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ML Bio Solutions

MENA Congress for Rare Diseases

May 16-19 2024

MENA Congress for RARE DISEASES

16-19 May 2024, Beach Rotana, Abu Dhabi, UAE

IN PARTNERSHIP WITH BMC BURJEEL MEDICAL CITY

By Burjeel Holdings

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LGMD2I, LGMDR9 FKRP-Related (LGMD2I/R9) is caused by mutations in FKRP and characterized by an established genotype/phenotype association

	Birth	Early Childhood	Late Childhood	Adolescence to adulthood
L276I Homozygotes **Prevalence** *(L276I/L276I)* **68%**	Asymptomatic	Asymptomatic	▪ **Age of symptom onset 18 ± 3 years old** ▪ Lower limb & proximal weakness ▪ +/-calf hypertrophy, muscle pain, ↑ serum CK levels	**Loss of ambulation:** 25% by age 40 **Respiratory decline**: Non-invasive assistance required by 10% by age 40 and invasive assistance required by <1% **Cardiac dysfunction**: ~30%
Other FKRP genotypes **Prevalence** *(L276I/ non-L276I)* **29%** *(non-L276I/ non-L276I)* **2%**	Asymptomatic	▪ **Age of symptom onset 5 ± 1 years old** ▪ Lower limb & proximal weakness ▪ +/-calf hypertrophy, muscle pain, ↑ serum CK levels	▪ Lower limb & proximal weakness ▪ +/-calf hypertrophy, muscle pain, ↑ serum CK levels	**Loss of ambulation:** most by age 20 **Respiratory decline**: Invasive assistance required by 5% by age 30 **Cardiac dysfunction**: ~60%

Source: 1) Sveen et al, Annals of Neurology, 2006; 2) Richard et al, Neuromuscular Disorders, 2016 3) Gedlinske et al, Neurology, 2020 4) GnomAD database 5) Liu, et al, Genet Med., 2018 6) Libell et al, Muscle Nerve, 2020

Alpha Dystroglycan (αDG), disrupted in LGMD2I/R9, is an integral part of the dystrophin-glycoprotein complex

