridgeBio in satisfaction of BridgeBio's liabilities.

***Founder Units***

Founder Unit members do not have any voting rights. Founder Units issued are not subject to any vesting conditions.

***Common Units***

Common Units do not have any voting rights. Common Units are generally subject to vesting over a period of up to five years as discussed in Note 14.

***Management Incentive Units***

Management Incentive Units do not have any voting rights. Management Incentive Units are generally subject to vesting over a period of up to five years as discussed in Note 14.

***MyoKardia Distribution***

In April 2015, the Company acquired an interest in MyoKardia, Inc. for \$1.0 million. This investment was subsequently disposed of in 2016 and the Company recognized a gain on disposal of \$1.2 million in the year ended December 31, 2016. Prior to the execution of the Merger described in Note 2, the Company distributed the \$1.2 million to its members in proportion to the number of units then outstanding. Pursuant to the Merger terms, the Company was required to distribute the remaining proceeds of \$1.0 million. This liability is included in accrued distributions to unitholders in the combined and consolidated balance sheets as of December 31, 2017 and 2018. The accrued distributions were paid to unitholders in February 2019.

**BRIDGEBIO PHARMA LLC****Notes to Combined and Consolidated Financial Statements****14. Equity-Based Compensation**

The Company recorded equity-based compensation in the following expense categories in its combined and consolidated statements of operations for employees and non-employees:

	December 31, 2017			Total
	BridgeBio	Eidos	Other	
	(in thousands)			
Research and development	\$ —	\$ 519	\$ 7	\$ 526
General and administrative	541	629	145	1,315
Total equity-based compensation	<u>\$ 541</u>	<u>\$1,148</u>	<u>\$152</u>	<u>\$1,841</u>

	December 31, 2018			Total
	BridgeBio	Eidos	Other	
	(in thousands)			
Research and development	\$ —	\$1,325	\$186	\$1,511
General and administrative	3,183	1,201	172	4,556
Total equity-based compensation	<u>\$ 3,183</u>	<u>\$2,526</u>	<u>\$358</u>	<u>\$6,067</u>

For the years ended December 31, 2017 and 2018, total BridgeBio equity-based compensation from Common Units was \$0.3 million and \$0.3 million, and from Management Incentive Units was \$0.2 million and \$2.9 million, respectively.

As a result of the execution of the LLC Agreement in November 2018 discussed in Note 13, BridgeBio concluded that all issued and outstanding Common Units and Management Incentive Units were modified. For Common Units, there was a decrease in fair value and no adjustment to the equity-based compensation expense was recorded in the combined and consolidated statement of operations for the year ended December 31, 2018. For Management Incentive Units, the modification resulted in an increase in the fair value of \$0.09 per outstanding unit and resulted in the recognition of incremental equity-based compensation expense of \$1.6 million in 2018 and incremental future equity-based compensation expense of \$2.7 million to be recognized over the remaining vesting period of the Management Incentive Units. There were no changes to the vesting terms or classification of the Common Units of Management Incentive Units as a result of the modification.

***BridgeBio Common Units and Management Incentive Units***

BridgeBio's Second Amended and Restated Limited Liability Company Agreement, Third Amended and Restated Limited Liability Company Agreement and LLC Agreement provided for the issuance of Management Incentive Units and Common Units to employees and non-employees. During 2017 and 2018, BridgeBio issued Management Incentive Units and Common Units based on the approval of the board of BridgeBio for each grant date.

Under the terms of the Management Incentive Units' agreements, the vesting schedule is typically 1/60th of the total number of Management Incentive Units, which vest on each monthly anniversary of the vesting commencement date, subject to continued service to BridgeBio. If a Fundamental Transaction takes place, the remaining vesting related to the Management Incentive Units and Common Units will accelerate. Under the terms of the Common Units' agreements, the vesting schedule is typically between two and five years with vesting taking place on each monthly anniversary of the vesting commencement date, subject to continued service to BridgeBio through the applicable vesting date.



**BRIDGEBIO PHARMA LLC****Notes to Combined and Consolidated Financial Statements**

No distributions can be made to the holders of Management Incentive Units until the aggregate distributions made to other members (Preferred Unit, Founder Unit and Common Unit members) exceed the Management Incentive Units' participation threshold. BridgeBio has determined that the underlying terms and intended purpose of the Management Incentive Units and Common Units are more akin to an equity-based compensation for employees and non-employees than a performance bonus or profit-sharing arrangement.

The estimated grant-date fair value of each Common Unit and Management Incentive Unit award was calculated using the Black-Scholes option pricing model, based on assumptions as follows:

	Year Ended December 31,	
	2017	2018
Expected term (in years)	1.5	0.75-1.5
Expected volatility	45.0%	40.0%-49.0%
Risk-free interest rate	1.70%	1.70%-2.56%
Dividend yield	—	—

The fair value of each Common Unit and Management Incentive Unit award was determined using the methods and assumptions discussed below. Each of these inputs is subjective and generally requires significant judgement and estimation.

*Fair value of Management Incentive Units and Common Units*—Because there is no public market for BridgeBio's units as BridgeBio is a private company, BridgeBio's board has determined the fair value of Common Units and Management Incentive Units by considering a number of objective and subjective factors, including having contemporaneous and retrospective valuations of its equity performed by a third-party valuation specialist, valuations of comparable peer public companies, sales of BridgeBio's redeemable convertible preferred units, operating and financial performance, the lack of liquidity of BridgeBio's units and general and industry-specific economic outlook.

*Expected term*—The expected term is based on BridgeBio's expectations with regard to an exit strategy such as an IPO or liquidation event.

*Expected volatility*—BridgeBio has been a private company and lacks company-specific historical and implied volatility information. Therefore, it estimates its expected share volatility based on the historical volatility of a set of publicly traded peer companies and expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded share price.

*Risk-free interest rate*—The risk-free interest rate is determined by reference to the United States Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award.

*Expected dividend*—The dividend yield was assumed to be immaterial based on future distribution expectations throughout the expected term.

Each of the above inputs is subjective and generally requires significant judgement and estimation.

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**Notes to Combined and Consolidated Financial Statements**

The following table summarizes BridgeBio's authorized Common Units activity:

	<b>Number of Authorized Units</b>
Balance as of January 1, 2017	9,098,522
Granted	2,390,000
Cancelled	<u>(2,390,000)</u>
Balance as of December 31, 2017	9,098,522
Granted	—
Cancelled	—
Balance as of December 31, 2018	<u>9,098,522</u>

The following table summarizes BridgeBio's Common Units activity:

	<b>Number of Common Units Outstanding</b>	<b>Weighted- Average Grant Date Fair Value</b>
Balance as of January 1, 2017	4,514,367	\$ 0.08
Vested	<u>1,341,708</u>	<u>\$ 0.08</u>
Balance as of December 31, 2017	5,856,075	\$ 0.08
Vested	<u>1,341,708</u>	<u>\$ 0.08</u>
Balance as of December 31, 2018	<u>7,197,783</u>	<u>\$ 0.08</u>

As of December 31, 2018, there were 1,900,739 unvested Common Units and total unrecognized compensation related to the unvested Common Units was \$0.1 million, which the Company expects to be recognized over a weighted-average period 1.3 years. All unvested Common Units as of December 31, 2018 will vest through May 2020.

The following table summarizes BridgeBio's authorized Management Incentive Units activity:

	<b>Number of Authorized Units</b>
Balance as of January 1, 2017	24,081,718
Authorized and granted	<u>21,346,384</u>
Balance as of December 31, 2017	45,428,102
Authorized and granted	3,275,000
Cancelled	<u>(7,500)</u>
Balance as of December 31, 2018	<u>48,695,602</u>

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## Notes to Combined and Consolidated Financial Statements

The following table summarizes BridgeBio's Management Incentive Units activity:

	Number of Management Incentive Units Outstanding	Weighted- Average Grant Date Fair Value
Balance as of January 1, 2017	3,588,901	\$ 0.03
Vested	6,247,024	\$ 0.06
Balance as of December 31, 2017	9,835,925	\$ 0.05
Vested	9,281,703	\$ 0.11
Balance as of December 31, 2018	<u>19,117,628</u>	<u>\$ 0.08</u>

As of December 31, 2018, there were 29,577,974 unvested Management Incentive Units and unrecognized compensation related to the unvested Management Incentive Units was \$8.8 million, which the Company expects to recognize over a weighted-average period of 3.9 years. All unvested Management Incentive Units as of December 31, 2018 will vest through October 2023.

**Eidos**2016 Equity Incentive Plan

In April 2016, Eidos established its 2016 Equity Incentive Plan (the "Eidos 2016 Plan"), which provides for the granting of equity awards to employees and consultants of Eidos. Awards granted under the Eidos 2016 Plan may be either incentive stock options ("ISOs"), nonqualified stock options ("NSOs") or restricted stock awards. ISOs may be granted only to Eidos employees (including officers and directors who are also employees). NSOs may be granted to Eidos employees and consultants. The exercise price of an ISO and NSO shall not be less than 100% of the estimated fair value of the shares on the date of grant, as determined by the Eidos Board of Directors. The exercise price of an ISO granted to an employee who at the time of grant is a 10% stockholder shall not be less than 110% of the estimated fair value of the shares on the date of grant, as determined by the Eidos Board of Directors. To date, ISOs and NSOs have a term of ten years and generally vest over a four-year period with annual cliff vesting and the balance monthly over 36 months. Upon completion of the Eidos IPO, the remaining shares available for issuance under the Eidos 2016 Plan were retired.

Amended and Restated 2018 Stock Option and Incentive Plan

In May 2018, the Eidos Board of Directors and stockholders approved the Amended and Restated 2018 Stock Option and Incentive Plan (the "Eidos 2018 Plan"), to replace the Eidos 2016 Plan. The Eidos 2018 Plan became effective upon the Eidos IPO and is administered by the Eidos Board of Directors or an appointed committee, which determines the types of awards to be granted, including the number of shares subject to the awards, the exercise price and the vesting schedule. Under the Eidos 2018 Plan, 598,000 shares of Eidos' common stock have been initially reserved for the issuance of stock options, restricted stock units and other awards to employees, directors and consultants. Options granted under the Eidos 2018 Plan expire no later than 10 years from the date of grant. The exercise price of each option may not be less than 100% of the fair market value of the common stock at the date of grant. Options may be granted to stockholders possessing more than 10% of the total combined voting power of all classes of stocks of Eidos at an exercise price at least 110% of the fair value of the common stock at the date of grant and the options are not exercisable after the expiration of 10 years from the date of grant. Employee stock options generally vest 25% upon one year of continued service to Eidos, with the remainder in monthly increments over three additional years. Upon adoption of the Eidos 2018 Plan, no additional stock awards will be issued under the Eidos 2016 Plan. Options granted under the Eidos 2016

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Plan that were outstanding on the date the Eidos 2018 Plan became effective remain subject to the terms of the Eidos 2016 Plan. In December 2018, the Eidos Board of Directors approved an increase in the number of shares reserved under the Eidos 2018 Plan by 700,000 shares. As of December 31, 2018, Eidos has reserved 1,298,000 shares of common stock for issuance under the 2018 Plan, of which the 700,000 shares subject to the December 2018 increase remain subject to stockholder approval.

*Employee Stock Purchase Plan*

In May 2018, the Eidos Board of Directors and stockholders approved the 2018 Employee Stock Purchase Plan (the “Eidos 2018 ESPP”), which became effective upon the Eidos IPO. The Eidos 2018 ESPP is intended to qualify as an employee stock purchase plan under Section 423 of the Internal Revenue Code of 1986, as amended, and is administered by the Eidos Board of Directors and the Compensation Committee of the Eidos Board of Directors. Under the Eidos 2018 ESPP, 143,520 shares of Eidos’ common stock have been initially reserved for employee purchases of Eidos’ common stock. The Eidos 2018 ESPP allows eligible employees to purchase shares of Eidos’ common stock at a discount through payroll deductions of up to 20% of their eligible compensation. At the end of each offering period, employees are able to purchase shares at 85% of the lower of the fair market value of Eidos’ common stock at the beginning of the offering period or at the end of each applicable purchase period. The first purchase period commenced upon the completion of the Eidos IPO and ended on November 30, 2018.

The fair value of the rights granted under the Eidos 2018 ESPP was calculated using the Black-Scholes option-pricing model with the following assumptions:

	<b>Year Ended December 31, 2018</b>
Expected term (in years)	0.48
Expected volatility	70.4%
Risk-free interest rate	1.50%
Dividend yield	—

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Notes to Combined and Consolidated Financial Statements

Stock Options

Activity under the Eidos equity incentive plans is set forth below:

	Options Available for Grant	Options Outstanding	Weighted-Average Exercise Price per Option	Weighted-Average Remaining Contractual Life (years)	Aggregate Intrinsic Value
(in thousands, except per share and per share data)					
Outstanding as of January 1, 2017	643,922	30,068	\$ 0.15	9.62	\$ —
Additional authorized	1,495,000	—	—		
Granted	(1,467,928)	1,467,928	0.55		
Exercised	—	(651,830)	0.48		
Outstanding as of December 31, 2017	670,994	846,166	\$ 0.59	9.97	\$ 4,384
Granted	(399,074)	399,074	5.03		
Cancelled	321,916	(321,916)	0.59		
Retired	(593,836)	—	—		
Additional authorized	1,298,000	—	—		
Granted	(696,364)	696,364	15.45		
Exercised	—	(184,871)	1.48		
Exercised options repurchased	40,366	—	0.33		
Cancelled	105,055	(105,055)	13.67		
Outstanding as of December 31, 2018	747,057	1,329,762	\$ 8.55	9.40	\$ 6,928
Vested and expected to vest as of December 31, 2018		1,329,762	\$ 8.55	9.40	\$ 6,928
Exercisable as of December 31, 2018		161,208	\$ 1.34	8.96	\$ 2,002

Aggregate intrinsic value represents the difference between Eidos' estimated fair value of its common stock and the exercise price of outstanding in-the-money options. The total intrinsic value of options exercised was \$2.9 million and \$0.9 million for the years ended December 31, 2017 and 2018, respectively.

The total fair value of shares vested during the years ended December 31, 2017 and 2018 was \$0.5 million and \$2.5 million, respectively.

Stock Options Valuation

The fair value of Eidos' shares of common stock underlying its stock options has historically been determined by Eidos' Board of Directors prior to the Eidos IPO. Because there had been no public market for Eidos' common stock, Eidos' Board of Directors had determined fair value of the common stock at the time of grant of the option by considering a number of objective and subjective factors including important developments in Eidos' operations, valuations performed by an independent third-party, sales of redeemable convertible preferred stock, actual operating results and financial performance, the conditions in the biotechnology industry and the economy in general, the stock price performance and volatility of comparable public companies, and the lack of liquidity of Eidos' common stock, among other factors. For stock options granted after the completion of the Eidos IPO, Eidos determines the fair value of each share of underlying common stock based on the closing price of Eidos' common stock as reported on the date of grant.

