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Limb-girdle Muscular Dystrophy Type 2I/R9 Investor Webinar

July 11, 2025

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Agenda

01	LGMD2I/R9 disease background		**Matthew Wicklund, MD, FAAN** Professor of Neurology Vice Chair of Research, Dept. of Neurology University of Texas at San Antonio
02	BBP-418 program overview		**Douglas Sproule, MD, MSc** Chief Medical Officer ML Bio Solutions, a BridgeBio Company
03	Looking ahead: Ph. 3 interim analysis & commercial opportunity		**Christine Siu** Chief Executive Officer ML Bio Solutions, a BridgeBio Company
04	Q&A		

LGMD2I/R9 (FKRP-related) is one of ~30 subtypes of limb-girdle muscular dystrophy, each driven by mutations in distinct genes impacting muscle function

Old nomenclature	Affected gene	New nomenclature
LGMD 1D	DNAJB6	LGMD D1 DNAJB6-related
LGMD 1F	TNP03	LGMD D2 TNP03-related
LGMD 1G	HNRNPDL	LGMD D3 HNRNPDL-related
LGMD 1I	CAPN3	LGMD D4 calpain3-related
Bethlem myopathy dominant	COL6A1, COL6A2, COL6A3	LGMD D5 collagen 6-related
LGMD 2A	CAPN3	LGMD R1 calpain3-related
LGMD 2B	DYSF	LGMD R2 dysferlin-related
LGMD 2C	SGCG	LGMD R5 γ-sarcoglycan-related
LGMD 2D	SGCA	LGMD R3 α-sarcoglycan-related
LGMD 2E	SGCB	LGMD R4 β-sarcoglycan-related
LGMD 2F	SGCD	LGMD R6 δ-sarcoglycan-related
LGMD 2G	TCAP	LGMD R7 telethonin-related
LGMD 2H	TRIM32	LGMD R8 TRIM32-related
LGMD 2I	**FKRP**	**LGMD R9 FKRP-related**
LGMD 2J	TTN	LGMD R10 titin-related
LGMD 2K	POMT1	LGMD R11 POMT1- related
LGMD 2L	ANO5	LGMD R12 anoctamin5- related
LGMD 2M	FKTN	LGMD R13 Fukutin-related
LGMD 2N	POMT2	LGMD R14 POMT2- related
LGMD 2O	POMGnT1	LGMD R15 POMGnT1-related
LGMD 2P	DAG1	LGMD R16 α-dystroglycan-related
LGMD 2Q	PLEC1	LGMD R17 plectin-related
LGMD 2S	TRAPPC11	LGMD R18 TRAPPC11- related
LGMD 2T	GMPPB	LGMD R19 GMPPB-related
LGMD 2U	ISPD	LGMD R20 ISPD-related
LGMD 2X	BVES	LGMD R25 BVES-related
LGMD 2Z	POGLUT1	LGMD R21 POGLUT1-related
Bethlem myopathy recessive	COL6A1, COL6A2, COL6A3	LGMD R22 collagen 6-related

Straub V et al, Neuromuscul Disord, 2018

An updated set of nomenclature for LGMDs was defined in 2018; however, many patients and HCPs still utilize the old nomenclature

Limb-girdle muscular dystrophy type 2I/R9: *Nomenclature*

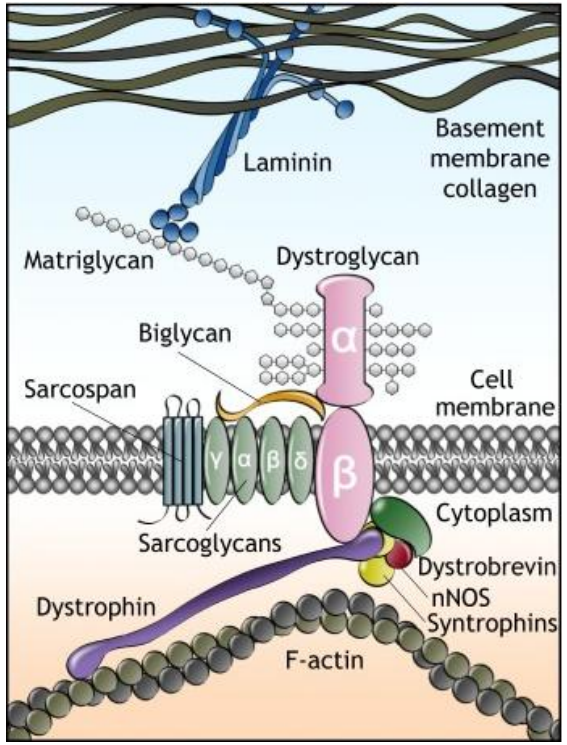
Old nomenclature	New nomenclature	Affected gene/protein
LGMD2I	LGMDR9 (FKRP-related)	Fukutin-related protein (FKRP)

Hybrid term (LGMD2I/R9) is used for clarity across audiences familiar with old and/or new nomenclature

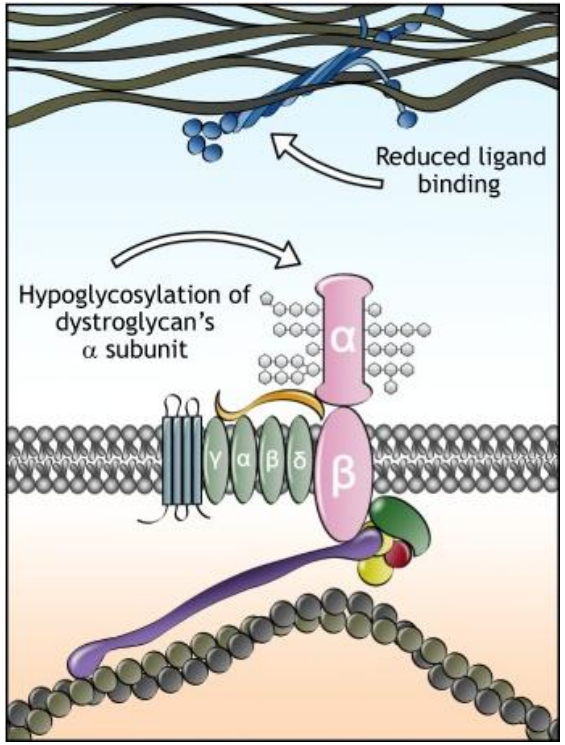
Straub V et al, Neuromuscul Disord, 2018

LGMD2I/R9 is caused by mutations in FKRP that result in reduced glycosylation of alpha-dystroglycan (αDG), leading to progressive muscle damage

Molecular pathogenesis of LGMD2I/R9



*In **healthy muscle cells**, glycosylated αDG bridges the muscle cell membrane to the extracellular matrix*



