bridgebio

COMPANY PRESENTATION

July 2019

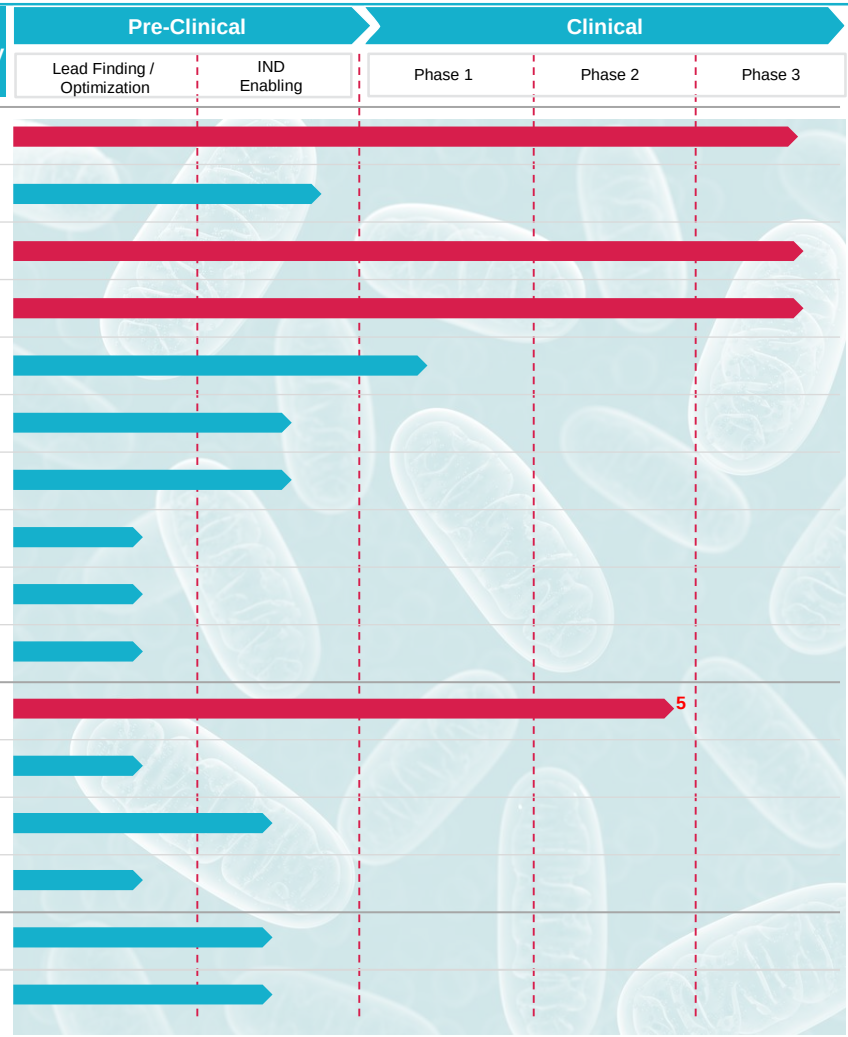
Forward-Looking Statements

Statements in this Presentation that are not statements of historical fact are forward-looking statements. Such forward-looking statements include, without limitation, statements regarding our research and clinical development plans, expected manufacturing capabilities, strategy, regulatory matters, market size and opportunity, future financial position, future revenue, projected costs, prospects, plans, objectives of management, and the Company's ability to complete certain milestones. Words such as "believe," "anticipate," "plan," "expect," "intend," "will," "may," "goal," "potential" and similar expressions are intended to identify forward-looking statements, though not all forward-looking statements necessarily contain these identifying words. These forward-looking statements are based on the beliefs of the Company's management as well as assumptions made by and information currently available to the Company. Such statements reflect the current views of the Company with respect to future events and are subject to known and unknown risks, including business, regulatory, economic and competitive risks, uncertainties, contingencies and assumptions about the Company, including, without limitation, risks inherent in developing therapeutic products, future results from the Company's ongoing and planned preclinical studies and clinical trials, the Company's ability to obtain adequate financing to fund its preclinical studies and planned clinical trials and other expenses, trends in the industry, the legal and regulatory framework for the industry and future expenditures. In light of these risks and uncertainties, the events or circumstances referred to in the forward-looking statements may not occur. The actual results may vary from the anticipated results and the variations may be material. These forward-looking statements should not be taken as forecasts or promises nor should they be taken as implying any indication, assurance or guarantee that the assumptions on which such forward-looking statements have been made are correct or exhaustive or, in the case of the assumptions, fully stated in the Presentation. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date this Presentation is given. This Presentation discusses product candidates that are under preclinical study or clinical trial and which have not yet been approved for marketing by the U.S. Food and Drug Administration (the "FDA"). No representation is made as to the safety or effectiveness of these product candidates for the therapeutic use for which such product candidates are being studied.

Certain information contained in this Presentation relates to or is based on studies, publications, surveys and other data obtained from third-party sources and the Company's own internal estimates and research. While the Company believes these third-party sources to be reliable as of the date of this Presentation, it has not independently verified, and makes no representation as to the adequacy, fairness, accuracy or completeness of, any information obtained from third-party sources. In addition, all of the market data included in this Presentation involves a number of assumptions and limitations, and there can be no guarantee as to the accuracy or reliability of such assumptions. Finally, while the Company believes its own internal research is reliable, such research has not been verified by any independent source.

We have built a sizeable pipeline over the past four years

	Program[1]	MOA / Diseases	Patient pop. (US+EU)[2]	Modality
Mendelian	**BBP-265 (Eidos)**	TTR stabilizer for ATTR ☆	>400K	
	BBP-831	FGFR1-3i for achondroplasia ☆	55K	
	BBP-870	cPMP replacement for MoCD Type A	100	
	BBP-009[4]	Topical SMOi for Gorlin / frequent BCC	120K	
	BBP-589	COL7A protein replacement for RDEB	1.5K	
	BBP-681	Topical PI3Kαi for venous malformations	117K	
	BBP-671	PanK 1/3 activator for PKAN, OAs	7K	
	BBP-711	GO1i for PH1 / FSF	5K / 1.5M	
	BBP-561	Topical KLKN 5/7i for Netherton	11K	
	BBP-761	Succinate pro-drug for LHON	20K	
Oncology	**BBP-831**	FGFR1-3i for FGFR+ cancers ☆	37K*	
	BBP-454	Pan-mutant KRASi for KRAS+ cancers ☆	>500K*	
	BBP-398	SHP2i for multiple cancer types	>500K*	
	BBP-954	GPX4i for multiple cancer types	>500K*	
Gene Therapy	**BBP-631**	AAV5 gene therapy for CAH ☆	>75K	
	BBP-812	AAV9 gene therapy for Canavan	1K	