The determination of the fair value of equity-based payment awards on the date of grant is affected by the stock price, as well as assumptions regarding a number of complex and subjective variables. These variables

**BRIDGEBIO PHARMA LLC**

**Notes to Combined and Consolidated Financial Statements**

include expected stock price volatility over the term of the awards, actual and projected employee/consultant stock option exercise behaviors, risk-free interest rates, and expected dividends. Estimating the fair value of equity-settled awards as of the grant date using valuation models, such as the Black-Scholes option pricing model, is affected by assumptions regarding a number of complex variables. These inputs include:

*Expected term*—The expected term represents the period that the stock-based awards are expected to be outstanding. Since Eidos does not have a long trading history for its common stock, the expected term is estimated based on the average expected term for comparable publicly traded biopharmaceutical companies. The comparable companies were chosen based on their similar size, stage in the life cycle or area of specialty. For non-employees, the term is the remaining contractual term of the option.

*Expected volatility*—Since Eidos does not have a long trading history for its common stock, the expected volatility is estimated based on the average volatility for comparable publicly traded biopharmaceutical companies over a period equal to the expected term of the stock option grants. The comparable companies were chosen based on their similar size, stage in the life cycle or area of specialty.

*Risk-free interest rate*—The risk-free interest rate is based on the United States Treasury zero coupon issues in effect at the time of grant for periods corresponding with the expected term of option.

*Expected dividend*—Eidos has never paid dividends on its common stock and has no plans to pay dividends on its common stock. Therefore, Eidos used an expected dividend yield of zero.

The fair value of employee and non-employee director stock option awards was estimated at the date of grant using a Black-Scholes option-pricing model with the following assumptions:

	Year Ended December 31, 2017		Year Ended December 31, 2018	
	Employee	Non-employee	Employee	Non-employee
Expected term (in years)	5.83	9.66	6.08	9.20
Expected volatility	68.4%	80.1%	72.0%	73.9%
Risk-free interest rate	2.27%	2.41%	2.87%	2.66%
Dividend yield	—	—	—	—

The weighted average fair value of share-based awards granted to employees during the years ended December 31, 2017 and 2018 was \$4.79 per share and \$8.46 per share, respectively.

During the years ended December 31, 2017 and 2018, Eidos granted 569,252 and 35,880 shares, respectively, to non-employee consultants. Eidos recognized stock-based compensation expense for non-employee awards during the years ended December 31, 2017 and 2018 of \$0.7 million and \$1.7 million, respectively.

Restricted Stock

In December 2017, Eidos issued 390,546 shares of common stock for no consideration to the founders pursuant to Eidos' Series Seed Preferred Stock Purchase Agreement and license agreement in connection with certain anti-dilution rights held by these parties. If the shares issued under the license agreement represent less than 1% of the shares issued and outstanding of common stock on an as-converted basis, Eidos will issue additional common stock to the founders and Stanford University. Eidos has the right to repurchase the common stock at the fair value per share on the date of repurchase; this repurchase right lapses as the shares vest. The shares cliff vest 25% after one year and vest monthly thereafter over 36 months. As of December 31, 2017 and 2018, 390,546 and 268,504 shares remain subject to repurchase.

**BRIDGEBIO PHARMA LLC****Notes to Combined and Consolidated Financial Statements**

Eidos recognizes stock-based compensation expense upon the approval of these awards by the Eidos Board of Directors in September 2017 as vesting provisions are not considered substantive due to the fair value repurchase right. Stock-based compensation expense related to the restricted stock is recognized based on the fair value of the stock on the approval date using the Black-Scholes pricing model. During the years ended December 31, 2017 and 2018, Eidos recognized expense related to these awards of \$0.2 million and nil, respectively.

Equity-Based Compensation

As of December 31, 2018, there was \$10.8 million of total unrecognized compensation cost related to unvested equity-based compensation arrangements under the Eidos 2016 Plan and Eidos 2018 Plan. The unrecognized equity-based compensation cost is expected to be recognized over a weighted-average period 3.3 years.

**15. Income Taxes**

BridgeBio is a “pass-through” entity under the Code and the members are taxed directly on their respective ownership interests in the combined and consolidated income. Therefore, no provision or liability for federal income tax has been included in the accompanying combined and consolidated financial statements related to BridgeBio.

For the Company’s consolidated entities, income taxes are accounted for in accordance with authoritative guidance, which requires the use of the asset and liability method. Under this method, deferred income tax assets and liabilities are determined based upon the difference between the consolidated financial statement carrying amounts and the tax basis of assets and liabilities and are measured using the enacted tax rate expected to apply to taxable income in the years in which the differences are expected to be reversed.

The following table presents the components of net loss before income taxes:

	<b>December 31,</b>	
	<b>2017</b>	<b>2018</b>
	<b>(in thousands)</b>	
Domestic	\$43,832	\$169,451
Foreign	—	—
Total loss before income taxes	<u>\$43,832</u>	<u>\$169,451</u>

The following table presents a reconciliation of the statutory federal rate and the Company’s effective tax rate:

	<b>Year Ended</b>	
	<b>December 31,</b>	
	<b>2017</b>	<b>2018</b>
Tax at statutory federal rate	34.0%	21.0%
State income taxes, net of federal benefit	—	—
Change in valuation allowance	(16.4)	(20.2)
Other	(1.7)	(0.8)
Impact of tax reform	<u>(15.9)</u>	<u>—</u>
Effective income tax rate	<u>— %</u>	<u>— %</u>

## BRIDGEBIO PHARMA LLC

## Notes to Combined and Consolidated Financial Statements

Deferred income taxes reflect the net tax effects of temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. The following table presents the significant components of the Company's deferred tax assets and liabilities for the periods presented:

	December 31,	
	2017	2018
	(in thousands)	
Deferred tax assets:		
Net operating loss carry-forwards	\$ 14,590	\$ 40,896
Amortization	1,501	4,424
Accruals and reserves	762	434
Equity-based compensation	412	268
Tax credits	649	3,728
Other	—	23
Gross deferred tax assets	17,914	49,773
Less valuation allowance	(15,914)	(49,755)
Deferred tax assets, net of valuation allowance	2,000	18
Deferred tax liabilities:		
Fixed assets	(4)	(18)
Other	(12)	—
Federal benefit of state	(909)	—
Prepaid expenses	(1,075)	—
Deferred tax liabilities	(2,000)	(18)
Net deferred tax assets	\$ —	\$ —

As of December 31, 2018, the Company has net operating loss carryforwards available to reduce future taxable income, if any, for federal and California state income tax purposes of approximately \$157.5 million and \$101.8 million. The net operating losses will begin to expire in 2033.

As of December 31, 2018, the Company has federal research and development credit carryforwards of \$4.2 million, which will expire beginning in 2033 if not utilized. As of December 31, 2018, the Company has California research and development tax credit carryforwards of \$0.8 million. The California research and development tax credits have no expiration date.

A valuation allowance is provided for deferred tax assets where the recoverability of the assets is uncertain. The determination to provide a valuation allowance is dependent upon the assessment of whether it is more likely than not that sufficient future taxable income will be generated to utilize the deferred tax assets. Based on the weight of the available evidence, which includes the Company's historical operating losses and forecast of future losses, the Company provided a full valuation allowance against the deferred tax assets resulting from the tax loss and credits carried forward.

Utilization of the net operating loss and credit carryforwards may be subject to a substantial annual limitation due to an ownership change limitation as provided by section 382 of the Internal Revenue Code of 1986, as amended, and similar state provisions. The annual limitation may result in the expiration of net operating losses and credits before utilization. In the event that the Company had a change of ownership, utilization of the net operating loss and tax credit carryforwards may be restricted.



**BRIDGEBIO PHARMA LLC****Notes to Combined and Consolidated Financial Statements**

A reconciliation of the beginning and ending amount of unrecognized tax benefits is as follows:

	As of December 31,	
	2017	2018
	(in thousands)	
Balance at the beginning of the year	\$ 37	\$ 296
Reversal of prior year positions	—	(42)
Additions based on tax positions related to current year	259	928
Balance at the end of the year	<u>\$ 296</u>	<u>\$ 1,182</u>

As of December 31, 2018, the Company has not recorded interest and penalties associated with its unrecognized tax benefits.

The Company's unrecognized gross tax benefits would not reduce the annual effective tax rate if recognized because it has recorded a full valuation allowance on its deferred tax assets. The Company does not foresee any material changes to the gross unrecognized tax benefit within the next twelve months. The Company's policy is to recognize interest and penalties related to income tax matters in income tax expense.

The Company files federal, California and Massachusetts income tax returns. The Company currently has no federal or state tax examinations in progress. All years are open for examination by federal and state authorities.

In December 2017, the United States government enacted comprehensive tax legislation commonly referred to as the Tax Cuts and Jobs Act (the "Tax Act") and the new legislation contains several key provisions, including a reduction of the federal corporate income tax rate to 21% effective January 1, 2018. The Company is required to recognize the effect of the tax law changes in the period of enactment, such as remeasuring its United States deferred tax assets and liabilities as well as its valuation allowance against its net United States deferred tax assets. In December 2017, the U.S. Securities and Exchange Commission staff issued Staff Accounting Bulletin No. 118 ("SAB 118"), which allowed the Company to record provisional amounts during a measurement period not to extend beyond one year of the enactment date. During the fourth quarter of 2018, the Company completed its accounting for the Tax Act as summarized below.

Due to the change in the statutory tax rate from the Tax Act, the Company remeasured its federal deferred tax assets as of December 31, 2017 to reflect the reduced rate that will apply in future periods when these deferred taxes are settled or realized. The result was a decrease of \$6.8 million to deferred tax assets. No adjustments were made to the provisional estimates recorded.

The Company determined the one-time transition tax would not be applicable given the Company's facts and circumstances. The one-time transition tax would be based on total post-1986 foreign earnings and profits that were previously deferred from United States income tax. The applicable tax rate is based on the amount of those post-1986 earnings that is held in cash and other specified assets (the "cash position"). BridgeBio does not have any foreign earnings and profits and thus the Company would not have any transition tax liability. The Company's position has not changed.

The Tax Act contains various provisions that went into effect as of January 1, 2018. The Company has analyzed all provisions enacted as of January 1, 2018 and determined that the newly enacted provisions do not materially impact the Company. The Company will continue to monitor all provisions for applicability in future periods.

BRIDGEBIO PHARMA LLC

Notes to Combined and Consolidated Financial Statements

16. Net Loss per Unit and Unaudited Pro Forma Net Loss per Share

The following table sets forth the calculation of basic and diluted net loss per unit:

	Year Ended December 31,	
	2017	2018
	(in thousands, except unit and per unit data)	
<b>Numerator:</b>		
Net loss attributable to BridgeBio	\$ (30,565)	\$ (130,749)
Cumulative returns on redeemable convertible preferred units (Series A, Series B and Series C)	(5,672)	(13,287)
Net loss attributable to Founder Units and Common Units	<u>\$ (36,237)</u>	<u>\$ (144,036)</u>
<b>Denominator:</b>		
Total weighted-average Founder Units and Common Units used in computing net loss per unit, basic and diluted	<u>16,650,073</u>	<u>17,991,781</u>
<b>Net Loss per Unit:</b>		
Net loss attributable to Founder Units and Common Units, basic and diluted	<u>\$ (2.18)</u>	<u>\$ (8.01)</u>

The following outstanding units were excluded from the computation of the diluted net loss per unit for the periods presented because their effect would have been anti-dilutive.

	Year Ended December 31,	
	2017	2018
Preferred Units	219,406,923	407,955,726
Management Incentive Units	45,428,102	48,695,602
Unvested Common Units	<u>3,242,447</u>	<u>1,900,739</u>
Total	<u>268,077,472</u>	<u>458,552,067</u>

**Unaudited Pro Forma Net Loss per Share**

The unaudited pro forma basic and diluted net loss per share attributable to common stockholders for the year ended December 31, 2018 has been prepared to give effect to the exchange of all Outstanding Units of BridgeBio into shares of common stock of the Corporation upon the Reorganization as if the Reorganization had occurred on the later of beginning of period or the issuance date of the Outstanding Units.

Upon conversion to a corporation, the Company will become subject to U.S. federal and state income taxes. Based on the Company's history of generating operating losses and its anticipation of operating losses continuing in the foreseeable future, the Company has determined that it is more likely than not that the tax benefits from its operating losses would not be realized and has determined that a full valuation allowance against its net deferred tax assets would be recorded on a pro forma basis. The Company also determined that any outside basis differences in its controlled entities would result in a deferred tax asset which would not be recognized since it would not reverse in the foreseeable future. With respect to its investment in PellePharm, which is accounted for under the equity method, a deferred tax asset is recorded but is subject to a valuation allowance and thus has no impact on a pro forma basis. Therefore, for the purposes of the pro forma tax provision, the Company has not recorded an income tax expense or benefit for the net losses incurred by the Company during the year ended December 31, 2018.

**BRIDGEBIO PHARMA LLC****Notes to Combined and Consolidated Financial Statements**

Unaudited pro forma basic and diluted net loss per share attributable to common stockholders was calculated as follows:

	<b>Year Ended December 31, 2018</b> <b>(in thousands, except share and per share data)</b>
<b>Numerator:</b>	
Pro forma net loss attributable to common stockholders	\$ (144,036)
<b>Denominator:</b>	
Total weighted-average common shares outstanding – basic and diluted	—
Pro forma adjustment to reflect the assumed exchange of Outstanding Units upon the Reorganization	62,087,997
Pro forma total weighted-average common shares outstanding – basic and diluted	62,087,997
<b>Net Loss per Share:</b>	
Pro forma net loss per share attributable to common stockholders – basic and diluted	\$ (2.32)

**17. Subsequent Events**

The Company evaluated subsequent events through April 15, 2019, the date on which the combined and consolidated financial statements were originally available to be issued, and has updated such evaluation for disclosure purposes through May 16, 2019, the date on which the revised combined and consolidated financial statements were available to be reissued.

***Management Incentive Unit grants***

Subsequent to December 31, 2018, the Company has granted 24,111,064 Management Incentive Units.

***Significant financing events in relation to controlled VIEs***

Subsequent to December 31, 2018, BridgeBio made additional investments in QED of \$40.0 million, Quartz of \$0.1 million, CoA of \$5.1 million, Orfan of \$1.5 million, Ferro of \$4.5 million, Aspa of \$8.0 million, Adrenas of \$8.0 million, and Navire of \$4.5 million.

***Lease Agreements***

In March 2019, Adrenas entered into a five-year agreement to rent 11,376 square feet of office space in Raleigh, North Carolina. The aggregate rent expense under the lease is \$2.4 million. The lease provides for a tenant improvement allowance for up to \$0.9 million. Upon signing the lease, Adrenas agreed to deliver the landlord two unconditional, irrevocable, transferrable letters of credit in the total amount of \$0.4 million.