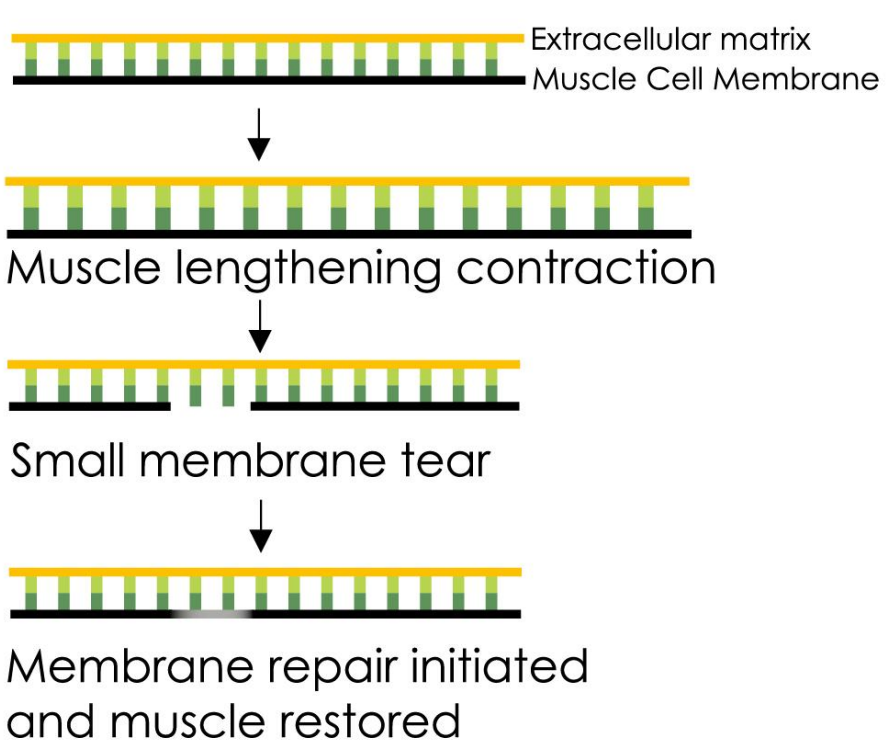
*In untreated **affected muscle cells**, hypoglycosylation of αDG results in loss of αDG-laminin binding*

- In healthy muscle cells, **alpha-dystroglycan (αDG) plays an important role** in linking the muscle cell membrane (sarcolemma) to the extracellular matrix
  - **Glycosylation of αDG is required** to form a glycopeptide chain that binds to laminin in the extracellular matrix
- LGMD2I/R9 is caused by **mutations in *FKRP*** that lead to **reduced function of the FKRP enzyme**, resulting in **decreased glycosylation of alpha-dystroglycan (αDG)**
- **Reduced glycosylation of αDG results in muscular dystrophy**, including the progressive muscle damage seen in LGMD2I/R9

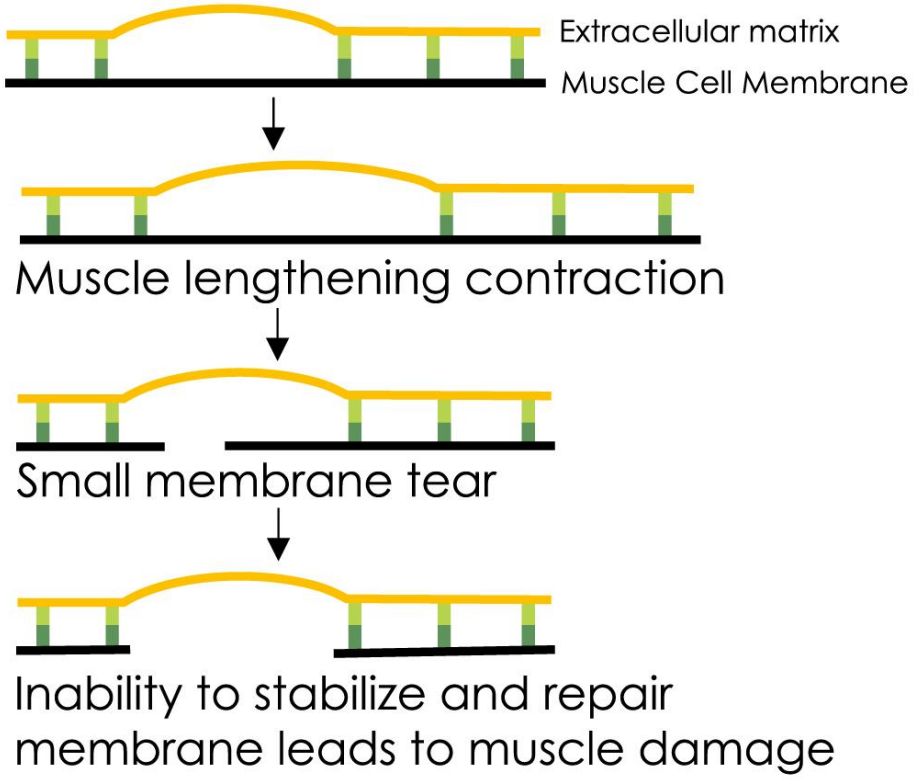
Nickolls AR, Bönnemann CG, Dis Model Mech, 2018; Kanagawa M et al, Cell, 2004

Without glycosylated αDG playing its role as a “shock absorber,” LGMD2I/R9 muscle tissue is susceptible to damage and eventually forms fibrotic tissue

Healthy muscle tissue



LGMD2I/R9 muscle tissue



Source: https://bridgebio.com/lgmd2i-r9/

LGMD2I/R9 has an established genotype/phenotype association; ~2/3 of patients are homozygous for the most common founder effect mutation (L276I)

	Birth	Early Childhood	Late Childhood	Adolescence to adulthood
L276I homozygous **Prevalence** *(L276I/L276I)* **68%**	Asymptomatic	Asymptomatic	▪ **Age of symptom onset 18 ± 3 years old** ▪ Lower limb & proximal weakness ▪ +/-calf hypertrophy, muscle pain, hyper-CKemia	**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, hyper-CKemia	▪ Lower limb & proximal weakness ▪ +/-calf hypertrophy, muscle pain, hyper-CKemia	**Loss of ambulation:** most by age 20 **Respiratory decline**: Invasive assistance required by 5% by age 30 **Cardiac dysfunction**: ~60%

Due to a founder effect mutation in Northern European populations, LGMD2I/R9 is among the most prevalent LGMDs in the U.S. and Europe

Sveen et al, Annals of Neurology, 2006; Richard et al, Neuromuscular Disorders, 2016; Gedlinske et al, Neurology, 2020; Libell et al, Muscle Nerve, 2020; Murphy et al, Ann Clin Transl Neurol, 2020; Liu W et al, Genet Med, 2019; Nallamilli et al, Ann Clin Transl Neurol, 2018

Diagnosis is confirmed through sponsored genetic testing, and SoC is limited to supportive care, making disease-modifying therapies the key unmet need

Diagnosis

Diagnosis of LGMD2I/R9 is confirmed by via genetic testing, which is available in the U.S. through **sponsored genetic testing panels** for muscular dystrophies

Standard of care

- There are **no approved therapies** for any form of LGMD today, including LGMD2I/R9
- SoC is **limited to supportive care**, including:
  - **Physical therapy** to prevent contractures and maintain mobility
  - **Cardiac monitoring** and medical management of cardiomyopathy
  - **Respiratory monitoring** and respiratory support
  - **Pain management** *(as needed)*
  - Monitoring for **spinal deformities**

Key unmet need **Disease-modifying therapies that slow or stop progressive decline experienced by LGMD2I/R9 patients**