☆ **Key Value Drivers** **Phase 2/3 clinical development**[3] Small molecule Topical small molecule Biologics Gene therapy

[1] Each of our programs is housed in a separate subsidiary; [2] Patient population: Prevalence except for asterisked figures which represent incidence; [3]A 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; [4] 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." [5]Planned New Drug Application (NDA) submission for the treatment of cholangiocarcinoma (CCA) as a second-line or later therapy (2L)

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Where we are headed: Profile by year-end 2021

“Shots on goal”

Based on our projected 2019-2021 catalyst map

Pipeline Advancement + **Add New Programs**

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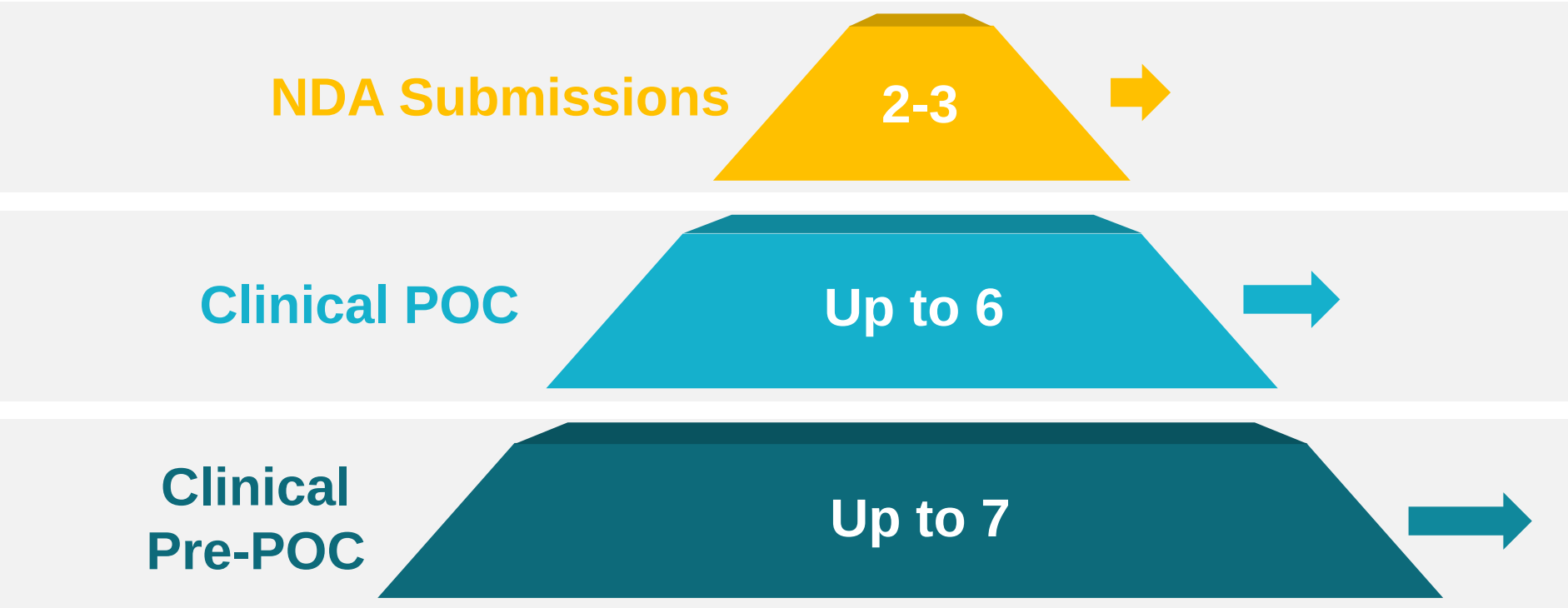
2-3 new programs/year



NDAs: BBP-831 (2L CCA), BBP-870, BBP-265
Clinical Proof-of-Concept (POC): BBP-831 (achon), BBP-631, BBP-009, BBP-681, BBP-589, 0-1 additional
Clinical: BBP-812, BBP-398, BBP-711, BBP-561, BBP-671, 1-2 additional
Note: The above represents potential non-risk adjusted outcomes

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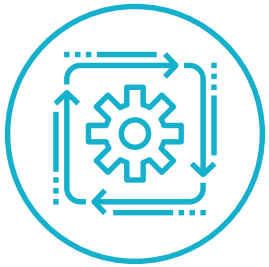
BridgeBio: A product platform and current pipeline



Product Platform

Systematic disease mapping

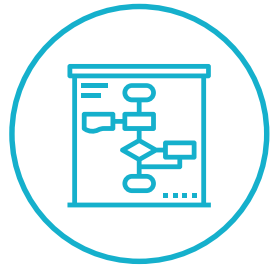
World-class R&D minds and capabilities

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

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BridgeBio is led by an experienced team

Neil Kumar, PhD — Co-Founder and CEO
- Principal at Third Rock Ventures
- Founding management team at MyoKardia
- Associate principal at McKinsey
- PhD MIT; BS / MS Stanford University

Frank McCormick, PhD, FRS — Chairman of Oncology
- Director of the UCSF Cancer Center and former Associate Dean of the UCSF School of Medicine
- Former AACR President
- Co-founder & first CSO of Onyx Pharma ($9B+)
- PhD Cambridge; BS University of Birmingham

Uma Sinha, PhD — Chief Scientific Officer
- CSO for Global Blood Therapeutics
- Senior leader in Portola, Millennium, and COR Therapeutics
- PhD Georgia; BS Presidency College

Phil Reilly, MD, JD
- Management Committee member
- Highly respected clinical geneticist
- Co-founder and former interim CMO of Voyager Therapeutics
- SAB member and former interim CMO of bluebird bio
- Co-founder and board member of Edimer, Lotus
- MD Yale; JD Columbia; BA Cornell

Andrew Lo, PhD
- Investment Committee member
- Pioneering researcher in economics and financial engineering at MIT
- Founder and CSO of multi-billion dollar hedge fund