In March 2019, Eidos entered into an amendment to the lease dated November 2017. In connection with the amendment Eidos will rent 10,552 square feet of office space. The amended lease term is for 87 months and has \$6.4 million of payments under this lease. In May 2019, BridgeBio entered into an amendment to the lease dated March 2017, which extends the lease term for an additional three years through April 2023. Base rent over the extended term is \$1.1 million and BridgeBio is also responsible for a monthly management fee and costs associated with certain tenant improvements.

**BRIDGEBIO PHARMA LLC**

**Condensed Consolidated Balance Sheets**  
**(unaudited)**  
**(in thousands, except units and per unit amounts)**

	December 31, 2018	March 31, 2019	Pro Forma March 31, 2019
<b>Assets</b>			
Current assets:			
Cash and cash equivalents	\$ 436,086	\$ 373,027	
Prepaid expenses and other current assets	9,137	9,446	
Total current assets	445,223	382,473	
Property and equipment, net	1,575	1,882	
PellePharm investment	17,050	12,452	
Other assets	1,093	4,567	
Total assets	<u>\$ 464,941</u>	<u>\$ 401,374</u>	
<b>Liabilities, Redeemable Convertible Preferred Units, Redeemable Founder Units, Redeemable Common Units, Management Incentive Units, Redeemable Convertible Noncontrolling Interests, Members' Deficit and Stockholders' Equity</b>			
Current liabilities:			
Accounts payable	\$ 13,509	\$ 12,344	
Accrued compensation and benefits	4,047	3,616	
Accrued research and development liabilities	8,915	10,624	
Accrued distributions to unitholders	997		
LEO call option liability	3,009	4,523	
Other accrued liabilities	2,100	4,365	
Total current liabilities	32,577	35,472	
Term loans, noncurrent	54,507	54,861	
Other liabilities	495	380	
Total liabilities	<u>87,579</u>	<u>90,713</u>	
Commitments and contingencies (Note 8)			
Redeemable convertible preferred units (Series A, Series B, Series C and Series D); no par value; 407,955,726 units authorized as of December 31, 2018 and March 31, 2019; 407,955,726 units issued and outstanding as of December 31, 2018 and March 31, 2019; aggregate liquidation value of \$507,032 as of March 31, 2019; no units authorized, issued or outstanding, pro forma as of March 31, 2019	478,865	479,044	—
Redeemable founder units; no par value; 11,420,741 units authorized as of December 31, 2018 and March 31, 2019; 11,420,741 units issued and outstanding as of December 31, 2018 and March 31, 2019; aggregate liquidation value of \$6,202 as of March 31, 2019; no units authorized, issued or outstanding, pro forma as of March 31, 2019	1,754	1,754	—
Redeemable common units; no par value; 9,098,522 units authorized as of December 31, 2018 and March 31, 2019; 7,197,783 and 7,533,210 units issued and outstanding as of December 31, 2018 and March 31, 2019; aggregate liquidation value of \$4,092 as of March 31, 2019; no units authorized, issued or outstanding, pro forma as of March 31, 2019	1,619	1,645	—
Management incentive units; no par value; 48,695,602 and 72,806,666 units authorized as of December 31, 2018 and March 31, 2019; 19,117,628 and 21,948,799 issued and outstanding as of December 31, 2018 and March 31, 2019; no units authorized, issued or outstanding, pro forma as of March 31, 2019	3,221	4,431	—
Redeemable convertible noncontrolling interests	122	202	202
Members' deficit and stockholders' equity:			
Undesignated preferred stock; \$.001 par value; no shares authorized, issued or outstanding as of December 31, 2018 and March 31, 2019; 25,000,000 shares authorized, no shares issued and outstanding, pro forma as of March 31, 2019	—	—	—
Common stock; \$.001 par value; no shares authorized, issued or outstanding as of December 31, 2018 and March 31, 2019; 500,000,000 shares authorized, 99,999,967 shares issued and outstanding, pro forma as of March 31, 2019	—	—	100
Additional paid-in capital	—	—	486,774
Accumulated deficit	(170,580)	(234,733)	(234,733)
Total BridgeBio members' deficit and stockholders' equity	(170,580)	(234,733)	252,141
Noncontrolling interests	62,361	58,318	58,318
Total members' deficit and stockholders' equity	<u>(108,219)</u>	<u>(176,415)</u>	<u>310,459</u>
Total liabilities, redeemable convertible preferred units, redeemable founder units, redeemable common units, management incentive units, redeemable convertible noncontrolling interests, members' deficit and stockholders' equity	<u>\$ 464,941</u>	<u>\$ 401,374</u>	<u>\$ 401,374</u>

*The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.*

## BRIDGEBIO PHARMA LLC

**Condensed Consolidated Statements of Operations and Comprehensive Loss**  
*(unaudited)*  
*(in thousands, except units and per unit amounts)*

	Three Months Ended March 31,	
	2018	2019
Operating expenses:		
Research and development	\$ 33,831	\$ 44,853
General and administrative	8,007	18,899
Total operating expenses	<u>41,838</u>	<u>63,752</u>
Loss from operations	(41,838)	(63,752)
Other income (expense), net:		
Interest income	1	2,107
Interest expense	(7)	(1,671)
Loss from PellePharm	—	(4,599)
LEO call option expense	—	(1,514)
Other expense	(586)	(7)
Total other income (expense), net	<u>(592)</u>	<u>(5,684)</u>
Net loss and comprehensive loss	(42,430)	(69,436)
Net loss attributable to redeemable convertible noncontrolling interests and noncontrolling interests	8,274	8,251
Net loss and comprehensive loss attributable to BridgeBio	(34,156)	(61,185)
Cumulative returns on redeemable convertible preferred units (Series A, Series B and Series C)	(3,121)	—
Net loss attributable to redeemable founder units and redeemable common units	<u>\$ (37,277)</u>	<u>\$ (61,185)</u>
Net loss per unit attributable to redeemable founder unitholders and redeemable common unitholders, basic and diluted	<u>\$ (2.13)</u>	<u>\$ (3.25)</u>
Total weighted-average redeemable founder units and redeemable common units used in computing net loss per unit, basic and diluted	<u>17,485,526</u>	<u>18,827,234</u>
Pro forma net loss per share attributable to common stockholders, basic and diluted		<u>\$ (0.66)</u>
Total weighted-average shares used in computing pro forma net loss per share, basic and diluted		<u>92,741,876</u>

*The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.*

BRIDGEBIO PHARMA LLC

Condensed Consolidated Statements of Redeemable Convertible Preferred Units, Redeemable Founder Units, Redeemable Common Units, Management Incentive Units, Redeemable Convertible Noncontrolling Interests and Members' Deficit  
(unaudited)  
(in thousands, except units and per unit amounts)

	Redeemable Convertible Preferred Units		Redeemable Founder Units		Redeemable Common Units		Management Incentive Units		Redeemable Convertible Noncontrolling Interests	Accumulated Deficit	Noncontrolling Interests	Total Members' Deficit
	Units	Amount	Units	Amount	Units	Amount	Units	Amount				
<b>Balances as of December 31, 2017</b>	219,406,923	\$ 143,867	11,420,741	\$ 1,754	5,856,075	\$ 1,431	9,835,925	\$ 226	\$ 833	\$ (61,427)	\$ 2,498	\$ (58,929)
Issuance and vesting of redeemable common units and associated equity-based compensation	—	—	—	—	335,427	47	—	—	—	—	—	—
Issuance and vesting of management incentive units and associated equity-based compensation	—	—	—	—	—	—	2,275,572	275	—	—	—	—
Issuance of noncontrolling interest	—	—	—	—	—	—	—	—	15,617	—	553	553
Transfers to (from) noncontrolling interest	—	—	—	—	—	—	—	—	(11,286)	3,876	7,410	11,286
Net loss and comprehensive loss	—	—	—	—	—	—	—	—	(3,614)	(34,156)	(4,660)	(38,816)
<b>Balances as of March 31, 2018</b>	<u>219,406,923</u>	<u>\$ 143,867</u>	<u>11,420,741</u>	<u>\$ 1,754</u>	<u>6,191,502</u>	<u>\$ 1,478</u>	<u>12,111,497</u>	<u>\$ 501</u>	<u>\$ 1,550</u>	<u>\$ (91,707)</u>	<u>\$ 5,801</u>	<u>\$ (85,906)</u>
	Redeemable Convertible Preferred Units		Redeemable Founder Units		Redeemable Common Units		Management Incentive Units		Redeemable Convertible Noncontrolling Interests	Accumulated Deficit	Noncontrolling Interests	Total Members' Deficit
	Units	Amount	Units	Amount	Units	Amount	Units	Amount				
<b>Balances as of December 31, 2018</b>	407,955,726	\$ 478,865	11,420,741	\$ 1,754	7,197,783	\$ 1,619	19,117,628	\$ 3,221	\$ 122	\$ (170,580)	\$ 62,361	\$ (108,219)
Issuance and vesting of redeemable common units and associated equity-based compensation	—	—	—	—	335,427	26	—	—	—	—	—	—
Issuance and vesting of management incentive units and associated equity-based compensation	—	—	—	—	—	—	2,831,171	1,210	—	—	—	—
Repayment of nonrecourse notes	—	179	—	—	—	—	—	—	—	—	—	—
Issuance of noncontrolling interest	—	—	—	—	—	—	—	—	—	—	1,320	1,320
Transfers to (from) noncontrolling interest	—	—	—	—	—	—	—	—	870	(2,968)	2,098	(870)
Net loss and comprehensive loss	—	—	—	—	—	—	—	—	(790)	(61,185)	(7,461)	(68,646)
<b>Balances as of March 31, 2019</b>	<u>407,955,726</u>	<u>\$ 479,044</u>	<u>11,420,741</u>	<u>\$ 1,754</u>	<u>7,533,210</u>	<u>\$ 1,645</u>	<u>21,948,799</u>	<u>\$ 4,431</u>	<u>\$ 202</u>	<u>\$ (234,733)</u>	<u>\$ 58,318</u>	<u>\$ (176,415)</u>

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

**BRIDGEBIO PHARMA LLC**
**Condensed Consolidated Statements of Cash Flows**  
*(unaudited)*  
*(in thousands)*

	Three Months Ended March 31,	
	2018	2019
<b>Operating activities</b>		
Net loss	\$(42,430)	\$ (69,436)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	26	112
Equity-based compensation	751	2,232
Loss on disposal of property and equipment, net	3	7
Loss from PellePharm	—	4,599
Accretion of term loans and convertible promissory notes	202	354
Acquired in-process research and development assets	16,886	2,000
Shares issued under license agreements	50	220
LEO call option expense	—	1,514
Change in fair value of Eidos financial instruments	416	—
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(1,673)	(309)
Other assets	(1,010)	(1,852)
Accounts payable	4,339	(1,952)
Accrued compensation and benefits	434	(432)
Accrued research and development liabilities	3,101	1,710
Other accrued liabilities	797	2,207
Other liabilities	157	(114)
Net cash used in operating activities	<u>(17,951)</u>	<u>(59,140)</u>
<b>Investing activities</b>		
Cash paid for in-process research and development assets acquired	(15,000)	(2,000)
Purchases of property and equipment	(177)	(427)
Net cash used in investing activities	<u>(15,177)</u>	<u>(2,427)</u>
<b>Financing activities</b>		
Proceeds from issuance of promissory notes	1,000	—
Proceeds from repayment of nonrecourse notes	—	179
Proceeds from term loans, net of issuance costs	2,463	—
Proceeds from third-party investors in redeemable convertible noncontrolling interests	12,199	—
MyoKardia distributions (Note 13)	—	(997)
Proceeds from repayment of the loans received by noncontrolling interest shareholder	(7)	—
Payment of deferred offering costs	—	(936)
Proceeds from subsidiary stock option exercises	38	103
Net cash provided by (used in) financing activities	<u>15,693</u>	<u>(1,651)</u>
Net decrease in cash, cash equivalents and restricted cash	(17,435)	(63,218)
Cash, cash equivalents and restricted cash at beginning of period	92,376	436,245
Cash, cash equivalents and restricted cash at end of period	<u>\$ 74,941</u>	<u>\$373,027</u>
<b>Supplemental Disclosures of Cash Flow Information:</b>		
Cash paid for interest	<u>\$ —</u>	<u>\$ 1,176</u>

	Three Months Ended March 31,	
	2018	2019
<b>Supplemental Disclosures of Non-Cash Investing and Financing Information:</b>		
Conversion of promissory note into redeemable convertible noncontrolling interest	\$ 1,005	\$ —
Transfers to (from) non-controlling interest (Note 6)	\$ 3,876	\$ 2,968
Deferred offering costs included in accounts payable and other accrued liabilities	\$ —	\$ 845
Fair value of redeemable convertible noncontrolling interest issued for acquired in-process research and development assets	\$ 1,886	\$ —

*The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.*



**BRIDGEBIO PHARMA LLC**

**Notes to Unaudited Interim Condensed Consolidated Financial Statements**

**1. Organization and Description of Business**

BridgeBio was established to identify and advance transformative medicines to treat patients who suffer from Mendelian diseases, which are diseases that arise from defects in a single gene, and cancers with clear genetic drivers. BridgeBio's pipeline of programs spans early discovery to late stage development. Since inception, BridgeBio has either created wholly-owned subsidiaries or has made investments in certain controlled entities, including partially-owned subsidiaries for which BridgeBio has a majority voting interest and VIEs for which BridgeBio is the primary beneficiary. BridgeBio is headquartered in Palo Alto, California.

***Liquidity and Going Concern***

The Company has incurred significant losses and negative cash flows from operations since its inception and has an accumulated deficit of \$234.7 million as of March 31, 2019. The Company has cash and cash equivalents of \$373.0 million as of March 31, 2019, of which \$172.8 million was held by BridgeBio. The remaining cash and cash equivalents were held by the Company's wholly-owned subsidiaries and controlled entities and these funds are designated for specific entity usage, except in limited circumstances.

The Company has historically financed its operations primarily through the sale of its equity securities and, to a lesser extent, debt borrowings. To date, none of the Company's product candidates have been approved for sale and therefore the Company has not generated any revenue from product sales. Management expects operating losses to continue for the foreseeable future. The Company believes that its existing cash and cash equivalents will be sufficient for the Company to continue as a going concern for at least one year from the issuance date of its March 31, 2019 unaudited condensed consolidated financial statements.

**2. Summary of Significant Accounting Policies**

***Unaudited Interim Financial Statements***

The unaudited interim condensed consolidated financial statements have been prepared on the same basis as the annual financial statements and, in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary to present fairly the Company's financial position as of March 31, 2019, and its results of operations and comprehensive loss, cash flows and members' deficit for the three months ended March 31, 2018 and 2019. The financial data and the other financial information contained in these notes to the condensed consolidated financial statements related to the three-month periods are also unaudited. The results of operations for the three months ended March 31, 2019 are not necessarily indicative of the results to be expected for the year ending December 31, 2019 or for any other future annual or interim period. These condensed consolidated financial statements should be read in conjunction with the Company's audited financial statements included elsewhere in this prospectus.