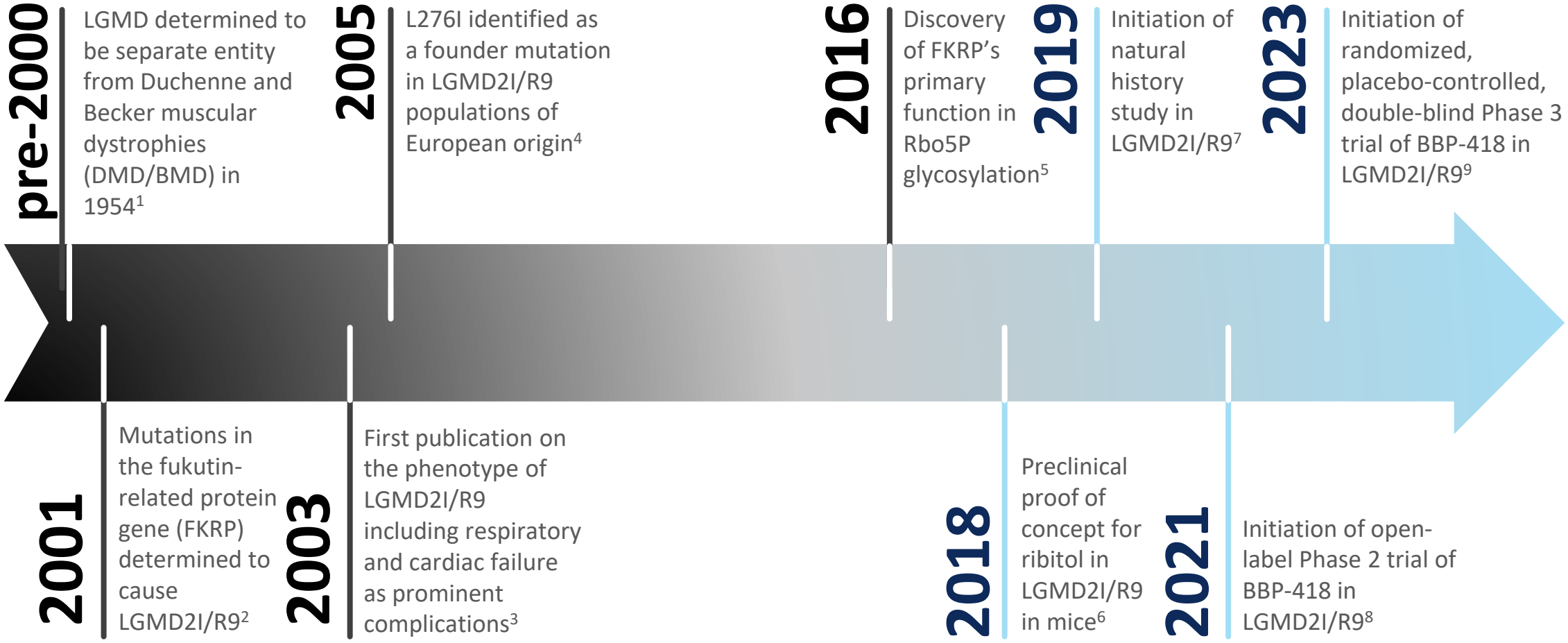
Summary: LGMD2I/R9 disease background

- LGMD2I/R9 (FKRP-related) is one of many **genetically distinct** subtypes of limb-girdle muscular dystrophy and causes progressive muscle weakness, including **loss of ambulation**, **cardiomyopathy**, and **respiratory dysfunction**

- The **foundational defect of LGMD2I/R9** is mutations in *FKRP* that reduce function of the FKRP enzyme, resulting in **decreased glycosylation of alpha-dystroglycan (αDG)**

- The standard of care for LGMD2I/R9 is **limited to supportive care** and **does not prevent the progressive decline** experienced by individuals living with LGMD2I/R9

The BBP-418 program builds on multiple decades of scientific progress



[1] Walton JN, Nattrass FJ, Brain, 1954; [2] Brockington M et al., Hum Mol Genet, 2001; [3] Poppe M et al., Neurology, 2003; [4] Frosk P et al., Hum Mutat, 2005; [5] Kanagawa M et al., Cell Rep., 2016; [6] Cataldi MP et al., Nat Commun, 2018; [7] https://clinicaltrials.gov/study/NCT04202627; [8] https://clinicaltrials.gov/study/NCT04800874; [9] https://clinicaltrials.gov/study/NCT05775848

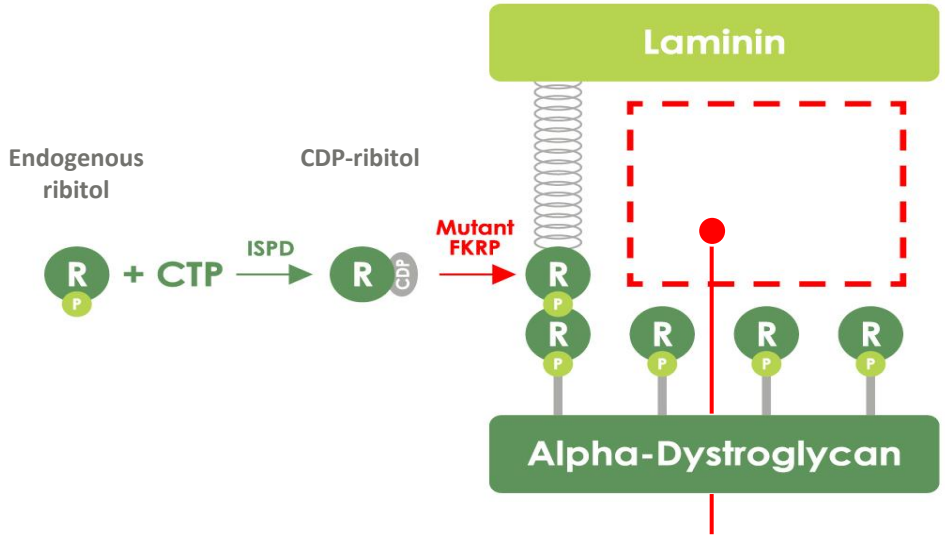
BBP-418 is an investigational therapy and has not been approved for use by any regulatory agency

BBP-418 is being investigated to target the disease at its source by driving residual activity of the affected FKRP enzyme and restoring glycosylation of αDG

Disease mechanism

FKRP glycosylates alpha-dystroglycan (αDG) which stabilizes muscle cells by binding extracellular ligands to act as a “shock absorber” for muscle fibers

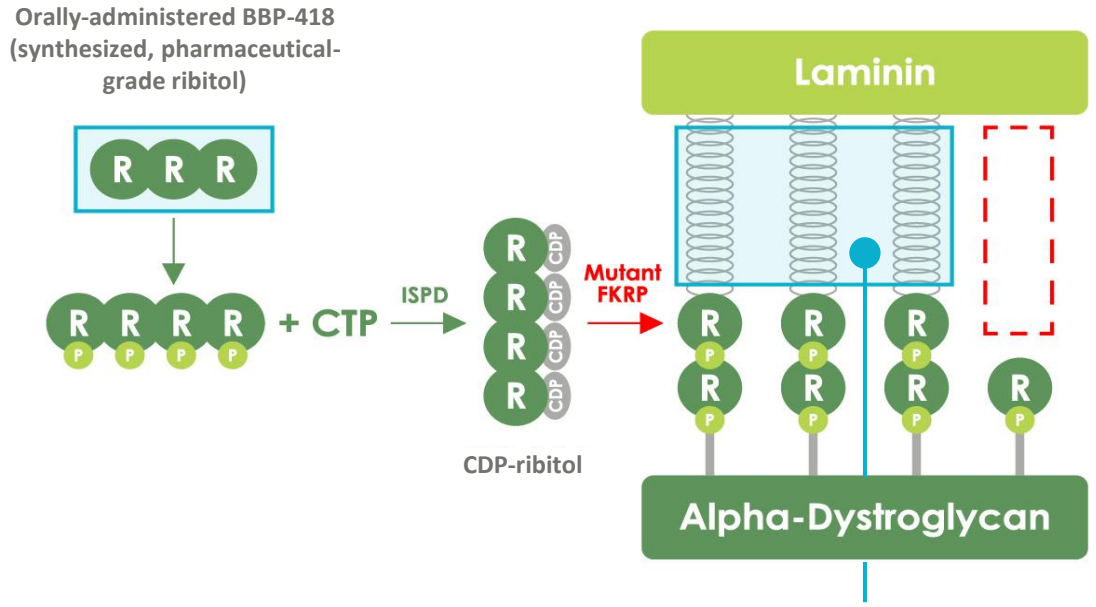
Partial loss of function of FKRP enzyme results in dysfunctional, hypo-glycosylated αDG in muscle cells which increases cell susceptibility to damage