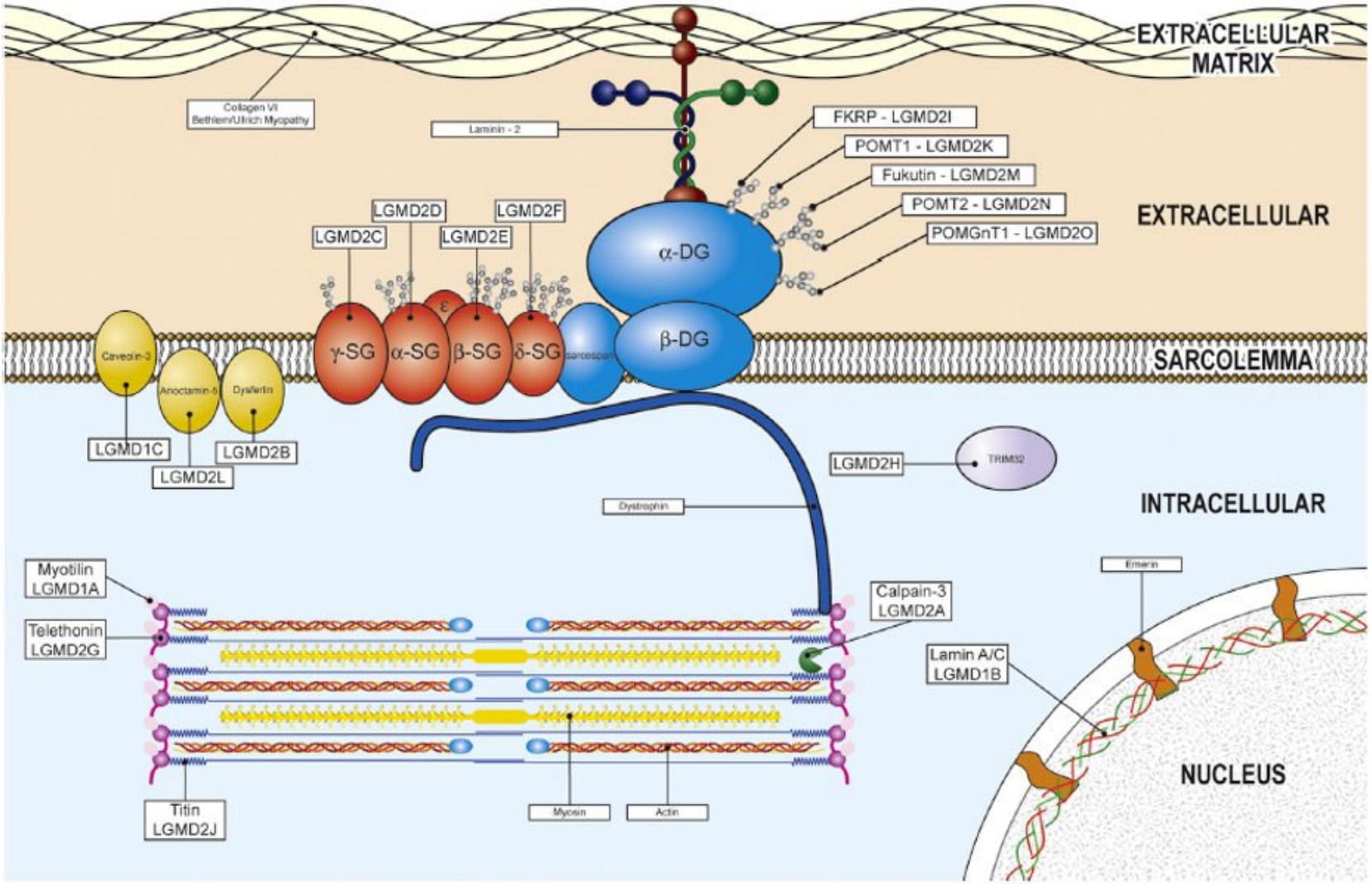


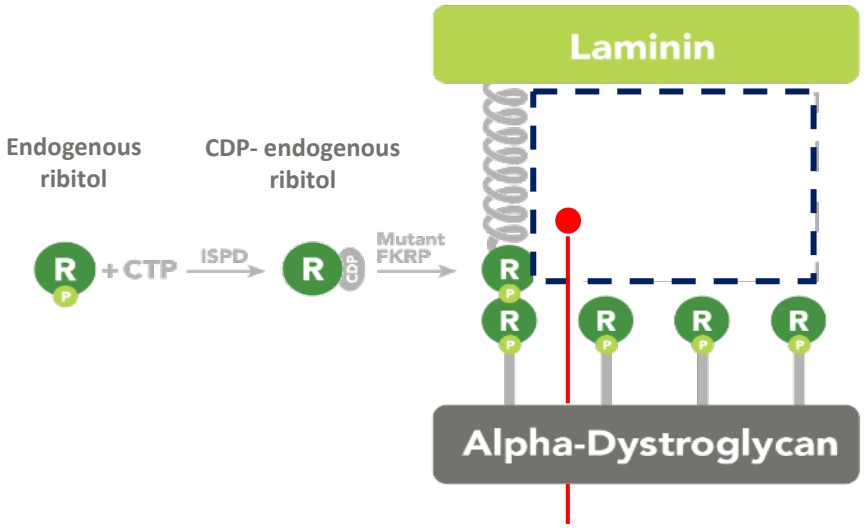
Figure from Wicklund et al., The limb-girdle muscular dystrophies. Neurologic Clinics, 2014.

Oral BBP-418 is under investigation as an upstream substrate supplement to drive residual activity of mutant FKRP in LGMD2I/R9, targeting the disease at its source

LGMD2I/R9 Disease Mechanism

Functional FKRP fully glycosylates alpha-dystroglycan (αDG) which stabilizes myocytes by binding extracellular ligands to act as a "shock absorber" for muscle fibers