***Unaudited Pro Forma Balance Sheet***

Prior to the completion of its initial public offering ("IPO"), the Company will complete a series of transactions pursuant to which BridgeBio will become a wholly-owned subsidiary of the Corporation, a newly formed Delaware corporation.

As part of the Reorganization, the holders of existing units in BridgeBio will exchange those units for shares of common stock or restricted stock of the Corporation, after which those holders will have received 100% of the outstanding capital stock of the Corporation as of immediately prior to the completion of the IPO. The capital stock of the Corporation will be allocated to holders of existing units of BridgeBio pursuant to the distribution

**BRIDGEBIO PHARMA LLC**

**Notes to Unaudited Interim Condensed Consolidated Financial Statements**

provisions of the LLC Agreement of BridgeBio based upon the liquidation value of BridgeBio, assuming it was liquidated prior to the completion of this offering with a value implied by the assumed IPO price of \$15.00 per share.

The accompanying unaudited pro forma balance sheet as of March 31, 2019 has been prepared to give effect to the exchange of all Outstanding Units into 99,999,967 shares of common stock of the Corporation upon the Reorganization as if the Reorganization had occurred on March 31, 2019 based on an IPO price of \$15.00 per share.

Common Units and Management Incentive Units that have not vested as of the Reorganization will be exchanged for shares of the Corporation's restricted common stock, which will be subject to time-based vesting conditions in accordance with the terms and conditions of the Common Units and Management Incentive Units of BridgeBio from which such shares are exchanged.

***Variable Interest Entities and Voting Interest Entities***

BridgeBio consolidates those entities in which it has a direct or indirect controlling financial interest based on either the VIE model or the VOE model.

At the VIE's inception, BridgeBio determines whether it is the primary beneficiary and if the VIE should be consolidated based on the facts and circumstances. BridgeBio then performs on-going reassessments of the VIE based on reconsideration events and reevaluates whether a change to the consolidation conclusion is required each reporting period. Refer to Note 5.

Entities that do not qualify as a VIE are assessed for consolidation under the VOE model. Under the VOE model, BridgeBio consolidates the entity if it determines that it, directly or indirectly, has greater than 50% of the voting shares and that other equity holders do not have substantive voting, participating or liquidation rights. Refer to Note 5.

**BRIDGEBIO PHARMA LLC**

**Notes to Unaudited Interim Condensed Consolidated Financial Statements**

BridgeBio has either created or made investments in the following entities:

<u>Consolidated Entities</u>	<u>Relationship as of March 31, 2019</u>	<u>Date Control First Acquired</u>	<u>Ownership % as of December 31, 2018</u>	<u>Ownership % as of March 31, 2019 (unaudited)</u>
TheRas, Inc.	Wholly-owned subsidiary	August 2016	100%	100%
BridgeBio Services, Inc.	Wholly-owned subsidiary	April 2017	100%	100%
Origin Biosciences, Inc.	Wholly-owned subsidiary	April 2018	100%	100%
Fortify Therapeutics, Inc.	Wholly-owned subsidiary	June 2018	100%	100%
Sub20, Inc.	Wholly-owned subsidiary	June 2018	100%	100%
Unnamed Entity	Wholly-owned subsidiary	December 2018	100%	100%
Eidos Therapeutics, Inc.(1)	Partially-owned subsidiary	April 2016	62.5%	62.3%
Molecular Skin Therapeutics, Inc.	Controlled VIE	July 2016	61.7%	59.9%
Quartz Therapeutics, Inc.	Controlled VIE	October 2016	89.0%	89.0%
PellePharm, Inc.(2)	VIE	December 2016	43.3%	43.3%
Navire Pharma, Inc.	Controlled VIE	February 2017	78.8%	79.0%
CoA Therapeutics, Inc.	Controlled VIE	February 2017	99.5%	99.6%
Dermeccular Therapeutics, Inc.	Controlled VIE	April 2017	87.6%	87.6%
Phoenix Tissue Repair, Inc (“PTR”).	Controlled VIE	July 2017	56.7%	56.6%
QED Therapeutics, Inc.	Controlled VIE	January 2018	94.4%	95.7%
Adrenas Therapeutics, Inc.	Controlled VIE	January 2018	90.1%	89.5%
Orfan Biotech, Inc.	Controlled VIE	January 2018	85.1%	87.5%
Ferro Therapeutics, Inc.	Controlled VIE	March 2018	89.4%	88.6%
Venthera, Inc. (“Venthera”)	Controlled VIE	April 2018	82.0%	82.0%
Aspa Therapeutics, Inc.	Controlled VIE	June 2018	92.5%	93.8%

- (1) Subsequent to the Eidos Therapeutics, Inc. (“Eidos”) initial public offering in June 2018 and through March 31, 2019, BridgeBio has a majority voting interest in Eidos and consolidates Eidos under the VOE model. Refer to Note 5.
- (2) Subsequent to the execution of a series of agreements (the “LEO Agreement”) with LEO Pharma A/S and LEO Spiny Merger Sub, Inc. (“LEO”) in November 2018, BridgeBio determined that it is no longer the primary beneficiary of PellePharm, Inc. (“PellePharm”) and deconsolidated PellePharm. Refer to Note 7.

***Use of Estimates***

The preparation of condensed consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities and disclosure of contingent assets and liabilities at the date of the condensed consolidated financial statements, and the reported amounts of expenses during the reporting period. Significant estimates and assumptions made in the accompanying condensed consolidated financial statements include, but are not limited to, the fair value of the redeemable convertible preferred units, the fair value of the redeemable founder units, the fair value of the Common Units, the fair value of the LEO Call Option liability, the valuation of equity-based awards, income tax uncertainties and accruals for research and development activities. The Company bases its estimates on historical experience and on various other assumptions that are believed to be reasonable. Actual results may differ from those estimates or assumptions.

**BRIDGEBIO PHARMA LLC****Notes to Unaudited Interim Condensed Consolidated Financial Statements*****Cash, Cash Equivalents and Restricted Cash***

As of December 31, 2018 and March 31, 2019, the Company had restricted cash of \$0.2 million and nil. Restricted cash is classified in other assets in the accompanying condensed consolidated balance sheets.

The following table provides a reconciliation of cash, cash equivalents and restricted cash reported within the condensed consolidated balance sheets that sum to the total of the amounts shown in the condensed consolidated statements of cash flows:

	<u>March 31,</u> <u>2018</u>	<u>March 31,</u> <u>2019</u>
	(in thousands)	
Cash and cash equivalents	\$ 74,583	\$ 373,027
Restricted cash	358	—
Total cash, cash equivalents and restricted cash shown in the condensed consolidated statements of cash flows	<u>\$ 74,941</u>	<u>\$ 373,027</u>

As of December 31, 2018 and March 31, 2019, total cash and cash equivalents held by BridgeBio is \$238.7 million and \$172.8 million. The remaining cash and cash equivalents were held by the Company's wholly-owned subsidiaries and controlled entities and these funds are designated for specific entity usage, except in limited circumstances.

***Fair Value Measurements***

Assets and liabilities recorded at fair value on a recurring basis in the balance sheets are categorized based upon the level of judgment associated with the inputs used to measure their fair values. Fair value is defined as the exchange price that would be received for an asset or an exit price that would be paid to transfer a liability in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. The authoritative guidance on fair value measurements establishes a three-tier fair value hierarchy for disclosure of fair value measurements as follows:

Level 1—Observable inputs such as unadjusted, quoted prices in active markets for identical assets or liabilities at the measurement date;

Level 2—Inputs (other than quoted prices included in Level 1) are either directly or indirectly observable for the asset or liability. These include quoted prices for similar assets or liabilities in active markets and quoted prices for identical or similar assets or liabilities in markets that are not active; and

Level 3—Unobservable inputs that are supported by little or no market activity and that are significant to the fair value of the assets or liabilities.

To the extent that the valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized in Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement.

The fair value of the Company's outstanding term loans with Hercules, Capital Inc. (see Note 9) is estimated using the net present value of the payments, discounted at an interest rate that is consistent with a market interest

**BRIDGEBIO PHARMA LLC**

**Notes to Unaudited Interim Condensed Consolidated Financial Statements**

rate, which is a Level 2 input. The estimated fair value of the Company's outstanding term loans approximates the carrying amount, as the term loan bears a floating rate that approximates the market interest rate.

***Deferred Offering Costs***

The Company has deferred offering costs consisting of legal, accounting and other fees and costs directly attributable to the Company's planned IPO. The deferred offering costs will be offset against the proceeds received upon the completion of the planned IPO. In the event the planned IPO is terminated, all of the deferred offering costs will be expensed. As of December 31, 2018, no amounts were deferred. As of March 31, 2019, \$1.8 million of deferred offering costs were recorded within other assets on the condensed consolidated balance sheet.

***Net Loss per Unit***

The holders of the Company's Preferred Units are entitled to receive distributions, including cumulative returns on their units outstanding, prior and in preference to any distributions on any of the Company's Founder Units and Common Units, which are also entitled to cumulative returns. Cumulative returns for Preferred Units, Common Units and Founder Units no longer accumulate subsequent to the Series D Preferred Unit financing in November 2018. For the three months ended March 31, 2018 and 2019, the Company determined that its Founder Units and Common Units are common stock equivalents.

Basic net loss per unit is the same as diluted net loss per unit as the inclusion of all potentially dilutive Preferred Units, unvested Common Units, and Management Incentive Units would have been anti-dilutive.

***Pro Forma Net Loss per Share***

The unaudited pro forma basic and diluted net loss per share attributable to common stockholders for the three months ended March 31, 2019 has been prepared to give effect to the exchange of all Outstanding Units of BridgeBio into shares of common stock of the Corporation upon the Reorganization as if the Reorganization had occurred on the later of beginning of period or the issuance date of the Outstanding Units.

***Recently Adopted Accounting Pronouncements***

**ASU 2015-17 Income Taxes (Topic 740).** In November 2015, the FASB issued *ASU 2015-17 Income Taxes (Topic 740): Balance Sheet Classification of Deferred Taxes ("ASU 2015-17")*, which simplifies the presentation of deferred taxes in a classified balance sheet by eliminating the requirement to separate deferred income tax liabilities and assets into current and noncurrent amounts. Instead, ASU 2015-17 requires that all deferred tax liabilities and assets be shown as noncurrent in a classified balance sheet. ASU 2015-17 is effective for fiscal years beginning after December 15, 2017 and may be applied either prospectively or retrospectively to all periods presented. The Company adopted this guidance on January 1, 2018. The condensed consolidated balance sheets as of March 31, 2018 and 2019 are presented in accordance with this guidance.

***Recently Issued Accounting Pronouncements Not Yet Adopted***

**ASU 2016-02 Leases (Topic 842).** In February 2016, the FASB issued *ASU 2016-02, Leases ("ASU 2016-02")*, which for operating leases requires the lessee to recognize a right-of-use asset and a lease liability, initially measured at the present value of the lease payments, in its balance sheet. The guidance also requires a lessee to recognize single lease costs, calculated so that the cost of the lease is allocated over the lease term, on a

**BRIDGEBIO PHARMA LLC**

**Notes to Unaudited Interim Condensed Consolidated Financial Statements**

generally straight-line basis. A modified retrospective transition approach is required for leases existing at, or entered into after, the beginning of the earliest comparative period presented in the financial statements, including a number of optional practical expedients that entities may elect to apply. ASU 2016-02 is effective for fiscal years beginning after December 15, 2019 and interim periods within fiscal years beginning after December 15, 2020, with early adoption permitted. The Company has not determined the potential effects of this ASU on its consolidated financial statements.

**ASU 2016-15 Statement of Cash Flows (Topic 230) Classification of Certain Cash Receipts and Cash Payments.** In August 2016, the FASB issued ASU 2016-15, *Statement of Cash Flows (Topic 230): Classification of Certain Cash Receipts and Cash Payments* (“ASU 2016-15”). The areas affected by ASU 2016-15 are debt prepayment and debt extinguishment costs, settlement of zero-coupon debt instruments or other debt instruments with coupon interest rates that are insignificant in relation to the effective interest rate of the borrowing, contingent consideration payments made after a business combination, proceeds from the settlement of insurance claims, proceeds from the settlement of corporate-owned life insurance policies (including bank-owned life insurance policies), distributions received from equity method investees, beneficial interests in securitization transactions and separately identifiable cash flows and application of the predominance principle. Specifically, under this guidance, cash payments for debt prepayment or debt extinguishment costs will be classified as cash outflows for financing activities. The amendments in ASU 2016-15 are effective for fiscal years beginning after December 15, 2018 and interim periods within fiscal years beginning after December 15, 2019. Early adoption is permitted, including adoption in an interim period. If an entity early adopts the amendments in an interim period, any adjustments should be reflected as of the beginning of the fiscal year that includes that interim period. The amendments in ASU 2016-15 will be applied using a retrospective transition method to each period presented. The adoption of ASU 2016-15 is not expected to materially impact the Company’s consolidated financial statements.

**ASU 2018-13, Fair Value Measurement – Disclosure Framework (Topic 820).** In August 2018, the FASB issued ASU 2018-13, *Fair Value Measurement - Disclosure Framework (Topic 820)* (“ASU 2018-13”). The updated guidance improves the disclosure requirements on fair value measurements and is effective for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2019. Early adoption is permitted upon issuance of the standard for disclosures modified or removed with a delay of adoption of the additional disclosures until their effective date. The adoption of ASU 2018-13 is not expected to materially impact the Company’s consolidated financial statements.

**BRIDGEBIO PHARMA LLC**

**Notes to Unaudited Interim Condensed Consolidated Financial Statements**

**3. Fair Value Measurement**

The following table presents information about the Company's financial assets and liabilities that are measured at fair value on a recurring basis and indicates the fair value hierarchy of the valuation:

	<b>December 31, 2018</b>			
	<b>Total</b>	<b>Level 1</b>	<b>Level 2</b>	<b>Level 3</b>
(in thousands)				
<b>Assets:</b>				
Money market funds	\$395,780	\$395,780	\$ —	\$ —
Total assets	\$395,780	\$395,780	\$ —	\$ —
<b>Liabilities:</b>				
LEO Call Option liability	\$ 3,009	\$ —	\$ —	\$3,009
Total liabilities	\$ 3,009	\$ —	\$ —	\$3,009
	<b>March 31, 2019</b>			
	<b>Total</b>	<b>Level 1</b>	<b>Level 2</b>	<b>Level 3</b>
(in thousands)				
<b>Assets:</b>				
Money market funds	\$319,793	\$319,793	\$ —	\$ —
Total assets	\$319,793	\$319,793	\$ —	\$ —
<b>Liabilities:</b>				
LEO Call Option liability	\$ 4,523	\$ —	\$ —	\$4,523
Total liabilities	\$ 4,523	\$ —	\$ —	\$4,523

There were no transfers between Level 1, Level 2 or Level 3 during the three months ended March 31, 2019.