Mutations in FKRP prevent addition of CDP-ribitol 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 synthesized, pharmaceutical-grade ribitol upstream, aiming to drive residual activity of mutant FKRP enzyme and increase αDG glycosylation levels

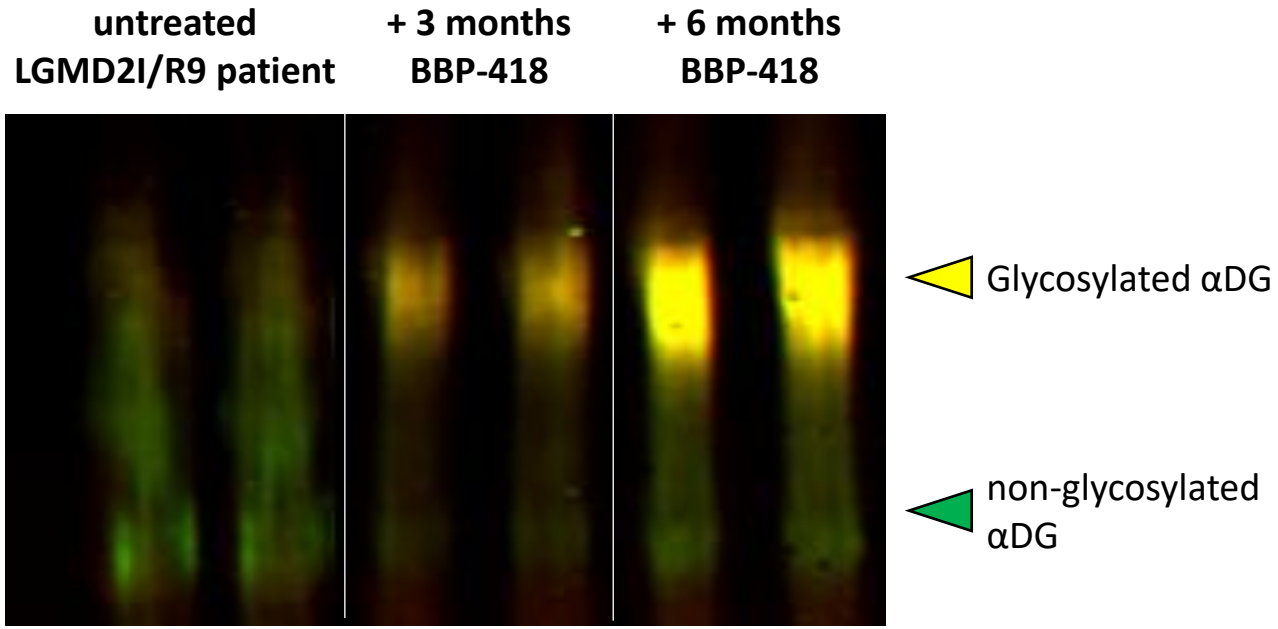


Potential partial restoration of αDG glycosylation

BBP-418 is an investigational therapy and has not been approved for use by any regulatory agency

We have developed a proprietary, validated Western Blot assay to accurately and reliably measure glycosylated αDG directly in skeletal muscle tissue

Validated Western Blot evaluates change from baseline in glycosylated alpha-dystroglycan (αDG)[1,2]



- Yellow signal corresponds to **functional, laminin-binding form of glycosylated αDG**
- Limited glycosylated αDG is seen in untreated LGMD2I/R9 patient muscle biopsy samples at baseline (shown in duplicate)
- **Increase in glycosylated αDG is observed** post treatment with BBP-418

FDA has indicated that our novel approach to measure glycosylated αDG by our validated assay appears reasonable

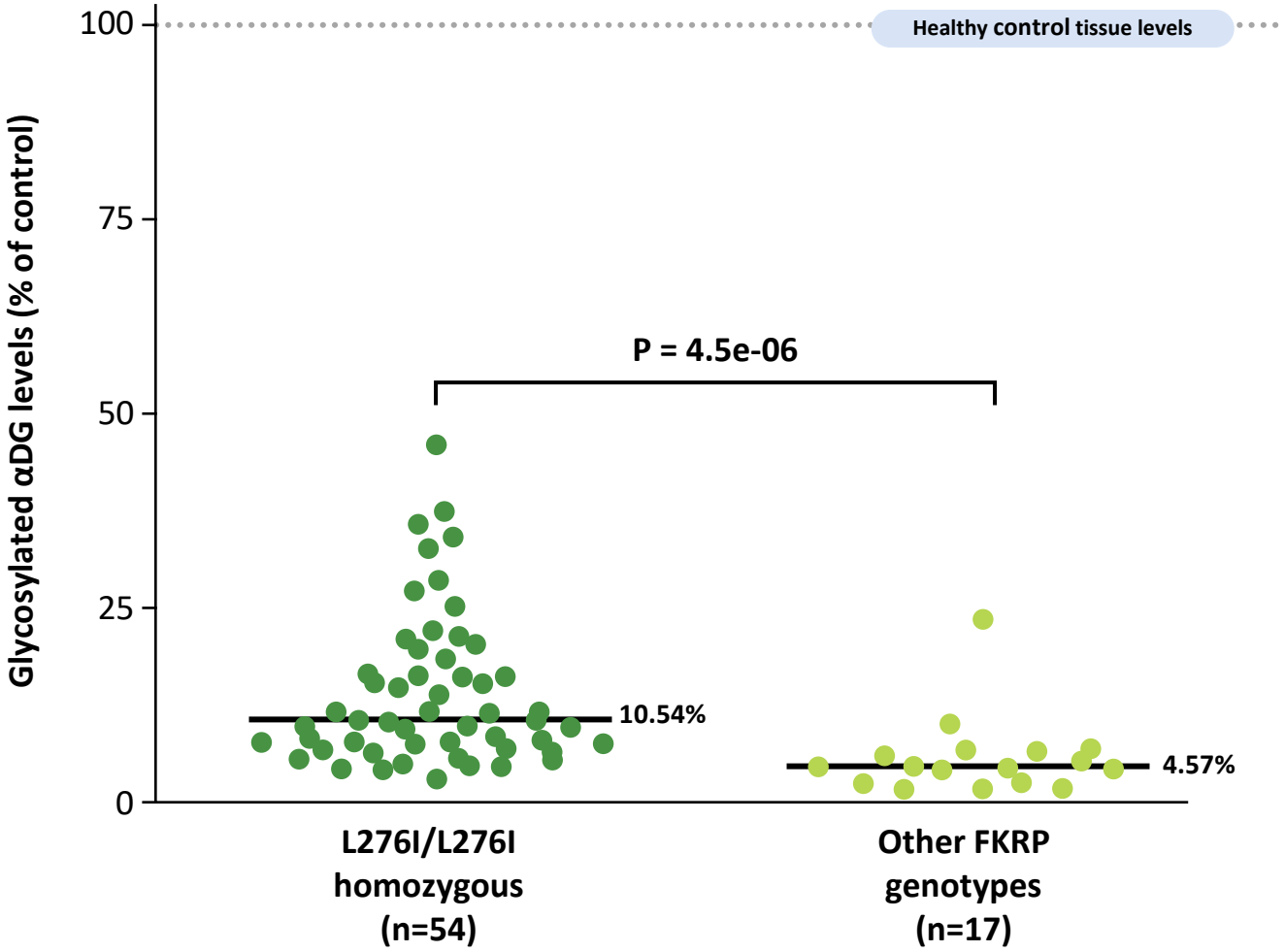
[1] Blots have been reordered to allow for side-by-side comparison of treatment effect within a patient; [2] See publication for details on this novel, multiplexed assay: Rajasingham et al, J Muscle Res Cell Motil, 2024