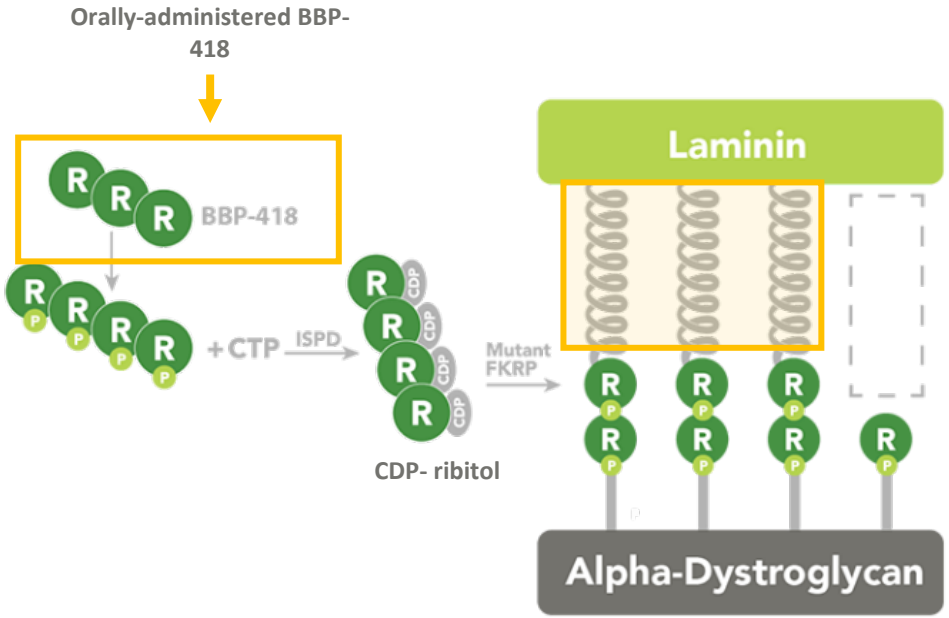
Partial loss of function mutation in FKRP results in dysfunctional, hypo-glycosylated αDG in myocytes which increases susceptibility to damage



Mutations in FKRP prevent addition of ribitol-5-P to alpha-dystroglycan (hypo-glycosylated αDG) limiting αDG's ability to function as a "shock absorber" for muscle fibers

Proposed BBP-418 Therapeutic Approach

Supply supraphysiological levels of pharmaceutical-substrate upstream aiming to drive residual activity of mutant FKRP enzyme and increase αDG glycosylation levels