Charles Homcy, MD — Chairman of Pharmaceuticals
- World renowned expert in drug discovery
- Developed >10 marketed drugs
- Founder, CEO, director of multiple $500M+ biotechnology companies
- MD Johns Hopkins; BA Johns Hopkins

Richard Scheller, PhD — Chairman of R&D
- Former CSO and EVP of Research and Early Development at Genentech
- CSO of 23andMe
- Lasker Award winner for pioneering CNS research
- Stanford and HHMI Faculty
- PhD Caltech; BS Univ of Wisconsin

Brian Stephenson, PhD — Chief Financial Officer
- Partner at Capital IP
- Director / VP at Leerink
- Manager at McKinsey
- PhD / MS MIT; BS Brigham Young

Hoyoung Huh, MD, PhD
- Investment Committee member
- Leader of multiple $100M+ biotech companies including Epizyme, Geron, BiPar, Nektar
- Partner, McKinsey & Co.
- MD / PhD Cornell; BS Dartmouth

Robert Zamboni, PhD
- Medical Chemist for BridgeBio and portfolio companies
- ACS Heroes of Chemistry Award for discovery of Singulair® (montelukast sodium)
- Author of over 80 papers, holder of over 20 patents
- PhD McGill; BS Yale

Companies

Genentech · Takeda MILLENNIUM · ONYX PHARMACEUTICALS · PORTOLA PHARMACEUTICALS · MyoKardia · MERCK FROSST · 23andMe · globalblood THERAPEUTICS · Epizyme · EXELIXIS pharmaceuticals, inc.

Drugs

TECENTRIQ atezolizumab · OCREVUS ocrelizumab · VELCADE (bortezomib) · Kyprolis (carfilzomib) · Enbrel etanercept · VENCLEXTA venetoclax tablets · Kadcyla trastuzumab emtansine · Entyvio vedolizumab · Nexavar (sorafenib) tablets · Andexxa · PERJETA pertuzumab · INTEGRILIN (eptifibatide) Injection · SINGULAIR (montelukast, MSD) · VIOXX (rofecoxib, MSD) · ARCOXIA (etoricoxib)

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Industry-leading R&D experts who have led 100+ INDs and the approval of 20+ marketed drugs

Robert Zamboni, PhD

- ACS Heroes of Chemistry Award for discovery of Singulair
- Developed Vioxx and Arcoxia (marketed in Europe)
- Holds over 20 patents
- Involved in DP antagonist and Catk inhibitor
- Several dozen development programs
- Retired from Merck in 2005 as VP of Medicinal Chemistry

Anjali Pandey, PhD

- 11 INDs and 1 NDA filed
- SVP of Medicinal Chemistry and Chemical Development at Portola
- Director of Chemistry at COR and Millennium
- 25+ years experience in industry

Hector Rodriguez, PhD

- 7 INDs filed for first in class agents in oncology, CV and inflammation
- Senior Director and founding member of biochemistry groups at Myokardia, Calithera, and Arresto
- Founder and SAB member of Edgewise Therapeutics

Athiwat Hutchaleelaha, PhD

- 2 marketed drugs including Bevyxxa and Andexxa from preclinical discovery through commercialization
- 20+ years in large and small molecule development at Global Blood Therapeutics, Portola, Millennium and COR

Andreas Betz, PhD

- Preclinical development of small molecule protease and kinase inhibitors
- Lead enzymologist in discovery and development of Bevyxxa
- 20+ years experience in industry at COR, Pharmacia-Pfizer, Chiron and Global Blood Therapeutics

Uma Sinha, PhD

- CSO of Global Blood Therapeutics, VP, Head of Biology and VP, Translational Biology at Portola
- 21 INDs and 4 marketed drugs, co-inventor of Andexxa
- 30+ years in biotech industry
- Discovery and preclinical development of small molecules in hematology, CV and inflammatory diseases.

James Kanter

- 25+ INDs filed at companies including Millennium, Exelixis, Pharmacofore, Gilead and Ardelyx
- 5 NDAs including Velcade, Sovaldi, Vemlidy and Vitekta
- Managed 4 drug substance commercial products
- 12+ drug substance, 3 drug product validations in NA, Europe and Asia

Jesper Jernelius, PhD

- 11+ INDs filed with Roche, Gilead and Medivation
- 3 NDAs for marketed drugs with Gilead (Sovaldi), Amgen (Kyprolis), and Medivation (Talazoparib)

Satish Rao, PhD

- 8 INDs filed at Endocyte, ARYx, and Scios
- 20+ years experience in DMPK and bioanalytical in industry, CROs, and academia

Ben Collman

- 11+ INDs and 3 NDAs filed at Pfizer, Abbott/AbbVie, Gilead and Ardelyx
- Worked on 8 marketed drugs including Lyrica, Steglatro, Xeljanz, Daurismo, Vizimpro, Technivie, Biktarvy, and Ibsrela
- 18+ years experience in CMC

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BridgeBio: A new model for the genetic disease space

Our model

Fit-for-purpose program building
Minimum viable program startup with extreme willingness to fail.

Aligned agendas
Incentives at the level of each program for program management teams and scientists.

Play-calling on the field
Decentralized decision-making left to the most appropriate decision makers.

The genetic disease space

Deep understanding of a privileged target space
Comprehensive mapping of the Mendelian disease space allows us to identify high value opportunities and novel approaches.

Unencumbered target selection
New opportunities not limited by potential peak-year sales or existing infrastructure.

Capabilities to enter the "chasm of death"
Unparalleled team of experienced translational scientists and company builders that enables us to pursue ultra-early-stage academic assets.