We have conducted several studies to characterize natural history of LGMD2I/R9 and evaluate safety of BBP-418; following an encouraging Ph. 2, we have fully enrolled a Ph. 3

Study	Phase	Description	Key takeaways
MLB-01-001	Natural history (N=96)	▪ Natural history study to characterize LGMD2I/R9 phenotypes ▪ Validate muscle biomarker (glycosylated αDG) to support BBP-418 development	▪ Defined disease trajectory over ~1 year ▪ Muscle biomarker reflects genotype/phenotype ▪ Longitudinal biopsies over a year demonstrate biomarker stability over time in untreated patients
MLB-01-002, -004, -006	Phase 1 (N=142)	▪ Three Phase 1 studies in healthy volunteers to evaluate safety and PK of BBP-418	▪ No serious adverse events (AEs) or discontinuations due to AEs related to BBP-418 in healthy volunteers ▪ PK of BBP-418 with and without food defined ▪ No QTc effects of BBP-418 seen
MLB-01-003	Phase 2 (N=14)	▪ Open label, dose-finding study to evaluate safety and tolerability of BBP-418 in LGMD2I/R9	▪ Encouraging safety profile in LGMD2I/R9 ▪ Biomarker data suggest BBP-418 is addressing disease at its source ▪ Encouraging evidence of clinical efficacy
MLB-01-005 (FORTIFY)	Phase 3 (N=112)	▪ Randomized, placebo-controlled study to evaluate efficacy and safety in LGMD2I/R9 ▪ Interim analysis planned at 12 mo. to support potential U.S. accelerated approval	▪ Fully enrolled as of Sept. 2024 ▪ Planned interim analysis topline data readout expected in 2H 2025

BBP-418 is an investigational therapy and has not been approved for use by any regulatory agency

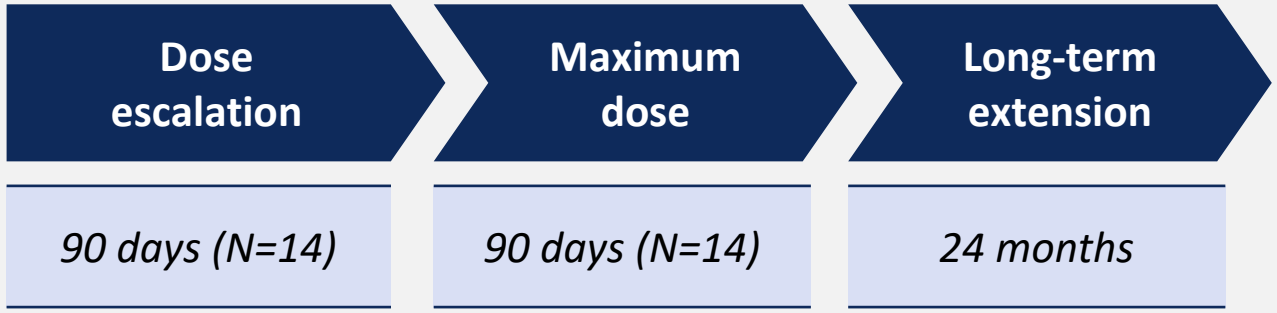
Natural history reflects that LGMD2I/R9 patients have reduced glycosylated αDG levels, and L276I homozygous patients have higher levels than other genotypes



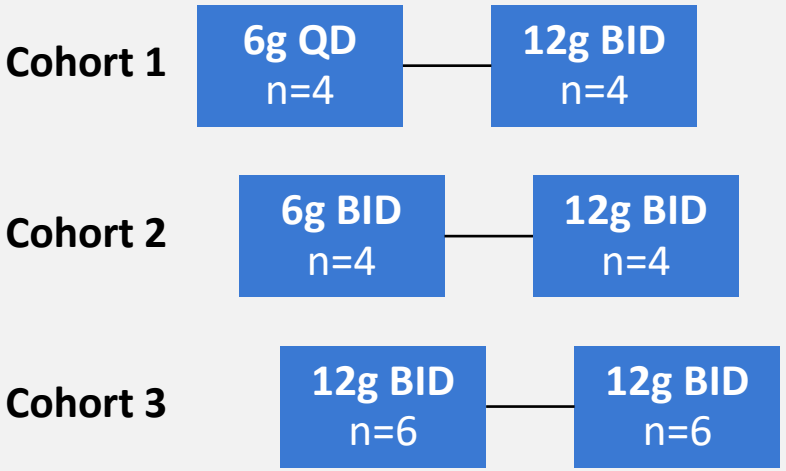
- Both L276I homozygous and other FKRP genotype patients have **reduced glycosylated αDG levels** compared with healthy individuals
- LGMD2I/R9 individuals with “other” (non-L276I homozygous) FKRP genotypes, which typically have a **more severe clinical presentation**, have **lower glycosylated αDG levels relative to L276I homozygous patients**

MLB 01-001 Figure 14.2.2.1

BBP-418 is being investigated in an open-label, dose-finding Ph. 2 study that has continued in a long-term extension



After dose escalation, all patients transitioned to highest dose 12g BID



Key Endpoints

- NSAD[1]
- Ambulatory measures
  - 10-meter walk test
  - 100-meter timed test
- Pulmonary function: FVC[2]
- Upper limb function: PUL 2.0[3]
- Biomarkers
  - Glycosylated αDG levels
  - Serum creatine kinase

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)

[1] NSAD = North Star Assessment for Dysferlinopathy, also known as the North Star Assessment for Limb-Girdle Type Muscular Dystrophies; [2] FVC = Forced vital capacity; [3] PUL2.0 = Performance of Upper Limb 2.0
BBP-418 is an investigational therapy and has not been approved for use by any regulatory agency

BBP-418 continues to be well tolerated, with only minor GI related adverse events recorded in the Ph. 2 study

- Most of the reported TEAEs in the Ph. 2 were Grade 1 (mild) or Grade 2 (moderate) in severity
- No discontinuations or interruptions in therapy due to AEs were observed