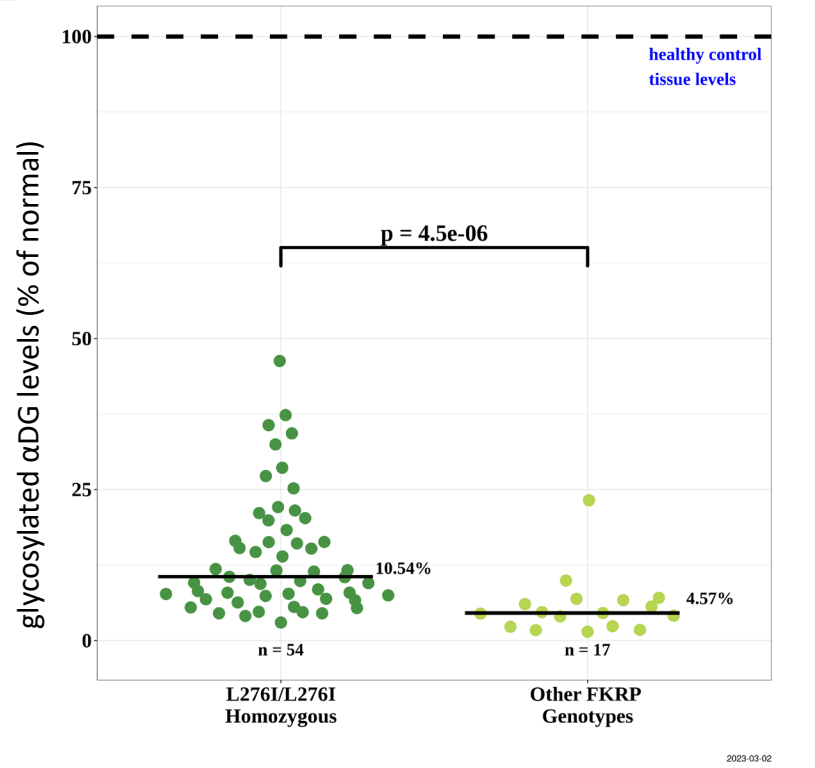


Potential partial restoration of αDG glycosylation

Courtesy from and proprietary to: ML Bio Solutions Inc., a BridgeBio company

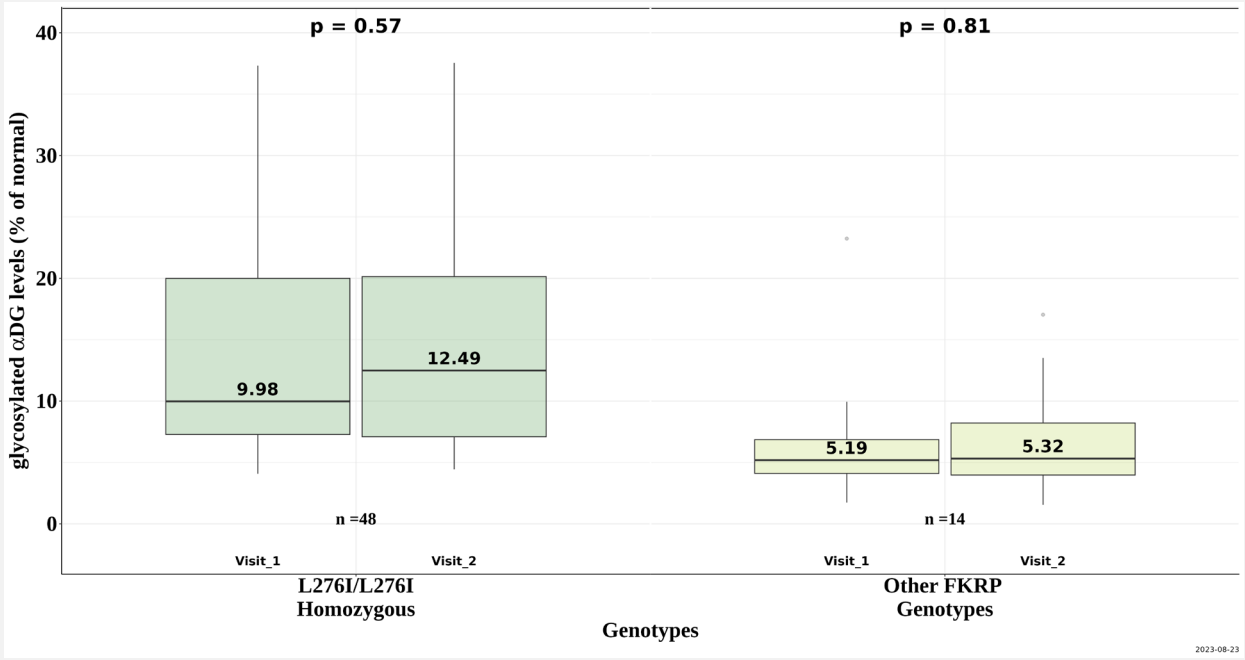
Natural history data supports the premise that glycosylation of αDG in muscle mirrors the severity of LGMD2I/R9 disease and remains stable over time

Reduced αDG glycosylation in other *FKRP* genotypes vs. L276I/L276I homozygous LGMD2I/R9 patients



Source: MLB-01-001 Listing 16.4.1 and 16.1.4.2.

Glycosylated αDG levels remain stable over 6–12 months in untreated LGMD2I/R9 patients



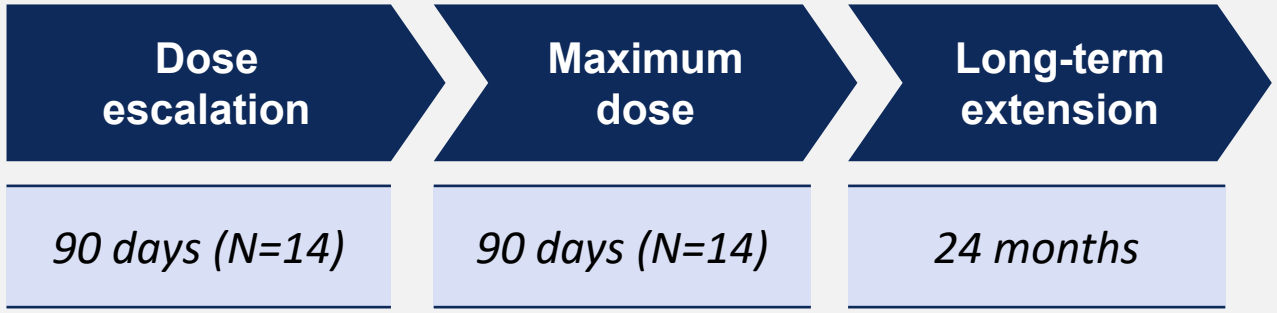
Source: MLB-01-001 Listing 16.4.1 and 16.1.4.2.