We believe performing more "reps" with the BridgeBio approach will create "flywheel momentum"

Mastery of executional efficiency
The right MVC 'minimum viable corporation' structure, amount of capital, and talent phenotypes

Reputation
Recognition from academics and top-tier talent that we are an ideal partner

We advanced our first two programs from academia to Ph 3 trials in three years

Academic origins

World-class biotech R&D

Advancement to pivotal trials, value inflection

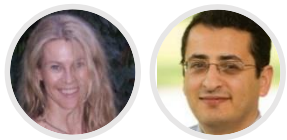
BBP-265
TTR stabilizer for ATTR (Eidos)

PNAS

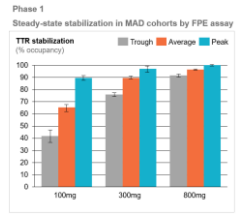
AG10 inhibits amyloidogenesis and cellular toxicity of the familial amyloid cardiomyopathy-associated V122I transthyretin

Sravan C. Penchala[a,1], Stephen Connelly[b,c,1], Yu Wang[a,1], Miki S. Park[d], Lei Zhao[f], Aleksandra Baranczak[f], Irit Rappley[f], Hannes Vogel[g], Michaela Liedtke[g], Ronald M. Witteles[e], Evan T. Powers[f], Natàlia Reixach[b], William K. Chan[a], Ian A. Wilson[b], Jeffery W. Kelly[f], Isabella A. Graef[d,2], and Mamoun M. Alhamadsheh[a,2]

[a]Department of Pharmaceutics and Medicinal Chemistry, University of the Pacific, Stockton, CA 95211; Departments of [b]Integrative Structural and Computational Biology and [c]Molecular and Experimental Medicine, The Scripps Research Institute, La Jolla, CA 92037; and [d]Department of Pathology and [e]Division of Cardiovascular Medicine, Stanford University School of Medicine, Stanford, CA 94305



SPARK AT STANFORD



IPOS

Eidos, Avrobio shoot for $201M as biotech IPOs continue to roll forward on Nasdaq

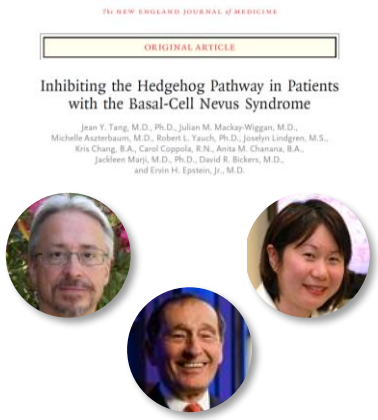
by JOHN CARROLL — on May 29, 2018 07:13 AM EDT

MarketWatch

Eidos Therapeutics stock soars in debut

By Emily Bary
Published: June 20, 2018 10:52 a.m. ET

BBP-009
Topical SMOi for Gorlin / BCC (PellePharm)

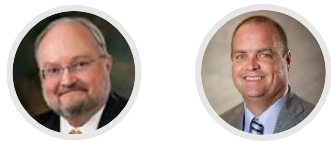


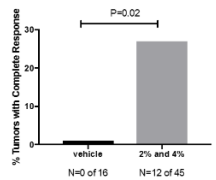
The NEW ENGLAND JOURNAL of MEDICINE

ORIGINAL ARTICLE

Inhibiting the Hedgehog Pathway in Patients with the Basal-Cell Nevus Syndrome

Jean Y. Tang, M.D., Ph.D., Julian M. Mackay-Wiggan, M.D., Michelle Aszterbaum, M.D., Robert L. Yauch, Ph.D., Joselyn Lindgren, M.S., Kris Chang, B.A., Carol Coppola, R.N., Anita M. Chanana, B.A., Jackleen Marji, M.D., Ph.D., David R. Bickers, M.D., and Ervin H. Epstein, Jr., M.D.





FierceBiotech

BIOTECH RESEARCH CRO MEDTECH

Biotech

LEO Pharma inks $760M rare skin disease R&D deal with PellePharm

by Conor Hale | Nov 20, 2018 6:00am

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Track record of capitally efficient value creation

BBP-265
TTR stabilizer
for ATTR (Eidos)

Cost: $25M

Time: 2 years

IPO at $625M post
(6/19/2018)

BBP-009
Topical SMOi for
Gorlin / BCC
(PellePharm)

Cost: $26M

Time: 3.5 years

Up to $760M* for late-stage funding, option to acquire and milestones

*Note: Total deal value of $760M; BridgeBio owns 43.3% of PellePharm (as of 12/31/2018)

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Key portfolio value drivers

Program / MOA	Disease	Patient population, '000s (US+EU)	Description
BBP-265 / TTR stabilizer	Transthyretin Amyloidosis	400	• Potentially most potent TTR stabilizer designed for the treatment of ATTR • One of the largest Mendelian diseases
BBP-831 / FGFR1-3i	Achondroplasia	55	• Only known oral FGFR1-3i in clinical development for achondroplasia* • No approved standard of care in the US or EU • Strong pre-clinical data and only oral product in development • Also being pursued for a variety of FGFR-driven cancers
BBP-454 / Pan-mutant KRASi	KRAS+ Cancers	500	• Pan-mutant KRAS inhibitor program for KRAS-driven cancers • ~30% of all cancers are driven by KRAS mutations, including large proportions of lung, colorectal and pancreatic tumors
BBP-631 / AAV5 21-OH gene replacement	Congenital Adrenal Hyperplasia	75	• Gene therapy product candidate for CAH • Likely one of the largest gene therapy markets, with prevalence >75,000 in the US and EU • High unmet need, with poor treatment options and lifelong risk of potentially fatal adrenal crisis
Total patient population for key portfolio value drivers		1,030	**These four assets alone could provide novel treatment options for over 1M people in the US and EU**

*Based on clinicaltrials.gov search

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AG10 (BBP-265): Potentially most potent transthyretin stabilizer for the treatment of transthyretin amyloidosis

Mechanism of Disease

Native transthyretin circulates in blood as a tetramer

Dissociation into toxic monomers initiates pathogenesis

Monomers aggregate and deposit as amyloid, causing disease
ATTR-CM, ATTR-PN

Impact on patients:

- Progressive, fatal cardiomyopathy (median life expectancy 3-5 yrs) and polyneuropathy
- Both manifestations includes significant disability
- We believe the diagnosed population of ATTR-CM is growing rapidly due to awareness and accurate, non-invasive, diagnostic methods

Mechanism of Drug

AG10 stabilizes TTR tetramer, potentially preventing disease

Dissociation into toxic monomers initiates pathogenesis

Monomers aggregate and deposit as amyloid, causing disease
ATTR-CM, ATTR-PN

AG10 is designed to bind TTR in a way that mimics a naturally-occurring protective mutation

Program Highlights

400k+
ATTR-CM patients worldwide

10k+
ATTR-PN patients worldwide

Clinical status:

Pre-IND	Phase 1	Phase 2	Phase 3

ATTR-CM Ph3 study ongoing (FPI 1Q19)

ATTR-PN Ph3 study expected 2H19

Catalysts:

- Ph2 ATTR-CM OLE data in 2H19
- Ph3 ATTR-CM 12-month data in 2021
- Potential ATTR-CM NDA submission in 2021

Key data:

- Ph2 ATTR-CM data presented in 2H18
  - Normalized serum TTR in all actively treated patients at d28
  - TTR stabilization of ≥90% in all actively treated pts at d28
- Ph3 ATTRibute study initiated in 1Q19
  - Potential registration on 12m 6MWD endpoint, followed by 30m CV outcome/hospitalization endpoint

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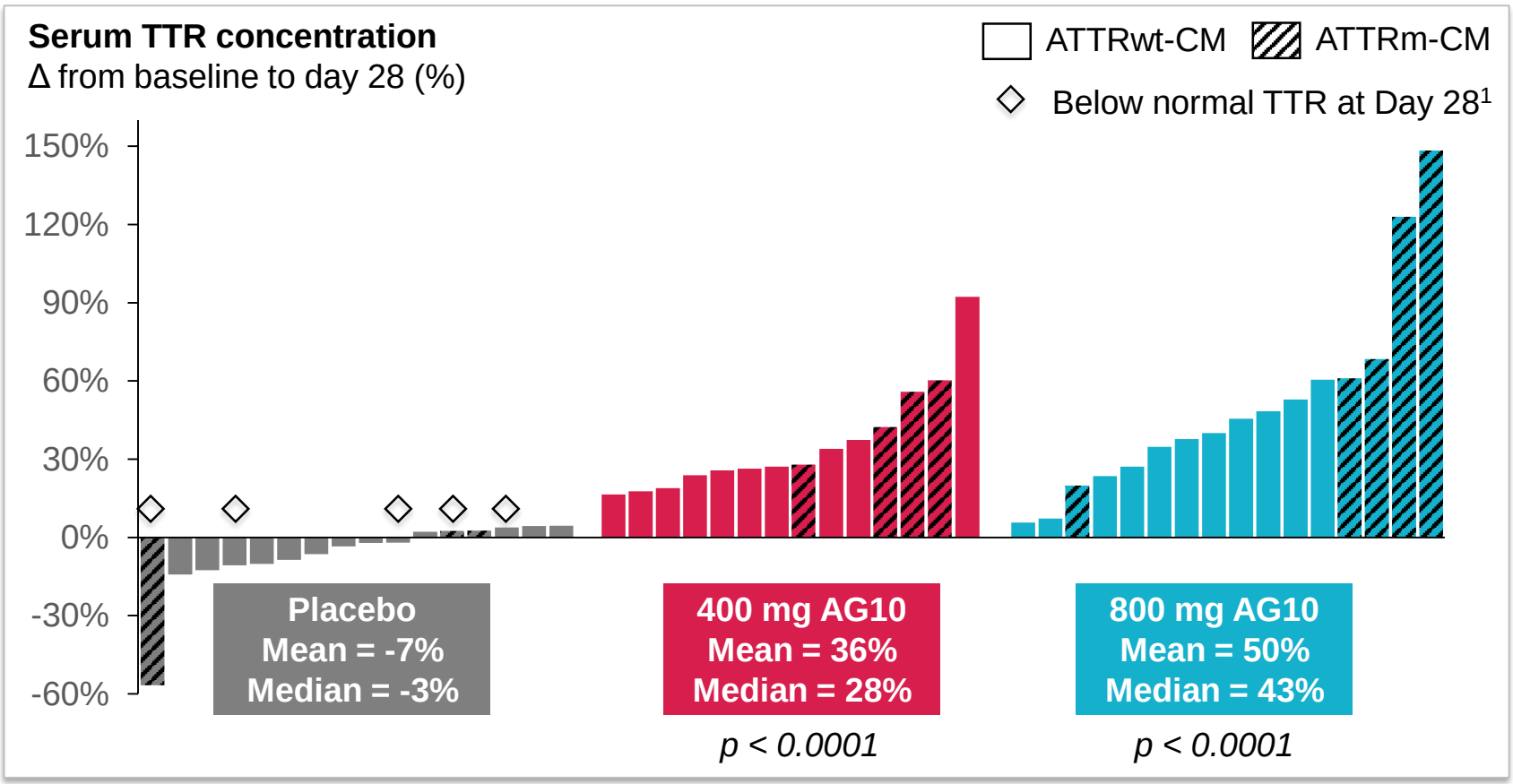
Treatment with AG10 restored serum TTR concentrations to normal range in all subjects



- **Dose-dependent increase in serum TTR concentrations in AG10-treated subjects**
- **Greater treatment effect observed in ATTRm subjects – final TTR concentrations normalized to comparable levels as treated ATTRwt subjects**

1 Normal reference range for serum TTR 20-40 mg/dL (3.6-7.3 µM)

Source: AG10 data on file

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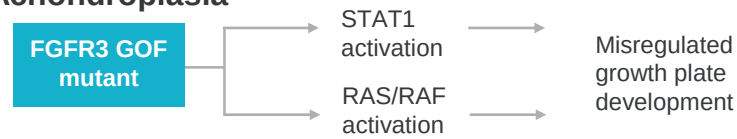
Infigratinib (BBP-831): Oral FGFR1-3 inhibitor to treat achondroplasia and FGFR-driven cancers

Mechanism of Disease

Achondroplasia

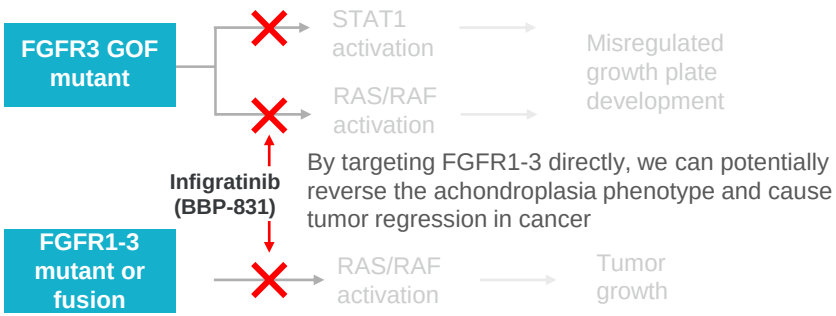


Impact on patients:
- Short stature, spinal compression, narrow foramen magnum
- Increased risk of infant death, sleep apnea, infections
- No currently approved therapies in the US or EU