***LEO Call Option Liability***

The valuation of the LEO Call Option (see Note 7) contains unobservable inputs that reflect the management's own assumptions for which there is little, if any, market activity at the measurement date. Accordingly, the LEO Call Option liability is remeasured to fair value on a recurring basis using unobservable inputs that are classified as Level 3 inputs.

The Company estimated the fair value of the LEO Call Option by estimating the fair value of various clinical, regulatory, and sales milestones based on the estimated risk and probability of achievement of each milestone, and allocated the value using a Black-Scholes option pricing model with the following assumptions:

	<b>December 31, 2018</b>	<b>March 31, 2019</b>
Probability of milestone achievement	12.0%-84.0%	12.0%-84.0%
Discount rate	2.7%-11.0%	2.3%-15.8%
Expected term (in years)	0.58-4.38	0.58-4.37
Expected volatility	67.0%-79.0%	65.0%-74.0%
Risk-free interest rate	2.51%-2.78%	3.09%-3.30%
Dividend yield	—	—

## BRIDGEBIO PHARMA LLC

## Notes to Unaudited Interim Condensed Consolidated Financial Statements

The following table sets forth a summary of the changes in the estimated fair value of the LEO Call Option:

	<b>Total</b> <b>(in thousands)</b>
Balance as of December 31, 2018	\$ 3,009
Change in fair value upon remeasurement recognized in other (income) expense	1,514
Balance as of March 31, 2019	<u>\$ 4,523</u>

**4. Balance Sheet Components*****Prepaid Expenses and Other Current Assets***

Prepaid expenses and other current assets consist of the following:

	<b>December 31,</b> <b>2018</b>	<b>March 31,</b> <b>2019</b>
	<b>(in thousands)</b>	
Prepaid clinical and research related expenses	\$ 7,087	\$ 7,170
Other current assets	2,050	2,276
Total prepaid expenses and other current assets	<u>\$ 9,137</u>	<u>\$ 9,446</u>

***Other Accrued Liabilities***

Other accrued liabilities consist of the following:

	<b>December 31,</b> <b>2018</b>	<b>March 31,</b> <b>2019</b>
	<b>(in thousands)</b>	
Accrued professional services	\$ 772	\$ 2,142
Accrued other liabilities	1,328	2,223
Total other accrued liabilities	<u>\$ 2,100</u>	<u>\$ 4,365</u>

**5. Variable Interest Entities and Voting Interest Model**

The entities consolidated by BridgeBio are comprised of wholly-owned subsidiaries and partially-owned entities consolidated under the VOE model and VIEs for which BridgeBio is the primary beneficiary under the VIE model. The results of operations of the consolidated entities are included within the BridgeBio condensed consolidated financial statements for the three months ended March 31, 2018 and 2019. As of March 31, 2019, there were no significant restrictions on the VIE assets or liabilities except for the cash held at each VIE as discussed in Note 1. For VIEs, BridgeBio calculates the maximum exposure to loss to be equal to the amount invested in the equity of the VIE and the amount of outstanding convertible notes.

Included within Note 2 is a list of partially-owned entities that were determined to be under BridgeBio's control as of December 31, 2018 and March 31, 2019. At each reporting period, the Company reassesses whether it has a majority voting interest for entities consolidated under the VOE model and whether it remains the primary beneficiary for VIEs consolidated under the VIE model.



**BRIDGEBIO PHARMA LLC**
**Notes to Unaudited Interim Condensed Consolidated Financial Statements**
**Eidos**

From the date of BridgeBio's initial investment until the Eidos IPO on June 22, 2018, Eidos was determined to be a VIE and BridgeBio consolidated Eidos as the primary beneficiary. Subsequent to the Eidos IPO, BridgeBio determined that Eidos was no longer a VIE due to it having sufficient equity at risk to finance its activities without additional subordinated financial support. From June 22, 2018 through March 31, 2019, BridgeBio determined that it held greater than 50% of the voting shares of Eidos and there were no other parties with substantive participating, liquidation or kick-out rights. BridgeBio consolidated Eidos under the VOE model as of December 31, 2018 and March 31, 2019.

**Consolidated VIEs**

The entities identified as a "Controlled VIE" in Note 2 are VIEs for which BridgeBio was determined to be the primary beneficiary as of March 31, 2019. BridgeBio also had a majority ownership interest in these entities as of December 31, 2018 and March 31, 2019.

During the three months ended March 31, 2019, BridgeBio made investments in QED of \$20.0 million, Quartz of \$0.1 million, CoA of \$5.1 million, Orfan of \$1.5 million, Ferro of \$1.5 million, Aspa of \$8.0 million, Adrenas of \$8.0 million, and Navire of \$4.5 million in exchange for shares of redeemable convertible preferred stock of the respective entities. Based on the above noted equity financing transactions, BridgeBio concluded that there was no change in the consolidation conclusion during the period ended March 31, 2019.

The following table provides the assets and liabilities for all consolidated VIEs as of December 31, 2018:

	Adrenas	Aspa	PTR	QED	Venthera	All Other	Total
	(in thousands)						
<b>Assets:</b>							
Current assets:							
Cash and cash equivalents	\$ 3,046	\$ 4,259	\$ 6,934	\$ 8,630	\$ 2,913	\$ 6,713	\$ 32,495
Prepaid expenses and other current assets	665	1,722	28	3,240	—	321	5,976
<b>Total current assets</b>	<b>3,711</b>	<b>5,981</b>	<b>6,962</b>	<b>11,870</b>	<b>2,913</b>	<b>7,034</b>	<b>38,471</b>
Property and equipment, net	584	129	88	181	—	277	1,259
Other assets	7	—	41	—	—	28	76
<b>Total assets</b>	<b>\$ 4,302</b>	<b>\$ 6,110</b>	<b>\$ 7,091</b>	<b>\$ 12,051</b>	<b>\$ 2,913</b>	<b>\$ 7,339</b>	<b>\$ 39,806</b>
<b>Liabilities:</b>							
Current liabilities:							
Accounts payable	\$ 1,876	\$ 1,187	\$ 621	\$ 3,537	\$ 333	\$ 1,737	\$ 9,291
Accrued compensation and benefits	377	30	287	1,392	—	467	2,553
Accrued research and development liabilities	227	728	—	4,390	—	1,251	6,596
Other accrued liabilities	28	32	8	229	9	82	388
<b>Total current liabilities</b>	<b>2,508</b>	<b>1,977</b>	<b>916</b>	<b>9,548</b>	<b>342</b>	<b>3,537</b>	<b>18,828</b>
Other liabilities	—	—	—	150	—	29	179
<b>Total liabilities</b>	<b>\$ 2,508</b>	<b>\$ 1,977</b>	<b>\$ 916</b>	<b>\$ 9,698</b>	<b>\$ 342</b>	<b>\$ 3,566</b>	<b>\$ 19,007</b>

**BRIDGEBIO PHARMA LLC**
**Notes to Unaudited Interim Condensed Consolidated Financial Statements**

The following table provides the assets and liabilities for all consolidated VIEs as of March 31, 2019:

	Adrenas	Aspa	PTR	QED	Venthera	All Other	Total
	(in thousands)						
<b>Assets:</b>							
Current assets:							
Cash and cash equivalents	\$ 7,370	\$ 7,933	\$ 2,974	\$ 8,792	\$ 2,045	\$ 12,890	\$ 42,004
Prepaid expenses and other current assets	1,108	1,794	107	2,861	—	288	6,158
<b>Total current assets</b>	<b>8,478</b>	<b>9,727</b>	<b>3,081</b>	<b>11,653</b>	<b>2,045</b>	<b>13,178</b>	<b>48,162</b>
Property and equipment, net	568	328	82	318	—	277	1,573
Other assets	10	—	41	—	—	29	80
<b>Total assets</b>	<b>\$ 9,056</b>	<b>\$ 10,055</b>	<b>\$ 3,204</b>	<b>\$ 11,971</b>	<b>\$ 2,045</b>	<b>\$ 13,484</b>	<b>\$ 49,815</b>
<b>Liabilities:</b>							
Current liabilities:							
Accounts payable	\$ 2,638	\$ 1,518	\$ 282	\$ 663	\$ 431	\$ 689	\$ 6,221
Accrued compensation and benefits	210	32	173	995	—	412	1,822
Accrued research and development liabilities	201	—	105	3,945	32	891	5,174
Other accrued liabilities	14	130	8	1,369	18	76	1,615
<b>Total current liabilities</b>	<b>3,063</b>	<b>1,680</b>	<b>568</b>	<b>6,972</b>	<b>481</b>	<b>2,068</b>	<b>14,832</b>
Other liabilities	—	—	—	155	—	24	179
<b>Total liabilities</b>	<b>\$ 3,063</b>	<b>\$ 1,680</b>	<b>\$ 568</b>	<b>\$ 7,127</b>	<b>\$ 481</b>	<b>\$ 2,092</b>	<b>\$ 15,011</b>

VIEs included in the “All Other” category of the above table are not significant individually for separate presentation. Going forward, BridgeBio may not provide any further investment in certain of these VIEs.

**6. Noncontrolling Interests**

As of March 31, 2019, the Company has both redeemable convertible noncontrolling interests and noncontrolling interests in consolidated partially-owned entities, for which BridgeBio has a majority voting interest under the VOE model and for which BridgeBio is the primary beneficiary under the VIE model. These balances are reported as separate components outside members’ deficit and as part of members’ deficit, respectively, in “Redeemable convertible noncontrolling interests” and “Noncontrolling interests” in the condensed consolidated balance sheets.

The Company adjusts the carrying value of noncontrolling interest to reflect the book value attributable to noncontrolling shareholders of consolidated partially-owned entities when there is a change in the ownership during respective reporting period. During the three months ended March 31, 2018 and 2019, such adjustments in the aggregate amounts of \$3.9 million and \$3.0 million, respectively, are recorded to accumulated deficit and disclosed within the “Transfers to (from) noncontrolling interest” line item in the Condensed Consolidated Statements of Redeemable Convertible Preferred Units, Redeemable Founder Units, Redeemable Common Units, Management Incentive Units, Redeemable Convertible Noncontrolling Interests and Members’ Deficit.

**BRIDGEBIO PHARMA LLC**
**Notes to Unaudited Interim Condensed Consolidated Financial Statements**

The following table provides a rollforward of the redeemable convertible noncontrolling interests balance, as follows:

	Orfan	QED (in thousands)	Total
Balance as of December 31, 2018	\$ 8	\$ 114	\$ 122
Net loss attributable to redeemable convertible noncontrolling interest	(34)	(756)	(790)
Transfers to redeemable convertible noncontrolling interest	57	813	870
Balance as of March 31, 2019	<u>\$ 31</u>	<u>\$ 171</u>	<u>\$ 202</u>

The following table provides a rollforward of the noncontrolling interests balance:

	Adrenas	Aspa	Eidos	PTR (in thousands)	Venthera	All Other	Total
Balance as of December 31, 2018	\$ 217	\$ 245	\$58,185	\$ 2,728	\$ 449	\$ 537	\$62,361
Issuance of noncontrolling interest	2	2	1,027	34	1	254	1,320
Transfers to (from) noncontrolling interest	874	472	(337)	(10)	(1)	1,100	2,098
Net loss attributable to noncontrolling interest	(451)	(222)	(4,365)	(1,580)	(192)	(651)	(7,461)
Balance as of March 31, 2019	<u>\$ 642</u>	<u>\$ 497</u>	<u>\$54,510</u>	<u>\$ 1,172</u>	<u>\$ 257</u>	<u>\$ 1,240</u>	<u>\$58,318</u>

**7. PellePharm Investment**

PellePharm is a clinical stage biopharmaceutical company developing BBP-009, a topical gel formulation of patidegib, a hedgehog inhibitor, for the treatment of Gorlin Syndrome and High-Frequency Basal Cell Carcinoma. In July 2015, BridgeBio made an initial investment of \$4.5 million in PellePharm and in a series of transactions through December 2016, the Company increased its ownership interest to greater than 50%. BridgeBio determined that its initial investment in PellePharm represented a variable interest, but that BridgeBio was not the primary beneficiary until December 2016.

On November 19, 2018, PellePharm entered into the LEO Agreement with LEO, pursuant to which LEO was granted an exclusive, irrevocable option to acquire PellePharm. The LEO Call Option is exercisable by LEO on or before the occurrence of certain events relating to PellePharm's clinical development programs and no later than July 30, 2021. The Company accounts for the LEO Call Option as a current liability in its condensed consolidated financial statements because BridgeBio is obligated to sell its shares in PellePharm to LEO at a pre-determined price, if the option is exercised. The Company will remeasure the LEO Call Option to fair value at each subsequent condensed consolidated balance sheet date until the LEO Call Option is either exercised or expires.

The date the LEO Agreement was entered into was determined to be a VIE reconsideration event. Based on the Company's assessment, BridgeBio concluded that PellePharm remains a VIE after the reconsideration event as it does not have sufficient equity at risk to finance its activities without additional subordinated financial support. However, based on changes to PellePharm's governance structure and Board of Directors composition as a result of the LEO Agreement, BridgeBio is no longer the primary beneficiary as it no longer has the power

## BRIDGEBIO PHARMA LLC

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over the key decisions that most significantly impact PellePharm's economic performance. Accordingly, BridgeBio deconsolidated PellePharm on November 19, 2018. After the deconsolidation in November 2018, PellePharm is considered a related party of BridgeBio.

Subsequent to deconsolidation of PellePharm, BridgeBio accounted for its retained common stock investment as an equity method investment and its retained preferred stock investment as a cost method investment. As of December 31, 2018 and March 31, 2019, the aggregate carrying amount of the Company's equity method investment in PellePharm is \$0.2 million and nil, respectively. As of December 31, 2018 and March 31, 2019, the aggregate carrying amount for the Company's cost method investment in PellePharm is \$16.8 million and \$12.5 million. After the equity method investment was reduced to zero during the period ended March 31, 2019, BridgeBio then recorded its percentage of net losses consistent with its preferred stock ownership percentage of 62%. The carrying amount of BridgeBio's investment in PellePharm in the condensed consolidated balance sheets represents its maximum loss exposure related to its VIE investment in PellePharm.

#### 8. Commitments and Contingencies

On December 31, 2018, Children Hospital Research Center at Oakland ("CHRCO") filed, but did not serve, a civil complaint against Dr. Ervin Epstein, Co-Founder and Chief Medical Officer of PellePharm and PellePharm in the Northern District of California. CHRCO asserts four causes of action against Dr. Epstein (conversion, breach of contract, breach of the implied covenant of good faith and fair dealing, and specific performance), and one related cause of action against PellePharm (constructive trust). All five causes of action are generally directed to a set of accusations relating to Dr. Epstein's prior employment at CHRCO. CHRCO has since withdrawn its complaint in the Northern District of California and filed, and as of May 16, 2019, has not yet served a revised civil complaint against PellePharm and Dr. Epstein in the Superior Court of the State of California, County of San Francisco, asserting the same five causes of action. On April 11, 2019, CHRCO filed an unopposed ex parte application with the Court to extend the deadline to serve the complaint to June 11, 2019. Dr. Epstein and PellePharm dispute all of CHRCO's allegations and believe they lack merit and they intend to contest the case vigorously. No responsive pleading is required at this time, nor has Dr. Epstein or PellePharm provided one.