Other *FKRP* genotypes, which are more rare and typically have a more severe clinical presentation, have lower glycosylated αDG levels compared to L276I/ L276I homozygous patients; both groups have reduced levels compared to healthy individuals

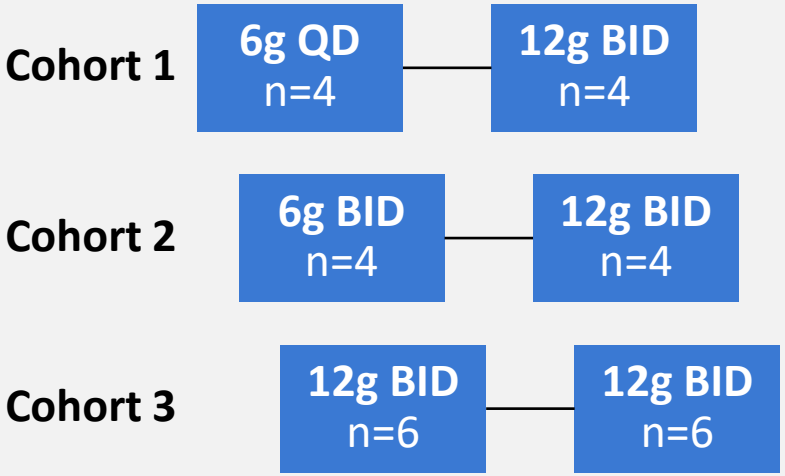
Patient samples were interpolated to standard curve to determine % of normal glycosylation of αDG; lines show medians; figure includes all patients with biopsies in MLB-01-001

Patient samples were interpolated to standard curve to determine % of normal glycosylation of αDG; median and 25-75% percentile are shown; figure includes all patients with repeat biopsies in MLB-01-001

BBP-418 is under investigation in a small, open label Phase 2 study in individuals with LGMD2I/R9



After Part 1, all participants transitioned to highest dose 12g BID



Key Endpoints

- North Star Assessment for limb girdle type muscular dystrophies (NSAD)
- 10-meter walk test/100-meter timed test
- Forced vital capacity (FVC)
- Performance of Upper Limb (PUL2.0)
- Glycosylated αDG levels
- Serum creatine kinase (CK)

Key inclusion criteria

- Age between 12-55 years at enrollment
- Genetically confirmed LGMD2I/R9
- Body weight >30kg
- Able to complete 10MWT ≤12 seconds unaided (moderate disease) or unable to (severe disease)

Courtesy from and proprietary to: ML Bio Solutions Inc., a BridgeBio company

BBP-418 has been well tolerated, with only minor GI related adverse events recorded in the Phase 2 study

- 159 adverse events (AEs) were recorded in the study
- Most of the reported TEAEs were Grade 1 (mild) or Grade 2 (moderate) in severity
- 20 were considered to be related to BBP-418 with the most common being diarrhea and bloating
- No discontinuations or interruptions in therapy due to AEs
- 3 severe adverse events recorded and were considered unrelated to BBP-418