Oncology

Impact on patients:
- Poor survival, multiple comorbidities that impact QoL

Mechanism of Drug



Program Highlights

55k

Achondroplasia pts in US+EU

37k

Annual new FGFR1-3+ oncology diagnoses in US+EU

Clinical status:

Pre-IND | Phase 1 | Phase 2 | Phase 3

ACH Ph1/2 planned for 2020

Oncology Ph3 enrolling (1L CCA)

Catalysts:

- Achondroplasia IND acceptance and FPI 2020 (expected)
- Achondroplasia clinical data 2021
- Potential oncology NDA in 2020 (2nd line cholangiocarcinoma, CCA)

Key data:

- Achondroplasia:
  - Strong preclinical data with effects on stature, foramen magnum and lumbar disc width in mouse model
  - Only known oral therapy in development
  - Anticipate active dose significantly lower than oncology
- Meaningful clinical data in oncology indications CCA and UC (26.9% and 25.4% ORR respectively)

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Infigratinib showed significant improvements in key disease features in the achondroplasia mouse model

Preclinical data from infigratinib

Ph1/2 clinical study to initiate by 2020 for infigratinib, the only oral asset in development

Percent increase compared to non-treated mouse

Asset	Company	Status	Mouse model	Tibia length	Femur length	Foramen magnum area	L4-L6 height
Infigratinib	bridgebio	Pre-Ph1/2	FGFR3$^{Y367C/+}$	33%	21%	16%	12%

Source: Komla-Ebri et al. J Clin Inv 2016
Note: Infigratinib dose of 2mg/kg

Preclinical data from other investigational achondroplasia therapies

Percent increase compared to non-treated mouse

Asset	Company	Status	Mouse model	Tibia length	Femur length	Foramen magnum area	L4-L6 height
Vosoritide (BMN111)	BIOMARIN	Pivotal	FGFR3$^{Y367C/+}$	7%	5%	No known publically available data	3%
TransCon CNP[1]	ascendis pharma	Ph1/2	FGFR3$^{Y367C/+}$	12%*			
TA-46	Therachon achieving potential	Ph1/2	FGFR3$^{ach/+}$	9%*	7%*		

Source: Lorget et al. Am J Hum Genet 2012, Garcia et al. Science Trans Med 2013, Breinholt ENDO 2017
Note: subcutaneous doses, percent increase compared to vehicle treated FGFR3Y367C/+, FGFR3ACH/+ mouse unless otherwise noted
1 Based on vosoritide continuous infusion; *Value estimated using DigitizeIt

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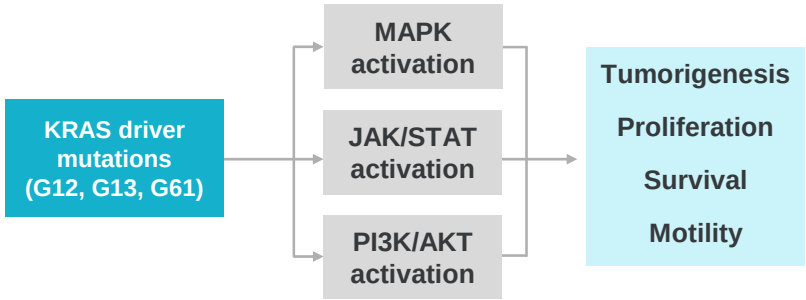
BBP-454: Pan-mutant KRAS inhibitor to treat lung, colon, and pancreatic cancer

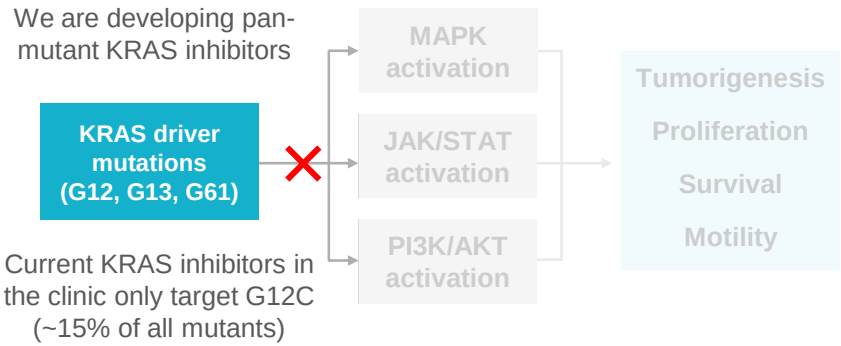
Mechanism of Disease



Impact on patients:

- KRAS is one of the well-known monogenic drivers of cancer
- ~30% of all cancers carry KRAS mutations (high proportions of lung, colorectal, pancreatic)

Mechanism of Drug



Program Highlights

500k+

Patients/year in US+EU are diagnosed with KRAS+ cancer

Tumor type	KRAS+ frequency
NSCLC	30%
Colorectal cancer	45%
Pancreatic cancer	98%

Development status:

Pre-IND	Phase 1	Phase 2	Phase 3

BBP-454

Catalysts:

- Development candidate selection and IND filing

Key info:

- Platform-like approach to KRAS inhibition driven by a world class research coalition:
  - Led by globally recognized RAS expert Frank McCormick
  - Discovery partnerships with NCI RAS initiative and Lawrence Livermore National Labs
- Two pan-mutant approaches being optimized
  - C185 binders block membrane tethering and activation
  - H95 binders trigger KRAS degradation
- Multiple novel pockets discovered through computational chemistry
- Co-crystal structure solved for H95 compound series

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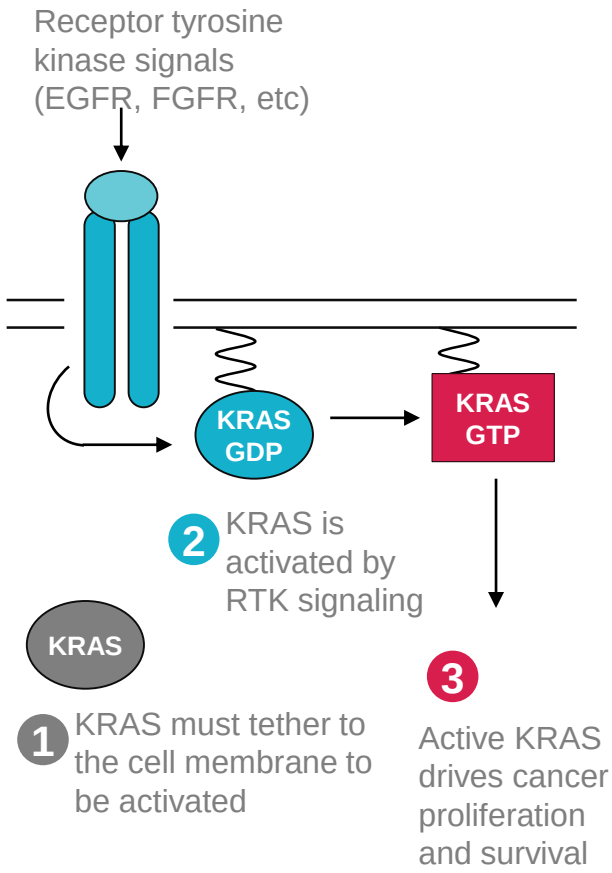
We have two shots on goal with our pan-mutant KRAS inhibitor programs – each with a unique and novel MOA