From time to time, the Company may become involved in legal proceedings arising in the ordinary course of business. The Company is unable to predict the outcome of these matters or the ultimate legal and financial liability, and at this time cannot reasonably estimate the possible loss or range of loss and accordingly has not accrued a related liability.

#### 9. Hercules Term Loan

##### *Hercules Loan and Security Agreement*

In June 2018, the Company executed a Loan and Security Agreement with Hercules Capital, Inc. ("Hercules"), under which the Company borrowed \$35.0 million. The term of the loan was approximately 42 months, with a maturity date of January 1, 2022 (the "Maturity Date"). No principal payments were due during an interest-only period, commencing on the initial borrowing date and continuing through July 1, 2020 (the "Amortization Date"). The outstanding balance of the loan was to be repaid monthly beginning on the Amortization Date and extending through the Maturity Date. The term loan bears interest at a floating rate equal to the greater of: (i) the prime rate as reported in the Wall Street Journal plus 4.35% and (ii) 9.35% (9.85% as of March 31, 2019 based on the prime rate as of that date), payable monthly.

In December 2018, the Company executed the First Amendment to the Loan and Security Agreement, whereby the Company borrowed an additional \$20.0 million to increase the total principal balance outstanding to

**BRIDGEBIO PHARMA LLC**

**Notes to Unaudited Interim Condensed Consolidated Financial Statements**

\$55.0 million (the “Amended Hercules Term Loan”). Upon draw of the additional \$20.0 million, the interest-only period on the entire facility was extended until January 1, 2021 (the “Amended Amortization Date”). The outstanding balance of the original loan of \$35.0 million and the additional borrowing of \$20.0 million is to be repaid monthly beginning on the Amended Amortization Date and extending through July 1, 2022 (the “Amended Maturity Date”). The additional \$20.0 million loan bears interest at a floating rate equal to the greater of: (i) the prime rate as reported in the Wall Street Journal plus 3.35% and (ii) 9.10% (9.10% as of March 31, 2019), payable monthly.

During the three months ended March 31, 2018 and 2019, the Company recognized interest expense related to the Amended Hercules Term Loan of nil and \$1.7 million, of which \$0.4 million relates to amortization of debt discount.

**10. License Agreements**

***Stanford License Agreement***

In April 2016, Eidos entered into a license agreement with the Board of Trustees of the Leland Stanford Junior University (“Stanford University”) relating to Eidos’ drug discovery and development initiatives. Under this agreement, Eidos has been granted certain worldwide exclusive licenses to make, use and sell products that are covered by licensed patent rights. During the three months ended March 31, 2019, Eidos recognized research and development expense of \$0.2 million in connection with this agreement.

***The University of Texas License Agreement***

In March 2017, Navire entered into a collaboration and license agreement with The Board of Regents of The University of Texas System (“Board of Regents”) and The University of Texas M.D. Anderson Cancer Center (“MD Anderson” and collectively “University of Texas”) relating to Navire’s drug discovery and development initiatives. Under this agreement, Navire and the University of Texas will carry out the development, manufacture and commercialization of licensed product under exclusive licenses granted by the University of Texas. The Company issued the Board of Regents shares of common stock of Navire valued at \$0.2 million during the three months ended March 31, 2019 that was recognized as research and development expense.

***The Regents of the University of California License Agreement***

In September 2016, TheRas, Inc. (“TheRas”) entered into a license agreement with The Regents of the University of California (“UCSF”) relating to TheRas’ drug discovery and development initiatives. Under this agreement, TheRas has been granted certain worldwide exclusive licenses to use the licensed compounds (the “UCSF License”). No expense was recognized in connection with this agreement during the three months ended March 31, 2019.

***St. Jude License Agreement***

In April 2017, CoA entered into a license agreement with St. Jude Children’s Research Hospital, Inc., (“St. Jude”) relating to the CoA’s drug discovery and development initiatives. Under this agreement, CoA has been granted a worldwide exclusive license to use a licensed compound. No expense was recognized in connection with this agreement during the three months ended March 31, 2019.

**BRIDGEBIO PHARMA LLC**

**Notes to Unaudited Interim Condensed Consolidated Financial Statements**

***K-Gen License Agreement***

In March 2018, Ferro entered into a license agreement with K-Gen, Inc. (“K-Gen”) relating to Ferro’s drug discovery and development initiatives. Under this agreement, Ferro has been granted certain worldwide exclusive licenses to use the licensed compounds. No expense was recognized in connection with this agreement during the three months ended March 31, 2019.

***Memorial Sloan Kettering Cancer Center License Agreement***

In April 2018, Venthera entered into a license agreement with Memorial Sloan Kettering Cancer Center (“MSK”) relating to Venthera’s drug discovery and development initiatives. Under this agreement, Venthera has been granted certain worldwide exclusive licenses to use the licensed products. No expense was recognized in connection with this agreement during the three months ended March 31, 2019.

***University of Massachusetts License Agreement***

In April 2018, Aspa entered into a license agreement with the University of Massachusetts (“UM”) relating to Aspa’s drug discovery and development initiatives. Under this agreement, Aspa has been granted certain worldwide exclusive licenses to use the licensed compounds. During the three months ended March 31, 2019, Aspa recognized nominal research and development expense in connection with this agreement.

***NeuroVive License Agreement***

In June 2018, Fortify Therapeutics, Inc. (“Fortify”) entered into a license agreement with NeuroVive Pharmaceutical AB (“NeuroVive”) relating to Fortify’s drug discovery and development initiatives. Under this agreement, Fortify has been granted certain worldwide exclusive licenses to use the licensed compounds. During the three months ended March 31, 2019, Fortify recognized nominal research and development expense in connection with this agreement.

***Life License Agreement***

In August 2018, Adrenas entered into a license agreement with Life Technologies Corporation (“Life”) relating to Adrenas’ drug discovery and development initiatives. Under this agreement, Adrenas has been granted certain worldwide non-exclusive licenses to use the licensed compounds. During the three months ended March 31, 2019, BridgeBio recognized nominal research and development expense in connection with this agreement.

***Unnamed Entity License Agreement***

In December 2018, the Company’s Unnamed Entity entered into a license agreement relating to the Unnamed Entity’s drug discovery and development initiatives. Under this agreement, the Unnamed Entity has been granted certain worldwide exclusive licenses to use the licensed compounds. No expense was recognized in connection with this agreement during the three months ended March 31, 2019.

***Other License and Collaboration Agreements***

In addition to the agreements described above, the Company has also entered into other license and collaboration agreements with various institutions and business entities on terms similar to those described above, none of which are material individually or in the aggregate.

**BRIDGEBIO PHARMA LLC**

**Notes to Unaudited Interim Condensed Consolidated Financial Statements**

**11. Asset Acquisitions**

***Phoenix Tissue Repair, Inc. (“PTR”) Asset Acquisition***

In July 2017, PTR entered into the Contribution Agreement and Asset Purchase Agreement with Shire Human Genetic Therapies, Inc. and its subsidiary Lotus Tissue Repair, Inc. to acquire the right, title, and interest in certain intellectual property, research program assets, and contracts relating to recombinant human collagen type VII. As consideration, in 2017, PTR made an upfront cash payment of \$1.5 million and issued 10,019,900 shares of PTR common stock valued at a nominal fair value at issuance. There were no material direct transaction costs related to the transaction.

During the three months ended March 31, 2019, PTR made a milestone payment of \$2.0 million in connection with this agreement related to the Phase I initiation milestone being met. This amount was charged to research and development expense as the underlying in-process research and development asset has no alternative future use.

If certain substantive milestones are met in the future, PTR could be required to pay up to \$25.0 million in regulatory milestone payments, \$60.0 million in sales milestone payments, and pay royalties of up to low single-digit percentages on future net sales, if any.

**12. Related Party Transactions**

***Nonrecourse Notes***

In 2016 and 2017 the Company entered into nonrecourse notes (the “Notes”) with two founders. The Notes were issued to facilitate the purchase of Series B Preferred Units by two founders. The principal amount of the Notes was \$0.3 million and mature in May 2021. The Notes were accounted for as an option for which the Company recognized equity-based compensation expense on issuance. The repayment of the Notes is recorded as an addition to the Series B Preferred Units balance as payments are received and the Notes were paid in full in February 2019.

***PellePharm***

During the three months ended March 31, 2019, the Company provided nominal services to PellePharm.

**13. Redeemable Convertible Preferred Units, Founder Units, Common Units and Management Incentive Units**

As of March 31, 2019, the LLC Agreement provided for the issuance of Series A Preferred Units, Series B Preferred Units, Series C Preferred Units, Series D Preferred Units, Founder Units, Common Units and Management Incentive Units.

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Outstanding Preferred Units, Founder Units and Common Units consist of the following:

	Units Issued and Outstanding	Original Issue Price Per Unit	Carrying Value	Liquidation Preference
(in thousands, except unit and per unit amounts)				
Series A Preferred Units	24,935,281	\$ 0.2627	\$ 4,919	\$ 13,542
Series B Preferred Units	90,909,090	\$ 0.4400	39,766	47,096
Series C Preferred Units	141,155,758	\$ 0.9656	135,482	147,155
Series D Preferred Units	150,955,597	\$ 1.9823	298,698	299,239
Total Preferred Units as of December 31, 2018	407,955,726		478,865	507,032
Founder Units	11,420,741	\$ —	1,754	6,202
Common Units	7,197,783	\$ —	1,619	3,910
Total outstanding units as of December 31, 2018	<u>426,574,250</u>		<u>\$482,238</u>	<u>\$ 517,144</u>

	Units Issued and Outstanding	Original Issue Price Per Unit	Carrying Value	Liquidation Preference
(in thousands, except unit and per unit amounts)				
Series A Preferred Units	24,935,281	\$ 0.2627	\$ 4,919	\$ 13,542
Series B Preferred Units	90,909,090	\$ 0.4400	39,945	47,096
Series C Preferred Units	141,155,758	\$ 0.9656	135,482	147,155
Series D Preferred Units	150,955,597	\$ 1.9823	298,698	299,239
Total Preferred Units as of March 31, 2019	407,955,726		479,044	507,032
Founder Units	11,420,741	\$ —	1,754	6,202
Common Units	7,533,210	\$ —	1,645	4,092
Total outstanding units as of March 31, 2019	<u>426,909,677</u>		<u>\$482,443</u>	<u>\$ 517,326</u>

As of December 31, 2018 and March 31, 2019, BridgeBio has classified all of its outstanding Preferred Units, Founder Units, Common Units, and Management Incentive Units outside of members' deficit in the accompanying condensed consolidated financial statements because these units contain certain redemption features that are not solely within the control of BridgeBio. Specifically, in the event an IPO does not take place by a pre-defined date, the majority preferred unitholders could force a "liquidation event" that is not solely within BridgeBio's control. As of December 31, 2018 and March 31, 2019, the Company did not adjust the carrying values of the Preferred Units, Founder Units and Common Units to their deemed liquidation values of such units since a liquidation event was not probable as of the condensed consolidated balance sheet dates. Subsequent adjustments to increase or decrease the carrying values to the ultimate liquidation values will be made if and when it becomes probable that such a liquidation event will occur.

***MyoKardia Distribution***

In April 2015, the Company acquired an interest in MyoKardia, Inc. for \$1.0 million. This investment was subsequently disposed of in 2016 and the Company recognized a gain on disposal of \$1.2 million in the year ended December 31, 2016. Prior to the execution of the merger of BridgeBio and BridgeBio LLC in June 2017, the Company distributed \$1.2 million to its members in proportion to the number of units then outstanding and was required to distribute the remaining proceeds of \$1.0 million. This liability is included in accrued distributions to unitholders in the condensed consolidated balance sheet as of December 31, 2018. The accrued distributions of \$1.0 million were paid to unitholders in February 2019.



**BRIDGEBIO PHARMA LLC**

**Notes to Unaudited Interim Condensed Consolidated Financial Statements**

**14. Equity-Based Compensation**

The Company recorded equity-based compensation in the following expense categories in its condensed consolidated statements of operations for employees and non-employees:

	<b>Three Months Ended March 31, 2018</b>			
	<b>BridgeBio</b>	<b>Eidos</b>	<b>Other</b>	<b>Total</b>
	(in thousands)			
Research and development	\$ —	\$175	\$ 33	\$ 208
General and administrative	323	210	10	543
<b>Total equity-based compensation</b>	<b>\$ 323</b>	<b>\$385</b>	<b>\$ 43</b>	<b>\$ 751</b>

	<b>Three Months Ended March 31, 2019</b>			
	<b>BridgeBio</b>	<b>Eidos</b>	<b>Other</b>	<b>Total</b>
	(in thousands)			
Research and development	\$ —	\$452	\$ 17	\$ 469
General and administrative	1,236	512	15	1,763
<b>Total equity-based compensation</b>	<b>\$ 1,236</b>	<b>\$964</b>	<b>\$ 32</b>	<b>\$2,232</b>

For the three months ended March 31, 2018 and 2019, total BridgeBio equity-based compensation from Common Units was less than \$0.1 million and \$0.1 million, and from Management Incentive Units was \$0.3 million and \$1.2 million, respectively.

The estimated grant-date fair value of each Common Unit and Management Incentive Unit award was calculated using the Black-Scholes option pricing model, based on assumptions as follows:

	<b>Three Months Ended March 31,</b>	
	<b>2018</b>	<b>2019</b>
Expected term (in years)	1.25-1.50	1.50
Expected volatility	40.0%-45.0%	48.0%-49.0%
Risk-free interest rate	1.70%-1.96%	2.34%-2.56%
Dividend yield	—	—

BridgeBio had 9,098,522 authorized Common Units at December 31, 2018 and March 31, 2019. The following table summarizes BridgeBio's Common Units activity:

	<b>Number of Common Units Outstanding</b>	<b>Weighted- Average Grant Date Fair Value</b>
Balance as of December 31, 2018	7,197,783	\$ 0.08
Vested	335,427	\$ 0.08
<b>Balance as of March 31, 2019</b>	<b>7,533,210</b>	<b>\$ 0.08</b>

As of March 31, 2019, there were 1,565,312 unvested Common Units and total unrecognized compensation related to the unvested Common Units was \$0.1 million, which the Company expects to be recognized over a weighted-average period 1.1 years. All unvested Common Units as of March 31, 2019 will vest through May 2020.