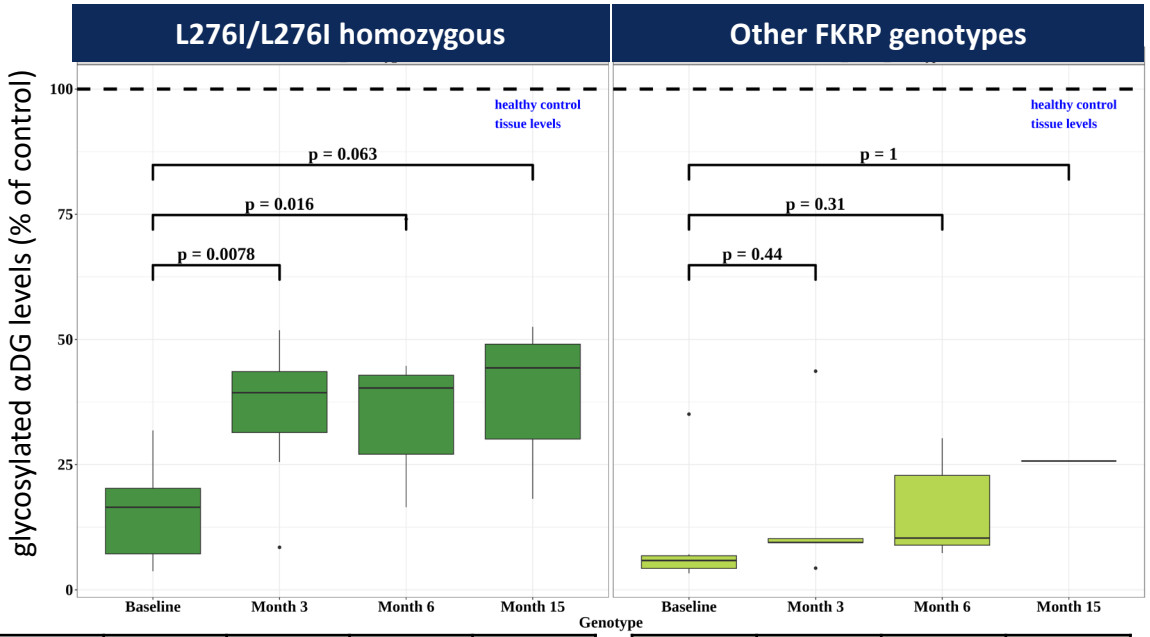
Treatment related TEAE	# of incidents	# of patients
Diarrhea	8	6
Dehydration	1	1
Nausea	3	2
Vomiting	2	2
Dyspepsia	1	1
Gastroenteritis	1	1
Bloating	2	2
Headaches	1	1
Abdominal pain	1	1
Overall	**20**	**9**

Source: MLB-01-003 Listing 14.3.1.3 Part 3 month 15

Preliminary data, subject to change

Data from an open-label Phase 2 study show that glycosylated αDG levels in muscle are responsive to therapy at 3 months and sustained over 15 months; this is supported by an impact on serum creatine kinase (CK)

Increase in glycosylated αDG in muscle observed post dosing with BBP-418 (median ± 95% CI)