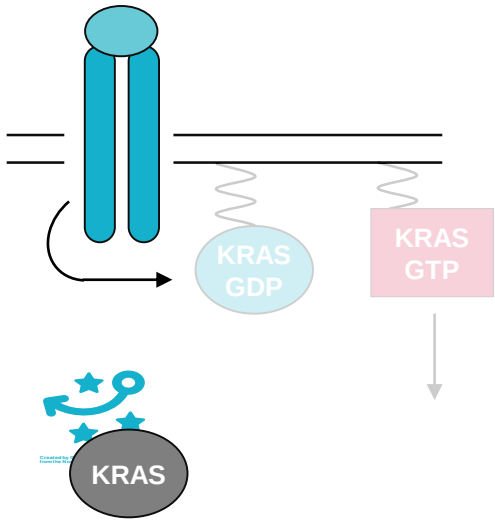
KRAS activation in cancer is a multi-step process



Our two programs target different steps of the KRAS activation process

1st approach: C185 targeting

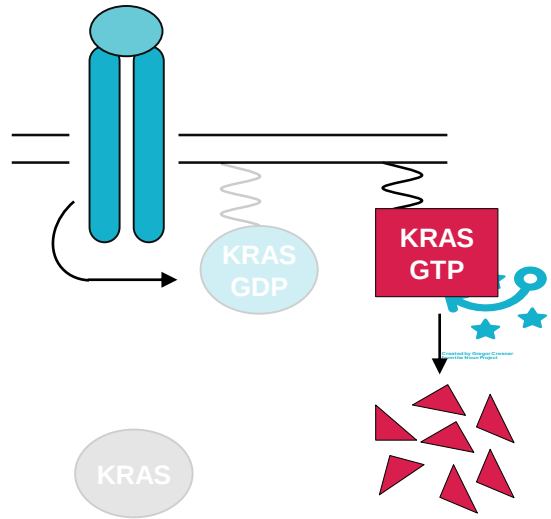
- Blocks KRAS from tethering
- Blocks activated KRAS GTP from signaling



KRAS tethering is blocked – cancer growth is inhibited

2nd approach: H95 targeting

- Directly binds activated KRAS
- Triggers KRAS degradation, blocking the signal



Activated KRAS is degraded – cancer growth is inhibited

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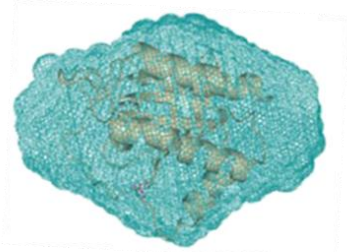
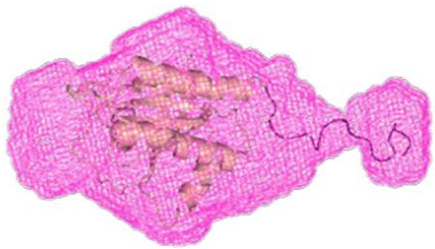
We've built out our C185 screening cascade with additional biophysical and cellular assays, enabling major advances

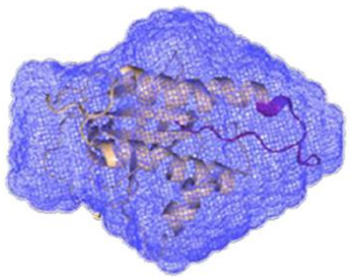
Small angle X-ray scattering supports our hypothesis



KRAS without HVR



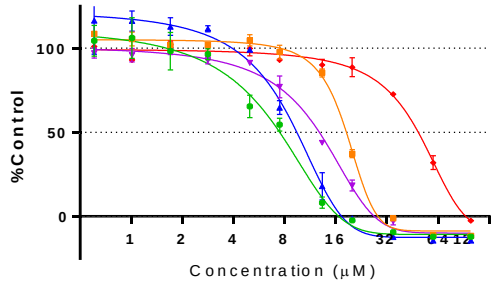
Full KRAS



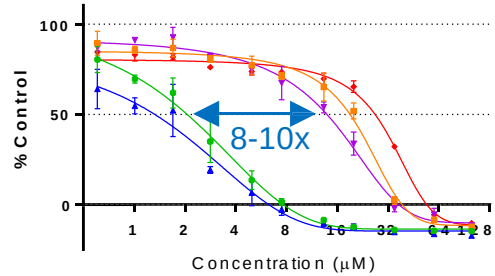
Full KRAS + our compound

RASless MEFs are our first pass selectivity screen

2017

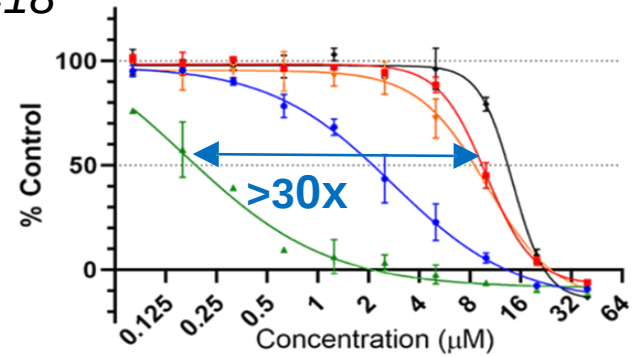


Q218



IC50	1.5uM
Selectivity	8-10x

Q418



IC50	250nM
Selectivity	>30x

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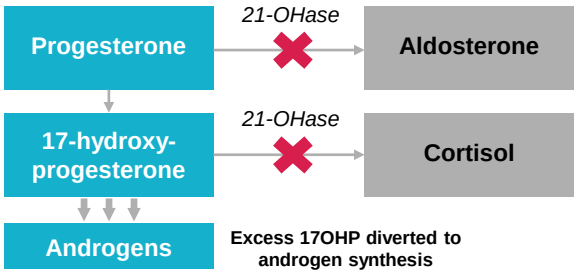
BBP-631: Gene therapy for CAH caused by 21OH deficiency