**BRIDGEBIO PHARMA LLC****Notes to Unaudited Interim Condensed Consolidated Financial Statements**

The following table summarizes BridgeBio's authorized Management Incentive Units activity:

	<b>Number of Authorized Units</b>
Balance as of December 31, 2018	48,695,602
Authorized and granted	24,111,064
Balance as of March 31, 2019	<u>72,806,666</u>

The following table summarizes BridgeBio's vested Management Incentive Units activity:

	<b>Number of Management Incentive Units Outstanding</b>	<b>Weighted- Average Grant Date Fair Value</b>
Balance as of December 31, 2018	19,117,628	\$ 0.08
Vested	2,831,171	0.35
Balance as of March 31, 2019	<u>21,948,799</u>	<u>\$ 0.11</u>

As of March 31, 2019, there were 50,857,867 unvested Management Incentive Units and unrecognized compensation related to the unvested Management Incentive Units was \$44.2 million, which the Company expects to recognize over a weighted-average period of 4.6 years. All unvested Management Incentive Units as of March 31, 2019 will vest through February 2024.

***Eidos******Common stock***

Eidos has reserved shares of common stock for issuance as follows:

	<b>Three Months Ended March 31,</b>	
	<b>2018</b>	<b>2019</b>
Redeemable convertible preferred stock outstanding, as converted	18,726,796	—
Options issued and outstanding	560,128	1,307,729
Options available for future grants	807,682	718,557
ESPP shares available for future	—	130,166
Total	<u>20,094,606</u>	<u>2,156,452</u>

BRIDGEBIO PHARMA LLC

Notes to Unaudited Interim Condensed Consolidated Financial Statements

Stock options

The following table summarizes Eidos's stock option activity for the three months ended March 31, 2019:

	Options Available for Grant	Options Outstanding	Weighted-Average Exercise Price per Option	Weighted-Average Remaining Contractual Life (years)	Aggregate Intrinsic Value
(in thousands, except per share and per share data)					
Outstanding as of December 31, 2018	747,057	1,329,762	\$ 8.55	9.40	\$ 6,928
Granted	(28,500)	28,500	\$ 18.07		
Exercised	—	(50,533)	\$ 0.50		
Outstanding as of March 31, 2019	<u>718,557</u>	<u>1,307,729</u>	<u>\$ 9.07</u>	<u>9.19</u>	<u>\$ 18,805</u>
Vested and expected to vest as of March 31, 2019		<u>1,307,729</u>	<u>\$ 9.07</u>	<u>9.19</u>	<u>\$ 18,805</u>
Exercisable as of March 31, 2019		<u>191,753</u>	<u>\$ 3.10</u>	<u>8.86</u>	<u>\$ 3,902</u>

Employee stock options valuation

The fair value of employee and non-employee director stock option awards was estimated at the date of grant using a Black-Scholes option-pricing model with the following assumptions:

	Three Months Ended March 31,	
	2018	2019
Expected term (in years)	6.08	6.08
Expected volatility	69.08%	72.91%
Risk-free interest rate	2.69%	2.60%
Dividend yield	—	—
Weighted average fair value of share-based awards granted	\$ 6.41	\$ 11.14

Stock options granted to non-employees

Stock-based compensation related to stock options granted to non-employees is recognized as the stock options are earned. The fair value of the stock options granted to non-employees was calculated at each reporting date using the Black-Scholes option-pricing model with the following assumptions:

	Three Months Ended March 31,	
	2018	2019
Expected term (in years)	9.95	6.08
Expected volatility	69.08%	73.54%
Risk-free interest rate	2.73%	2.74%
Dividend yield	—	—

During the three months ended March 31, 2018 and 2019, Eidos granted 35,880, and 18,500 shares, respectively, to non-employee consultants. Eidos recognized stock-based compensation expense for non-employee awards during the three months ended March 31, 2018 and 2019 of \$0.2 million and \$0.0 million, respectively.

**BRIDGEBIO PHARMA LLC****Notes to Unaudited Interim Condensed Consolidated Financial Statements***Stock-based compensation*

As of March 31, 2019, there was \$10.7 million of total unrecognized compensation cost related to unvested equity-based compensation arrangements under the Eidos 2016 Plan and Eidos 2018 Plan. The unrecognized equity-based compensation cost is expected to be recognized over a weighted-average period 3.05 years.

**15. Income Taxes**

The Company's tax provision and the resulting effective tax rate for interim periods is determined based upon its estimated annual effective tax rate adjusted for the effect of discrete items arising in that quarter.

Upon conversion to a corporation, the Company will become subject to U.S. federal and state income taxes. Based on the Company's history of generating operating losses and its anticipation of operating losses continuing in the foreseeable future, the Company has determined that it is more likely than not that the tax benefits from its operating losses would not be realized and has determined that a full valuation allowance against its net deferred tax assets would be recorded on a pro forma basis. The Company also determined that any outside basis differences in its controlled entities would result in a deferred tax asset which would not be recognized since it would not reverse in the foreseeable future. With respect to its investment in PellePharm, which is accounted for under the equity method, a deferred tax asset is recorded but is subject to a valuation allowance and thus has no impact on a pro forma basis. Therefore, for the purposes of the pro forma tax provision, the Company has not recorded an income tax expense or benefit for the net losses incurred by the Company during the three months ended March 31, 2019.

The Company's policy is to recognize interest and penalties associated with uncertain tax benefits as part of the income tax provision and include accrued interest and penalties with the related income tax liability on our consolidated balance sheet. To date, the Company has not recognized any interest and penalties in its consolidated statements of operations, nor has it accrued for or made payments for interest and penalties. The Company's unrecognized gross tax benefits would not reduce the estimated annual effective tax rate if recognized because it has recorded a full valuation allowance on its deferred tax assets.

**16. Net Loss per Unit and Unaudited Pro Forma Net Loss per Share**

The following outstanding units were excluded from the computation of the diluted net loss per unit for the periods presented because their effect would have been anti-dilutive.

	<b>Three Months Ended March 31,</b>	
	<b>2018</b>	<b>2019</b>
Preferred Units	219,406,923	407,955,726
Management Incentive Units	45,678,102	72,806,666
Unvested Common Units	2,907,020	1,565,312
Total	<u>267,992,045</u>	<u>482,327,704</u>

***Unaudited Pro Forma Net Loss per Share***

The unaudited pro forma basic and diluted net loss per share attributable to common stockholders for the three months ended March 31, 2019 has been prepared to give effect to the exchange of all Outstanding Units of BridgeBio into shares of common stock of the Corporation upon the Reorganization as if the Reorganization had occurred on the later of beginning of period or the issuance date of the Outstanding Units.

**BRIDGEBIO PHARMA LLC****Notes to Unaudited Interim Condensed Consolidated Financial Statements**

Unaudited pro forma basic and diluted net loss per share attributable to common stockholders was calculated as follows:

	<b>Three Months Ended March 31, 2019 (in thousands, except share and per share data)</b>
<b>Numerator:</b>	
Pro forma net loss attributable to common stockholders	\$ (61,185)
<b>Denominator:</b>	
Total weighted-average common shares outstanding – basic and diluted	—
Pro forma adjustment to reflect the assumed exchange of Outstanding Units upon the Reorganization	92,741,876
Pro forma total weighted-average common shares outstanding – basic and diluted	92,741,876
<b>Net Loss per Share:</b>	
Pro forma net loss per share attributable to common stockholders – basic and diluted	\$ (0.66)

**17. Subsequent Events**

Subsequent events through May 16, 2019, the date on which the unaudited interim condensed consolidated financial statements were available to be issued, were evaluated by the Company to determine the need, if any, for recognition or disclosure in its unaudited interim condensed consolidated financial statements. This has been updated through May 24, 2019 for the amended Hercules Loan and Security Agreement and additional Eidos investment described below.

***Significant financing events in relation to controlled VIEs***

Subsequent to March 31, 2019, BridgeBio made additional investment in QED of \$20.0 million and Ferro of \$3.0 million.

***Lease Agreements***

In May 2019, BridgeBio entered into an amendment to the lease dated March 2017, which extends the lease term for an additional three years through April 2023. Base rent over the extended term is \$1.1 million and BridgeBio is also responsible for a monthly management fee and costs associated with certain tenant improvements.

***Amended Hercules Loan and Security Agreement***

In May 2019, the Company executed the Second Amendment to the Loan and Security Agreement whereby the Company borrowed an additional \$20.0 million (“Tranche III”) to increase the total principal balance outstanding to \$75.0 million. The outstanding balance of the entire facility is to be repaid monthly beginning on

**BRIDGEBIO PHARMA LLC**

**Notes to Unaudited Interim Condensed Consolidated Financial Statements**

the Amended Amortization Date and extending through the Amended Maturity Date, as defined in Note 9. Tranche III bears interest at a floating rate equal to the greater of: (i) the prime rate as reported in the Wall Street Journal plus 3.10% and (ii) 9.10%, payable monthly.

***Purchase of Shares of Eidos Therapeutics, Inc.***

On May 24, 2019, the Company purchased 1,103,848 shares of common stock of Eidos from an existing stockholder of Eidos for an aggregate price of \$28,627,746 in a private purchase transaction.

**15,000,000 shares**



**Common stock**

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**Prospectus**

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**J.P. Morgan**

**Goldman Sachs & Co. LLC**

**Jefferies**

**SVB Leerink**

**KKR**

**Piper Jaffray**

**Mizuho Securities**

**BMO Capital Markets**

**Raymond James**

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, 2019

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**PART II**  
**INFORMATION NOT REQUIRED IN PROSPECTUS**

**Item 13. Other Expenses of Issuance and Distribution.**

The following table sets forth all expenses, other than the underwriting discounts and commissions, payable by BridgeBio Pharma, Inc., or the Company or the Registrant, in connection with the sale of the common stock being registered. All the amounts shown are estimates except the SEC registration fee and the FINRA filing fee.

	<b>Amount Paid or to be Paid</b>
SEC registration fee	\$ 33,451
FINRA filing fee	41,900
Nasdaq initial listing fee	295,000
Legal fees and expenses	2,469,000
Accountants' fees and expenses	1,253,000
Printing expenses	686,000
Transfer and registrar fee	100,000
Miscellaneous	675,000
Total	<u>\$ 5,553,351</u>

**Item 14. Indemnification of directors and officers**

As permitted by Section 102 of the Delaware General Corporation Law, we have adopted provisions in our amended and restated certificate of incorporation and amended and restated bylaws that limit or eliminate the personal liability of our directors for a breach of their fiduciary duty of care as a director. The duty of care generally requires that, when acting on behalf of the corporation, directors exercise an informed business judgment based on all material information reasonably available to them. Consequently, a director will not be personally liable to us or our stockholders for monetary damages for breach of fiduciary duty as a director, except for liability for:

- any breach of the director's duty of loyalty to us or our stockholders;
- any act or omission not in good faith or that involves intentional misconduct or a knowing violation of law;
- any act related to unlawful stock repurchases, redemptions or other distributions or payment of dividends; or
- any transaction from which the director derived an improper personal benefit.

These limitations of liability do not affect the availability of equitable remedies such as injunctive relief or rescission. Our amended and restated certificate of incorporation also authorizes us to indemnify our officers, directors and other agents to the fullest extent permitted under Delaware law.

Section 145 of the Delaware General Corporation Law permits a corporation to include in its charter documents, and in agreements between the corporation and its directors and officers, provisions expanding the scope of indemnification beyond that specifically provided by the current law.

Section 145(a) of the Delaware General Corporation Law provides, in general, that a corporation may indemnify any person who was or is a party or is threatened to be made a party to any threatened, pending or completed action, suit or proceeding, whether civil, criminal, administrative or investigative (other than an action by or in the right of the corporation), because he or she is or was a director, officer, employee or agent of the corporation, or is or was serving at the request of the corporation as a director, officer, employee or agent of another corporation, partnership, joint venture, trust or other enterprise, against expenses (including attorneys' fees), judgments, fines and amounts paid in settlement actually and reasonably incurred by the person in connection



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with such action, suit or proceeding, if he or she acted in good faith and in a manner he or she reasonably believed to be in or not opposed to the best interests of the corporation and, with respect to any criminal action or proceeding, had no reasonable cause to believe his or her conduct was unlawful.

Section 145(b) of the Delaware General Corporation Law provides, in general, that a corporation may indemnify any person who was or is a party or is threatened to be made a party to any threatened, pending or completed action or suit by or in the right of the corporation to procure a judgment in its favor because the person is or was a director, officer, employee or agent of the corporation, or is or was serving at the request of the corporation as a director, officer, employee or agent of another corporation, partnership, joint venture, trust or other enterprise, against expenses (including attorneys' fees) actually and reasonably incurred by the person in connection with the defense or settlement of such action or suit if he or she acted in good faith and in a manner he or she reasonably believed to be in or not opposed to the best interests of the corporation, except that no indemnification shall be made with respect to any claim, issue or matter as to which he or she shall have been adjudged to be liable to the corporation unless and only to the extent that the Court of Chancery or other adjudicating court determines that, despite the adjudication of liability but in view of all of the circumstances of the case, he or she is fairly and reasonably entitled to indemnity for such expenses which the Court of Chancery or other adjudicating court shall deem proper.

Section 145(g) of the Delaware General Corporation Law provides, in general, that a corporation may purchase and maintain insurance on behalf of any person who is or was a director, officer, employee or agent of the corporation, or is or was serving at the request of the corporation as a director, officer, employee or agent of another corporation, partnership, joint venture, trust or other enterprise against any liability asserted against such person and incurred by such person in any such capacity, or arising out of his or her status as such, whether or not the corporation would have the power to indemnify the person against such liability under Section 145 of the Delaware General Corporation Law.

The Company's amended and restated certificate of incorporation, which will become effective upon completion of the offering, provides for the indemnification of directors to the fullest extent permissible under Delaware law.

The Company's amended and restated bylaws, which will become effective upon completion of the offering, provide for the indemnification of officers, directors and third parties acting on the Company's behalf if such persons act in good faith and in a manner reasonably believed to be in and not opposed to the Company's best interest, and, with respect to any criminal action or proceeding, such indemnified party had no reason to believe his or her conduct was unlawful.

The Company is entering into indemnification agreements with each of its directors and executive officers, in addition to the indemnification provisions provided for in its charter documents, and the Company intends to enter into indemnification agreements with any new directors and executive officers in the future. These agreements will provide that we will indemnify each of our directors and executive officers, and such entities to the fullest extent permitted by law.

The underwriting agreement (to be filed as Exhibit 1.1 hereto) will provide for indemnification and contribution by the underwriters of the Company, and its executive officers and directors, and indemnification and contribution of the underwriters by the Company for certain liabilities, including liabilities arising under the Securities Act of 1933, as amended, in connection with matters specifically provided in writing by the underwriters for inclusion in the registration statement.

The Company intends to purchase and maintain insurance on behalf of any person who is or was a director or officer against any loss arising from any claim asserted against him or her and incurred by him or her in that capacity, subject to certain exclusions and limits of the amount of coverage.