Well-tolerated therapy may provide foundation for favorable risk-benefit ratio

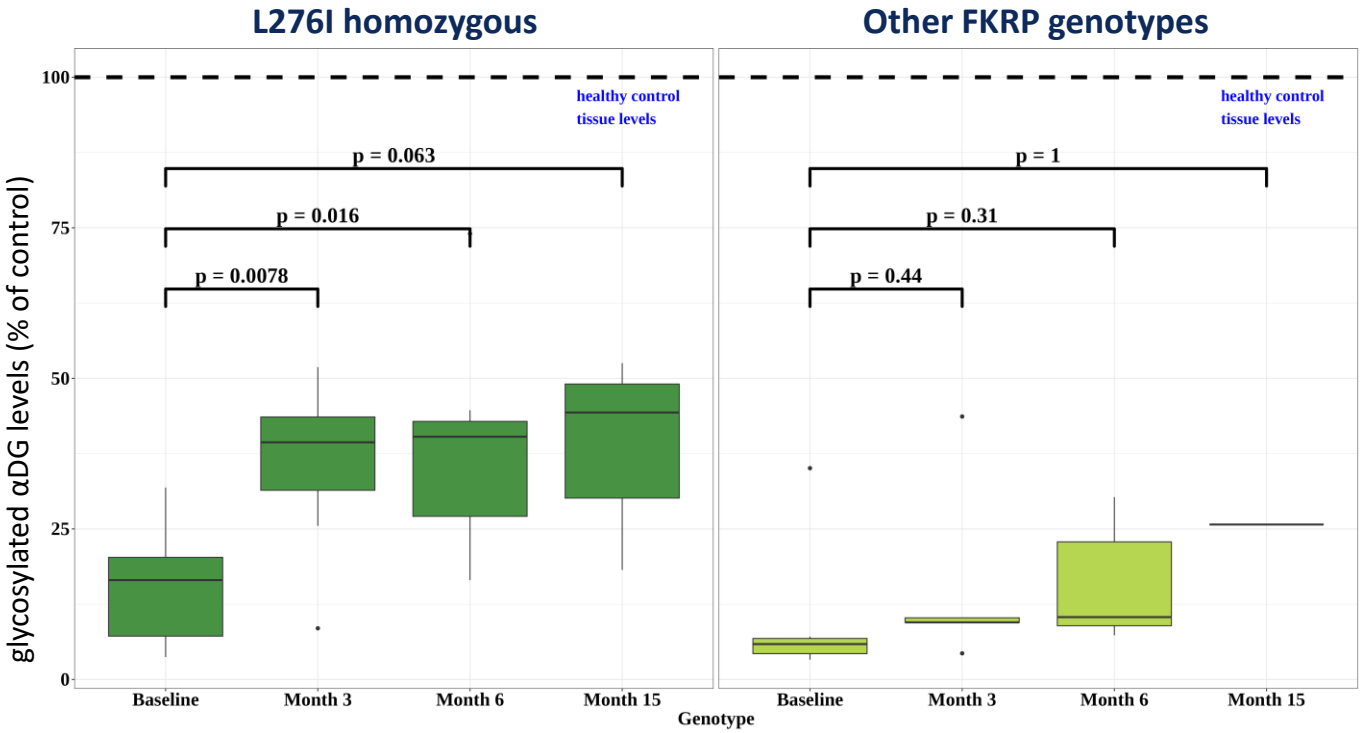
Treatment-related TEAE	# of incidents	# of patients (%)
Diarrhea	8	6 (43%)
Dehydration	1	1 (7%)
Nausea	3	2 (14%)
Vomiting	2	2 (14%)
Dyspepsia	1	1 (7%)
Gastroenteritis	1	1 (7%)
Bloating	2	2 (14%)
Headaches	1	1 (7%)
Abdominal pain	1	1 (7%)
Overall	**20**	**9 (64%)**

MLB-01-003 Listing 14.3.1.3 Part 3 month 15

BBP-418 is an investigational therapy and has not been approved for use by any regulatory agency

Sustained increases in levels of glycosylated αDG in muscle and decreases in serum creatine kinase were observed in Ph. 2 study of BBP-418

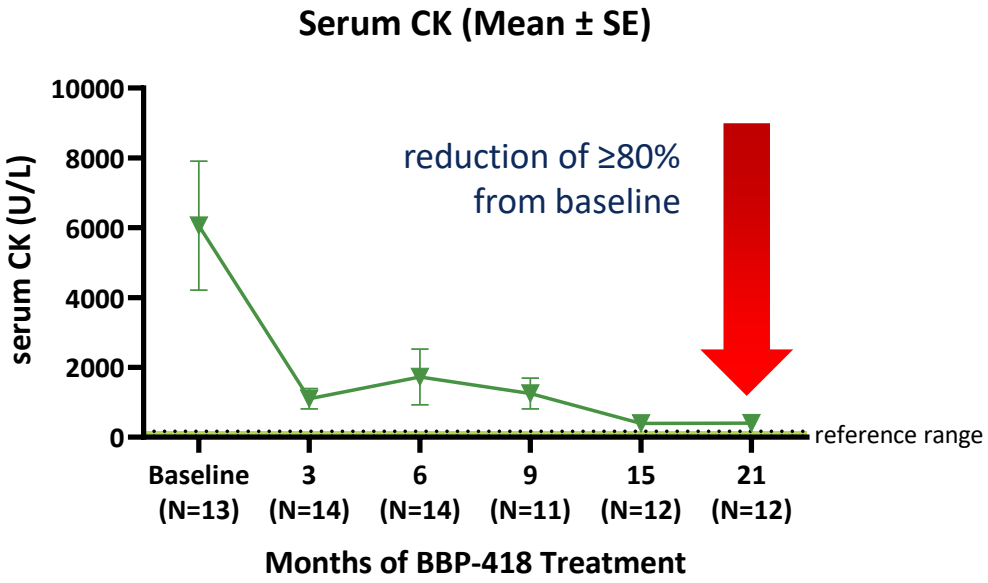
Increase in glycosylated αDG in muscle observed post dosing with BBP-418 (median ± 95% CI)



	L276I homozygous				Other FKRP genotypes			
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, + 15 mo = Part 3, Month 9
Median and 10–90% percentile are shown, Wilcoxon test was used to determine significance
MLB-01-003 Listing 16.4.1 and 16.1.4.2

Reduction in mean serum CK observed post dosing with BBP-418

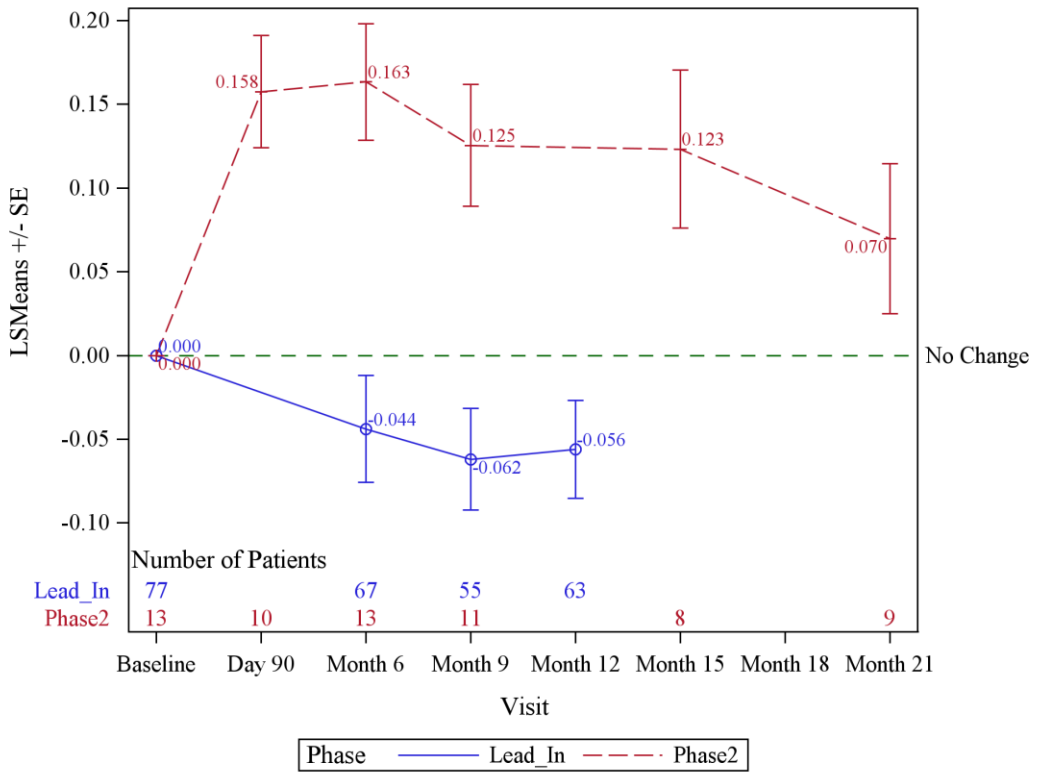


Cohort 1 Day 1 CK draws taken after functional assessments; all other draws done prior to functional assessment
After Day 90, all subjects received 12 g BID (weight-adjusted)
+ 3 mo = Part 1, 90-day; +6 mo = Part 2, Month 3; + 9 mo = Part 3, Month 3; + 15 mo = Part 3, Month 9; +21 mo = Part 3, Month 15; Figure shows reference range from 30–170 units/L
MLB-01-003 Table 14.2.1.1