Mechanism of Disease

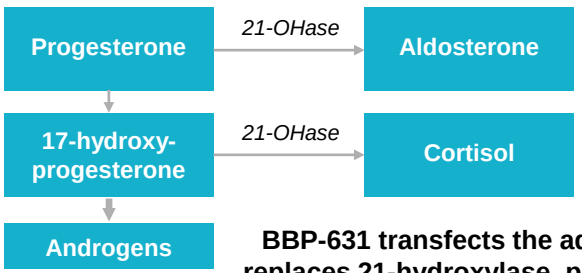
Steroid synthesis pathway



Impact on patients:

- Adrenal crises (can be fatal)
- Lifelong treatment with supraphysiologic steroid, which can cause significant morbidity (CV disease, obesity, bone disease)
- Abnormal sexual development, infertility

Mechanism of Drug



BBP-631 transfects the adrenal glands and replaces 21-hydroxylase, potentially restoring production of aldosterone and cortisol and reducing excess synthesis of androgens

Program Highlights

75K+

Patients with CAH in US+EU

1 / 11,000

Newborns born with CAH

Development status:

Pre-IND	Phase 1	Phase 2	Phase 3

BBP-631

Catalysts:

- IND filing in 2020
- Anticipated clinical proof-of-concept data in 2021

Key info:

- Durability of expression shown in NHP studies; sustained vector copy number and RNA expression out to at least 6 months
- Clinical GMP manufacturing underway at Paragon; in-house process development and analytical capabilities being developed
- Vector construct designed by Dr. Guangping Gao, a world leader in AAV design
- Genotype-phenotype studies show that 5-10% of enzyme activity may be sufficient for clinical impact

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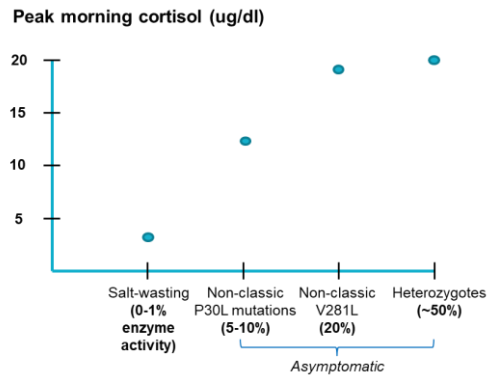
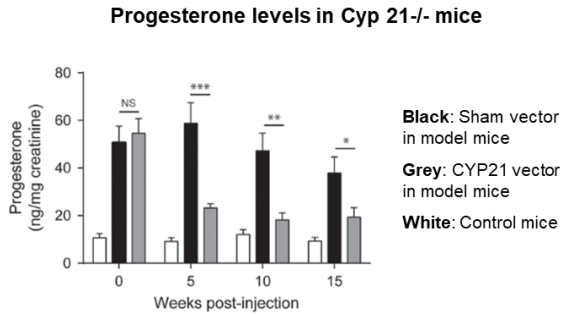
BBP-631 NHP experiments show durable transgene expression; 5-10% of WT enzyme may be sufficient for clinical impact

Genotype-phenotype studies show that >5-10% of enzyme activity results in nonclassical CAH



- Due to the high enzymatic efficiency/selectivity of 21-OHase, **only a small amount of enzyme is required to rescue the phenotype**

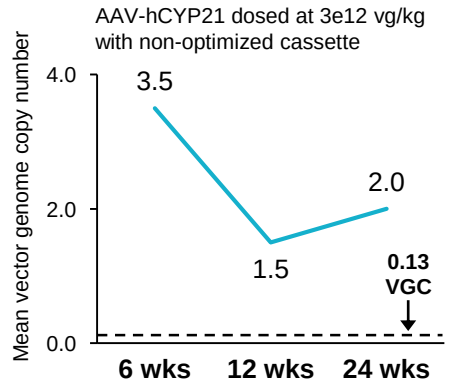
Mouse studies show a VGC of only 0.13 at 18 wks was sufficient for phenotypic reversal



VGC from the whole adrenal gland was 0.13 at wk 18

- At 15 weeks in treated mice, **progesterone** (the key substrate of 21OHase in mice) was **significantly reduced vs untreated mice**

Ongoing NHP studies show sustained VGC and RNA out to at least 6 months



- Mean vector genome copies per cell appear stable at 24 wks
- VGC of 2.0 at 24 wks is >10x the level required to correct the CAH phenotype in mice

Catalyst map

	2019	2020	2021
★ BBP-265 / TTR stabilizer	Ph 3 CM Initiation✓ Ph 3 PN Initiation Ph 2 OLE Data		Ph 3 CM Data
★ BBP-831 / FGFR 1-3 inhibitor	1L CCA FPI	Achondroplasia Ph 1/2 FPI 2L+ CCA NDA Adjuvant UC Phase 3 FPI	Achondroplasia PoC Data
★ BBP-631 / CAH gene therapy		IND	POC Data
★ BBP-454 / Pan-mutant KRASi	H95 Crystal Structure✓	DC Nomination	
BBP-870 / cPMP replacement	Rolling NDA	Approval + PRV	
BBP-009 / Topical SMO inhibitor	Gorlin Ph 3 1st Pt Dosed✓ HF-BCC Ph 2b Initiation	Gorlin Ph 3 Data	
BBP-681 / Topical PI3Ka inhibitor		IND	
BBP-589/COL7 protein replacement	Ph 1/2 1st Pt Dosed✓	Ph 1/2 PoC Data	
BBP-812 / Canavan gene therapy		IND	
BBP-398 / SHP2 inhibitor		IND	
BBP-711 / GO1 inhibitor		IND	
BBP-561 / KLKN5/7 inhibitor		IND	
BBP-671 / PanK1/3 activator		IND	
BBP-954 / GPX4 inhibitor			IND
BBP-671 / Succinate prodrug			IND

★ Key value driver ✓ Achieved

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