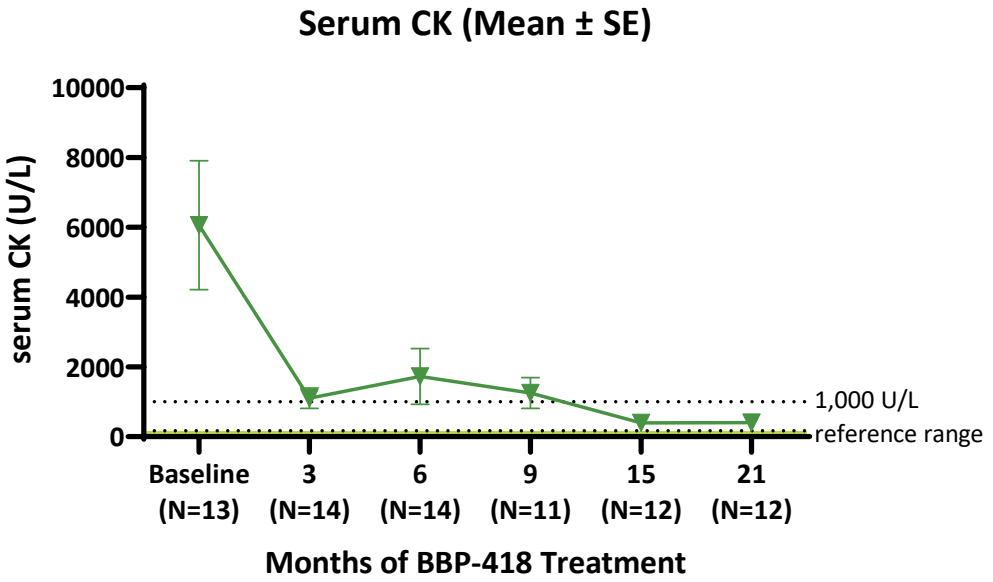


Median (%)	16.5	39.4	39.7	44.3	5.9	9.5	10.4	25.7
N	8	8	7	6	6	5	6	1

Patient samples were interpolated to standard curve to determine % of normal glycosylation of αDG
+ 3 mo = Part 1, 90-day; +6 mo = Part 2, Month 3; + 9 mo = Part 3, Month 3; + 15 mo = Part 3, Month 9
Median and 25-75% percentile are shown, Wilcoxon test was used to determine significance

Source: MLB-01-003 Listing 16.4.1 and 16.1.4.2.

Reduction in mean serum creatine kinase (CK) observed post dosing with BBP-418

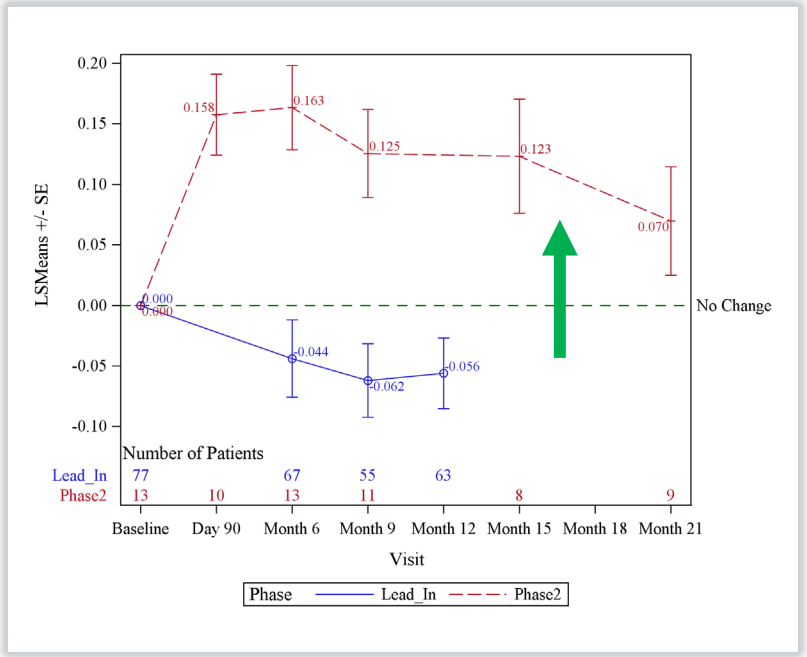


Cohort 1 Day 1 CK draw taken after functional assessments; all other draws done prior to functional assessment
After Day 90, all subjects received 12 g BID (weight-adjusted)
+ 3 mo = Part 1, 90-day; +6 mo = Part 2, Month 3; + 9 mo = Part 3, Month 3; + 15 mo = Part 3, Month 9; +21 mo = Part 3, Month 15; Reference range for CK is 55–170 units/L for men and 30–135 units/L for women, figure shows reference range from 30–170 units/L

Source: MLB-01-003 Table 14.2.1.1.

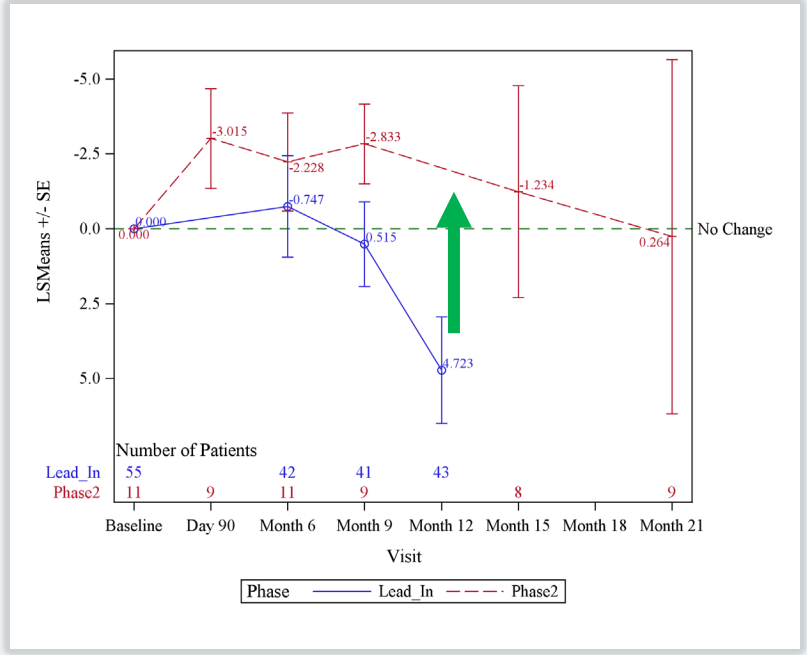
Stabilization in ambulatory and clinical measures observed after 21 months of dosing with BBP-418 in Phase 2 study