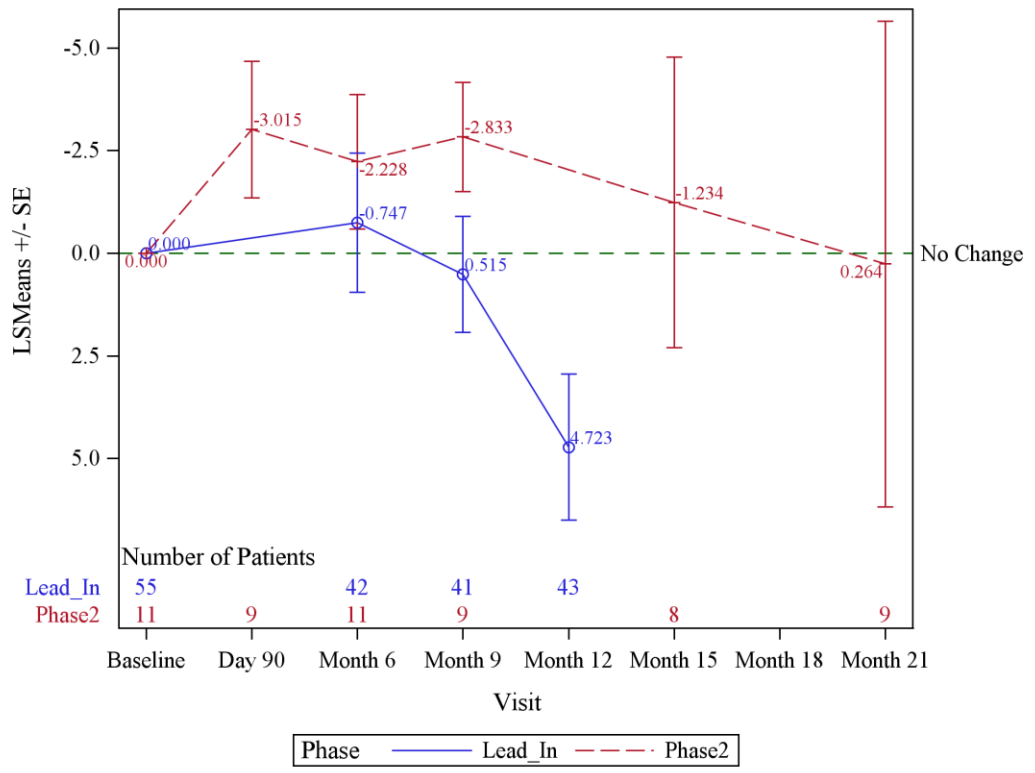
BBP-418 is an investigational therapy and has not been approved for use by any regulatory agency

Stabilization in ambulatory measures has also been observed after treatment with BBP-418 in Ph. 2 study, suggesting change in trajectory from natural history

Change from baseline in 10-meter walk test (m/s)



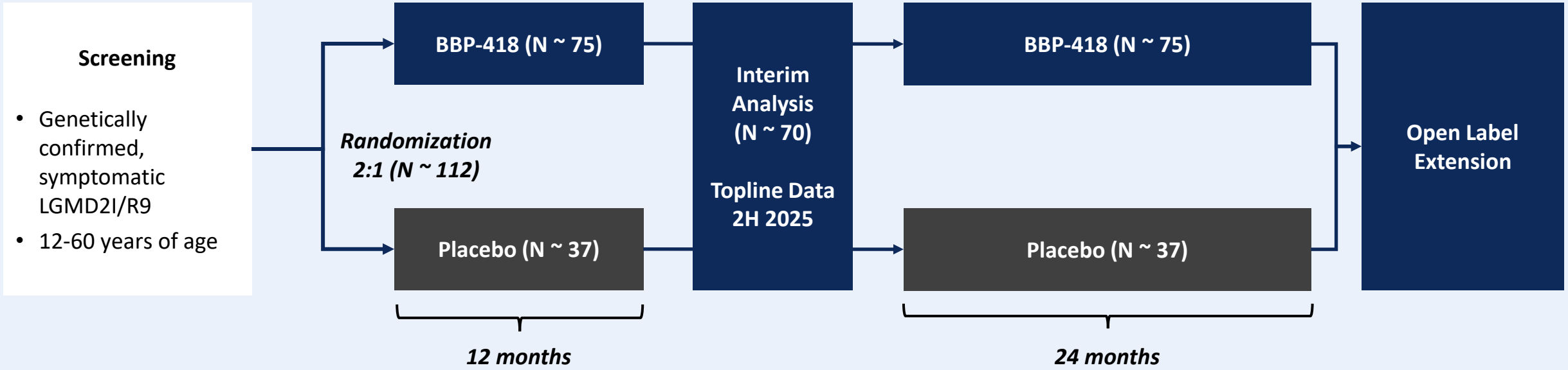
Change from baseline in 100-meter timed test (s)



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

Source: 10MWT: MLB-01-001 Listing 16.2.1 and MLB-01-003 Listing 16.2.1; 100MTT: MLB-01-001 Listing 16.2.2 and MLB-01-003 Listing 16.2.2
Data exclude 1 subject from month 15 timepoint due to post-COVID decline
BBP-418 is an investigational therapy and has not been approved for use by any regulatory agency.

We have fully enrolled a Phase 3 study (FORTIFY) of BBP-418 in LGMD2I/R9 and expect topline interim analysis data readout in 2H 2025



Interim Endpoints:

- Glycosylated αDG (*primary*)
- Serum creatine kinase (CK)
- Trends in clinical measures

Final Analysis Endpoints:

- NSAD (*primary*)
- Ambulatory measures
  - 10MWT
  - 100MTT
- Pulmonary function: FVC
- Upper limb function: PUL 2.0
- QoL measures

Phase 3 Trial Fully Enrolled; Topline Results Expected 2H 2025

Note: Ph. 3 trial population limited to 18-60 years in EU region; final analysis exploratory endpoints include cardiac endpoints such as HS Troponin I
BBP-418 is an investigational therapy and has not been approved for use by any regulatory agency.