**Item 15. Recent Sales of Unregistered Securities.**

Set forth below is information regarding securities we have issued within the past three years that were not registered under the Securities Act. The numbers of shares of common stock for which units will be exchanged set forth below assume the exchange of all outstanding units of BridgeBio Pharma LLC into 99,999,967 shares of our common stock, based on an assumed initial public offering price of \$15.00 per share, the midpoint of the price range set forth on the cover of this prospectus.

***(a) Private Placements of Convertible Preferred Stock***

Between March 2016 and May 2017, we issued 75,340,907 Series B preferred units in several closings at a price of \$0.44 per unit to seven accredited investors for aggregate gross proceeds of approximately \$33.1 million. The Series B preferred units will be exchanged for an aggregate of 14,681,239 shares of common stock in connection with the Reorganization.

In June 2017, in connection with the merger transaction with BridgeBio LLC, we issued (i) 2,390,000 common units to six accredited investors, (ii) 3,000,000 founder units to six accredited investors, (iii) 6,550,000 Series A preferred units to seven accredited investors and (iv) 15,568,183 Series B preferred units to seven accredited investors, in each case in exchange for the same number of corresponding units in BridgeBio LLC held by such investors. The above units will be exchanged for an aggregate of 5,420,614 shares of common stock in connection with the Reorganization.

Between August 2017 and March 2018, we issued 141,155,758 Series C preferred units in several closings at a price of \$0.9656 per unit to seven accredited investors for aggregate gross proceeds of approximately \$136.3 million. As part of the Series C preferred unit transaction, we issued to one accredited investor an aggregate of 4,142,502 Series C preferred units, in exchange for cancellation of an aggregate of approximately \$4.0 million of outstanding indebtedness under a convertible promissory note we had previously issued. The Series C preferred units will be exchanged for an aggregate of 32,503,831 shares of common stock in connection with the Reorganization.

In November and December 2018, we issued 150,955,597 Series D preferred units at a purchase price of \$1.9823 per unit to 10 accredited investors for aggregate gross proceeds of approximately \$299.2 million. The Series D preferred units will be exchanged for an aggregate of 30,505,452 shares of common stock in connection with the Reorganization.

No underwriters were involved in the foregoing sales of securities. Unless otherwise stated, the sales of securities described above were deemed to be exempt from registration pursuant to Section 4(a)(2) of the Securities Act, including Regulation D and Rule 506 promulgated thereunder, as transactions by an issuer not involving a public offering. All of the purchasers in these transactions represented to us in connection with their purchase that they were acquiring the securities for investment and not distribution, that they could bear the risks of the investment and could hold the securities for an indefinite period of time. Such purchasers received written disclosures that the securities had not been registered under the Securities Act and that any resale must be made pursuant to a registration or an available exemption from such registration. All of the foregoing securities are deemed restricted securities for the purposes of the Securities Act.

***(b) Grants***

Through May 16, 2019, we have granted an aggregate of 72,806,666 management incentive units, with a grant date fair value ranging from \$0.02 to \$1.52 per unit, to employees, directors and consultants.

No underwriters were involved in the foregoing issuances of securities. The offers, sales and issuances of the securities described above were deemed to be exempt from registration under the Securities Act in reliance upon Rule 701 or Section 4(a)(2) of the Securities Act. The offers, sales and issuances of the securities that were deemed to be exempt in reliance on Rule 701 were transactions under compensatory benefit plans and contracts relating to compensation as provided under Rule 701. The offers, sales and issuances of the securities that were

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deemed to be exempt in reliance upon Section 4(a)(2) were each transactions not involving any public offering, and all recipients of these securities were accredited investors within the meaning of Rule 501 of Regulation D of the Securities Act who were acquiring the applicable securities for investment and not distribution and had represented that they could bear the risks of the investment. Each of the recipients of securities in these transactions had adequate access, through employment, business or other relationships, to information about us.

### **Item 16. Exhibits and Financial Statement Schedules.**

#### **(a) Exhibits**

<u>Exhibit Number</u>	<u>Description</u>
1.1*	Form of Underwriting Agreement.
3.1+	<a href="#"><u>Certificate of Incorporation of the Registrant, as currently in effect.</u></a>
3.2*	Form of Amended and Restated Certificate of Incorporation of the Registrant, to be in effect immediately prior to completion of this offering.
3.3+	<a href="#"><u>Bylaws of the Registrant, as currently in effect.</u></a>
3.4*	Form of Amended and Restated Bylaws of the Registrant, to be in effect immediately prior to completion of this offering.
4.1*	Specimen Common Stock Certificate.
4.2+	<a href="#"><u>Fourth Amended and Restated Limited Liability Company Agreement, dated November 20, 2018, by and among BridgeBio Pharma LLC and its members.</u></a>
4.3+	<a href="#"><u>Form of Registration Rights Agreement, among the Registrant and certain of its shareholders, to be in effect immediately prior to completion of this offering.</u></a>
5.1	<a href="#"><u>Opinion of Goodwin Procter LLP.</u></a>
10.1*	2019 Stock Option and Incentive Plan and forms of award agreements thereunder.
10.2*	2019 Employee Stock Purchase Plan.
10.3*	Senior Executive Cash Incentive Bonus Plan.
10.4*	Form of Indemnification Agreement, between the Registrant and each of its directors.
10.5*	Form of Indemnification Agreement, between the Registrant and each of its executive officers.
10.6+	<a href="#"><u>Loan and Security Agreement, between BridgeBio Pharma LLC and Hercules Capital, Inc., dated as of June 19, 2018.</u></a>
10.7+	<a href="#"><u>First Amendment to the Loan and Security Agreement, between BridgeBio Pharma LLC and Hercules Capital, Inc., dated as of December 28, 2018.</u></a>
10.8+	<a href="#"><u>Lease Agreement, between BridgeBio Pharma LLC and Michael J. Harbour, dated as of March 23, 2017.</u></a>
10.9++	<a href="#"><u>Exclusive (Equity) Agreement, by and between Eidos Therapeutics, Inc. and the Board of Trustees of the Leland Stanford Junior University, effective as of April 10, 2016, as amended by Amendment No. 1 effective September 25, 2017.</u></a>
10.10++	<a href="#"><u>License Agreement, between QED Therapeutics, Inc. and Novartis International Pharmaceutical Ltd., dated as of January 29, 2018.</u></a>
10.11++	<a href="#"><u>Asset Purchase Agreement, among BridgeBio Pharma LLC, Origin Biosciences, Inc., and Alexion Pharma Holding Unlimited Company, dated as of June 7, 2018.</u></a>

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<u>Exhibit Number</u>	<u>Description</u>
10.12†+	<a href="#"><u>Option Agreement, among PellePharm, Inc., Leo Pharma A/S and Leo Spiny Merger Sub, Inc., dated as of November 19, 2018, as amended on March 13, 2019.</u></a>
10.13†+	<a href="#"><u>Asset Purchase Agreement, among Phoenix Tissue Repair, Inc., Shire Human Genetic Therapies, Inc., and Lotus Tissue Repair, Inc., dated as of July 21, 2017.</u></a>
10.14†+	<a href="#"><u>Exclusive License Agreement, between The Regents of the University of California and TheRas, Inc., dated September 28, 2016, as amended by First Amendment effective January 10, 2017, Second Amendment effective August 10, 2017 and Third Amendment effective September 7, 2018.</u></a>
10.15†+	<a href="#"><u>Collaboration and License Agreement, between Navire Pharma, Inc. (formerly known as PTP Pharmaceuticals, Inc.) and the Board of Regents of the University of Texas System and The University of Texas M.D. Anderson Cancer Center, dated March 3, 2017, as amended by Amendment No. 1 dated July 10, 2017.</u></a>
10.16†+	<a href="#"><u>Exclusive Patent License Agreement, between The Frederick National Laboratory for Cancer Research, operated by Leidos Biomedical Research, Inc., under sponsorship from the National Cancer Institute, and TheRas, Inc., dated December 14, 2018.</u></a>
10.17†+	<a href="#"><u>Cell Line License Agreement, by and between Life Technologies Corporation and BridgeBio Services, Inc., effective as of November 15, 2018.</u></a>
10.18+	<a href="#"><u>Second Amendment to the Loan and Security Agreement, between BridgeBio Pharma LLC and Hercules Capital Inc., dated as of May 17, 2019.</u></a>
10.19+	<a href="#"><u>Offer Letter, between BridgeBio Services, Inc. and Neil Kumar, dated December 14, 2017.</u></a>
10.20+	<a href="#"><u>Offer Letter, between BridgeBio Services, Inc. and Brian Stephenson, dated October 28, 2018.</u></a>
10.21+	<a href="#"><u>Offer Letter, between Eidos Therapeutics, Inc. and Uma Sinha, dated June 1, 2016, as amended on May 24, 2018.</u></a>
10.22+	<a href="#"><u>Offer Letter, between BridgeBio Services, Inc. and Charles Homcy, dated February 20, 2019.</u></a>
10.23+	<a href="#"><u>Offer Letter, between BridgeBio Services, Inc. and Richard Scheller, dated April 5, 2019.</u></a>
10.24+	<a href="#"><u>Offer Letter, between BridgeBio Services, Inc. and Michael Henderson, dated March 22, 2016, as amended on May 5, 2017.</u></a>
10.25+	<a href="#"><u>Offer Letter, between BridgeBio Services, Inc. and Cameron Turtle, dated December 13, 2016, as amended on May 5, 2017.</u></a>
10.26+	<a href="#"><u>Offer Letter, between Eidos Therapeutics, Inc. and Cameron Turtle, dated June 13, 2018.</u></a>
10.27*	Form of Tax Sharing Agreement, between the Registrant and each of its subsidiaries.
21+	<a href="#"><u>List of Subsidiaries of the Registrant.</u></a>
23.1	<a href="#"><u>Consent of Deloitte &amp; Touche LLP independent registered public accounting firm to BridgeBio Pharma LLC.</u></a>
23.2	<a href="#"><u>Consent of Deloitte &amp; Touche LLP independent registered public accounting firm to BridgeBio Pharma, Inc.</u></a>
23.3	<a href="#"><u>Consent of Goodwin Procter LLP (included in Exhibit 5.1).</u></a>
24	<a href="#"><u>Power of Attorney (included on signature page).</u></a>

Unless otherwise indicated, exhibits are filed herewith.

+ Previously filed.

\* To be filed by amendment.

† Certain confidential portions (indicated by brackets and asterisks) have been omitted from this exhibit.

**(b) Financial Statement Schedules**

All schedules have been omitted because they are not required or because the required information is given in the financial statements or notes to those statements.

**Item 17. Undertakings.**

Insofar as indemnification for liabilities arising under the Securities Act of 1933, as amended, may be permitted to directors, officers and controlling persons of the Registrant pursuant to the foregoing provisions, or otherwise, the Registrant has been advised that in the opinion of the Securities and Exchange Commission such indemnification is against public policy as expressed in the Securities Act and is, therefore, unenforceable. In the event that a claim for indemnification against such liabilities (other than the payment by the Registrant of expenses incurred or paid by a director, officer or controlling person of the Registrant in the successful defense of any action, suit or proceeding) is asserted by such director, officer or controlling person in connection with the securities being registered, the Registrant will, unless in the opinion of its counsel the matter has been settled by controlling precedent, submit to a court of appropriate jurisdiction the question whether such indemnification by it is against public policy as expressed in the Securities Act of 1933 and will be governed by the final adjudication of such issue.

The undersigned Registrant hereby undertakes that:

(a) The undersigned Registrant will provide to the underwriters at the closing specified in the underwriting agreement certificates in such denominations and registered in such names as required by the underwriters to permit prompt delivery to each purchaser.

(b) For purposes of determining any liability under the Securities Act, the information omitted from the form of prospectus filed as part of this registration statement in reliance upon Rule 430A and contained in a form of prospectus filed by the Registrant pursuant to Rule 424(b)(1) or (4) or 497(h) under the Securities Act shall be deemed to be part of this registration statement as of the time it was declared effective.

(c) For the purpose of determining any liability under the Securities Act, each post-effective amendment that contains a form of prospectus shall be deemed to be a new registration statement relating to the securities offered therein, and the offering of such securities at that time shall be deemed to be the initial bona fide offering thereof.





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June 17, 2019

BridgeBio Pharma, Inc.  
421 Kipling Street  
Palo Alto, CA 94301

Re: Securities Registered under Registration Statement on Form S-1

Ladies and Gentlemen:

We have acted as counsel to you in connection with your filing of a Registration Statement on Form S-1 (File No. 333-231759) (as amended or supplemented, the "Registration Statement") pursuant to the Securities Act of 1933, as amended (the "Securities Act"), relating to the registration of the offering by BridgeBio Pharma, Inc., a Delaware corporation (the "Company") of up to 17,250,000 shares (the "Shares") of the Company's Common Stock, \$0.001 par value per share, including Shares purchasable by the underwriters upon their exercise of an over-allotment option granted to the underwriters by the Company. The Shares are being sold to the several underwriters named in, and pursuant to, an underwriting agreement among the Company and such underwriters (the "Underwriting Agreement").

We have reviewed such documents and made such examination of law as we have deemed appropriate to give the opinions set forth below. We have relied, without independent verification, on certificates of public officials and, as to matters of fact material to the opinions set forth below, on certificates of officers of the Company.

The opinion set forth below is limited to the Delaware General Corporation Law.

Based on the foregoing, we are of the opinion that the Shares have been duly authorized and, upon issuance and delivery against payment therefor in accordance with the terms of the Underwriting Agreement, the Shares will be validly issued, fully paid and non-assessable.

We hereby consent to the inclusion of this opinion as Exhibit 5.1 to the Registration Statement and to the references to our firm under the caption "Legal Matters" in the Registration Statement. In giving our consent, we do not admit that we are in the category of persons whose consent is required under Section 7 of the Securities Act or the rules and regulations thereunder.

Very truly yours,

/s/ Goodwin Procter LLP  
GOODWIN PROCTER LLP

**CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM**

We consent to the use in this Amendment No. 2 to Registration Statement No. 333-231759 of our report dated April 15, 2019 (May 16, 2019 as to the effects of the restatements discussed in Note 2 and Note 7) relating to the combined and consolidated financial statements of BridgeBio Pharma LLC appearing in the Prospectus, which is part of this Registration Statement, and to the reference to us under the heading “Experts” in such Prospectus.

/s/ DELOITTE & TOUCHE LLP

San Francisco, California

June 14, 2019



**CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM**

We consent to the use in this Amendment No. 2 to Registration Statement No. 333-231759 of our report dated May 24, 2019 relating to the financial statement of BridgeBio Pharma, Inc. appearing in the Prospectus, which is part of this Registration Statement, and to the reference to us under the heading "Experts" in such Prospectus.

/s/ DELOITTE & TOUCHE LLP

San Francisco, California

June 14, 2019