Change from baseline in 10MWT (m/s)



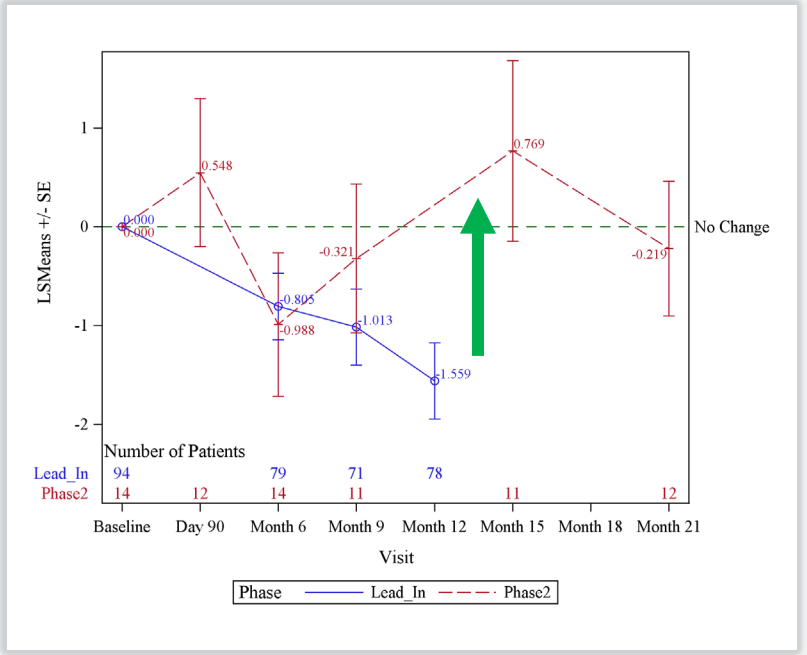
Source: MLB-01-001 Listing 16.2.1 and MLB-01-003 Listing 16.2.1

Change from baseline in 100MTT (s)



Source: MLB-01-001 Listing 16.2.2 and MLB-01-003 Listing 16.2.2

Change from baseline in NSAD



Source: MLB-01-001 Listing 16.2.5 and MLB-01-003 Listing 16.2.5

Blue lines denote natural history data and **red lines** denote on-treatment data collected during the Phase 2 study

Data exclude 1 subject from month 15 timepoint due to post-COVID decline

Phase 2 data: + 3 mo = Part 1, 90-day; +6 mo = Part 2, Month 3; + 9 mo = Part 3, Month 3; + 15 mo = Part 3, Month 9; +21 mo = Part 3, Month 15

Preliminary data, subject to change

Summary

Phase 2 Study (MLB-01-003)

- Increased glycosylation of αDG observed following BBP-418 dosing which is sustained over time
- Large, sustained reduction in CK observed over an extended (up to 21-months) treatment period
- Stabilization in NSAD and ambulatory measures observed over 21-month treatment period
- No treatment-related SAEs or dose limiting toxicities observed with BBP-418

Phase 3 FORTIFY Study (MLB-01-005)

- The Phase 3 FORTIFY study is a double-blind, randomized, placebo-controlled clinical trial actively enrolling at sites in the US, EU, UK, and Australia

Future Development

- We hope to explore the potential use of BBP-418 in other alpha-dystroglycanopathies LGMD2M/R13 Fukutin-related and LGMD2U/R20 ISPD-related

Thank You!

- ML Bio study participants
- LGMD2I/R9 patients, families, and patient advocates

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