We have received positive feedback from FDA on potential for accelerated approval in the U.S. based on the surrogate endpoint of glycosylated αDG

FDA may grant accelerated approval based on a surrogate endpoint…

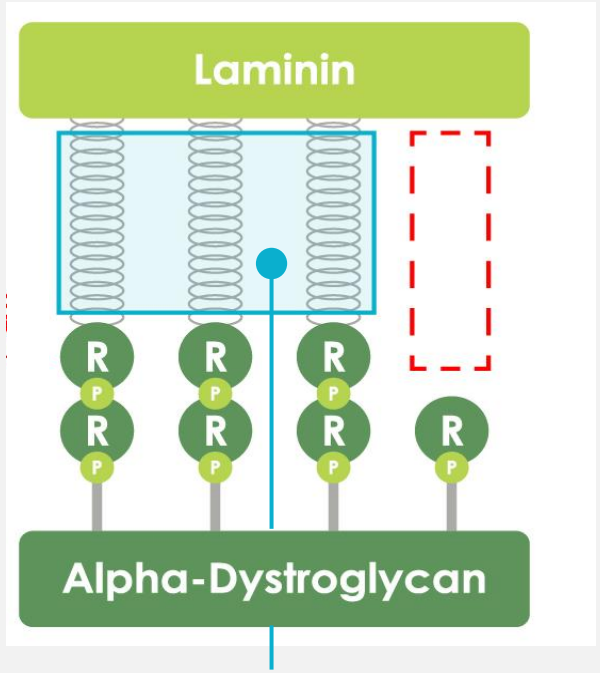
…such as glycosylated αDG in LGMD2I/R9

Definition

"A surrogate endpoint, or 'marker,' is a laboratory measurement or physical sign that is used in therapeutic trials as a substitute for a clinically meaningful endpoint that is a direct measure of how a patient feels, functions, or survives and is expected to predict the effect of the therapy."[1]

Key criteria

01	Biological Plausibility
02	Reliable Measurement
03	Association with Clinical Outcomes



Potential surrogate endpoint in LGMD2I/R9: glycosylated alpha-dystroglycan (αDG)

[1] New drug, antibiotic and biological drug product regulations: accelerated approval. Proposed Rule. 57 Federal Register 13234-13242 (1992)

Summary: BBP-418 program overview

- BBP-418 aims to **target LGMD2I/R9 at its source** by **restoring glycosylation of alpha-dystroglycan (αDG)**, which we measure by a **proprietary, validated assay**

- In a small, open-label Ph. 2 study, BBP-418 was **well tolerated**, and **sustained improvements on glycosylated αDG and serum CK** were observed in addition to **trends toward stabilization** on ambulatory measures

- Consistent with positive feedback from FDA, our fully-enrolled Ph. 3 study (FORTIFY) is designed with a **planned interim analysis at 12 months** focused on assessing **glycosylated αDG as a surrogate endpoint** to support a **potential accelerated approval** in the U.S.

BBP-418 is an investigational therapy and has not been approved for use by any regulatory agency.

Continuing momentum from successful regulatory interactions and rapid Ph. 3 enrollment, we anticipate topline data from the Ph. 3 interim analysis in 2H

Multiple encouraging discussions with the FDA support plan to pursue accelerated approval in U.S. for BBP-418 based on a surrogate endpoint
2023 – 2024

Manuscript describing novel, validated muscle tissue-based bioassay to detect proposed surrogate endpoint, glycosylated αDG levels, published
April 2024

Phase 3 FORTIFY study of BBP-418 in LGMD2I/R9 interim analysis enrollment target surpassed
June 2024

Completion of full enrollment of Phase 3 FORTIFY study of BBP-418 in LGMD2I/R9
Sept. 2024

Topline data from Phase 3 FORTIFY study interim analysis
2H 2025

The primary endpoint for the interim analysis is glycosylated αDG at 3 mo. with additional biomarkers and clinical measures as secondary endpoints at 12 mo.

fortify

Ph. 3 FORTIFY interim analysis

Type		Endpoint	Timepoint	N	Base case target	Upside target
Efficacy	**Primary**	Glycosylated αDG *(Powered at >99%)*	3 mo.	N~112	• Statistically significant increase vs. placebo • Absolute increase ≥5% CFB in BBP-418 treated	• Statistically significant increase vs. placebo • 1.5x CFB in BBP-418 treated vs. approx. no change in placebo
	Key secondary	Creatine kinase (CK)	12 mo.	N~70	• Average decline of ≥40% CFB in BBP-418 treated	• Average decline of ≥50% CFB in BBP-418 treated
		• Ambulatory measures • Pulmonary (FVC)	12 mo.	N~70	• Trends in one or more measures favoring BBP-418 treated vs. placebo • Statistical significance on clinical outcomes is not expected or required for accelerated approval	
Safety			12 mo.		• Well-tolerated *(consistent with Ph. 2 results)*	

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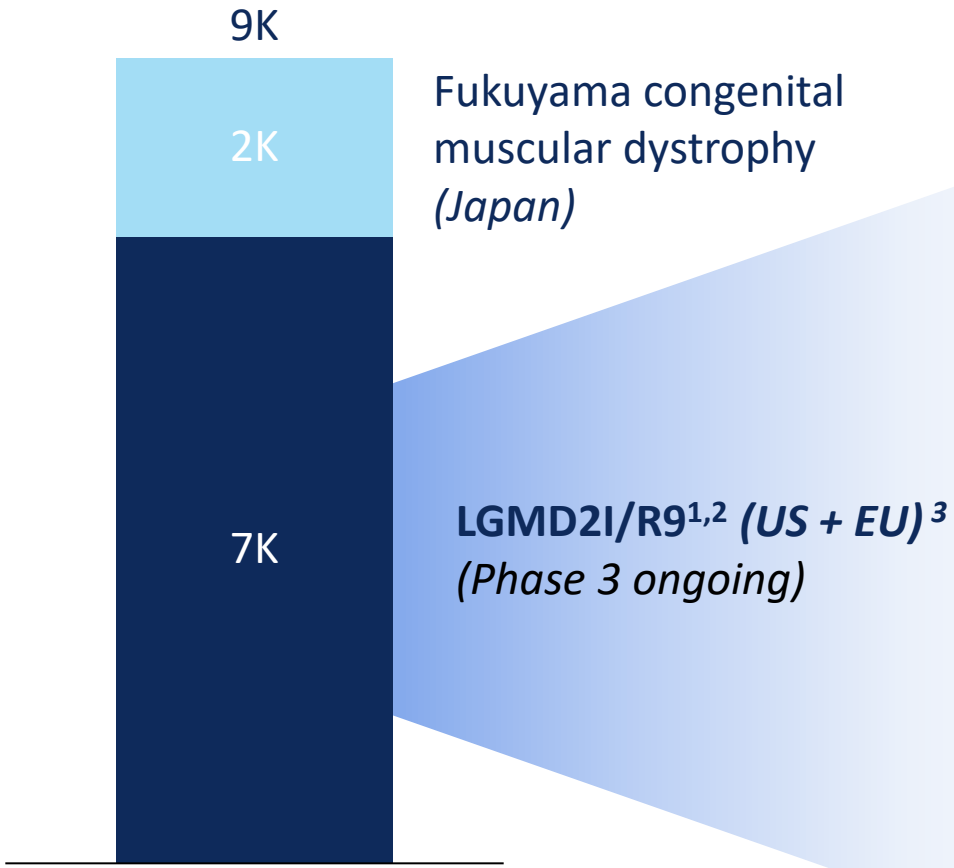
LGMD2I/R9 represents a $1B+ market opportunity across the U.S. and EU

Addressable patients by indication



LGMD2I/R9 market opportunity

First-to-market to address high unmet need
Opportunity to treat debilitating progressive neuromuscular disease with no approved therapies

Disease-modifying oral therapy
Attractive safety and convenience for patients while targeting the disease at its source

Targeting broad label for LGMD2I/R9
Ph. 3 design provides foundation for indication statement with limited exclusion factors

Market opportunity $1B+

[1] Liu W et al, Genet Med, 2019; [2] Includes all patients with potentially treatable mutations in FKRP, FKTN, and ISPD; [3] Includes UK
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Based on prevalence of pathogenic variants in public sequencing databases and U.S. demographics, the prevalence of LGMD2I/R9 is ~6-7 per million in the U.S.

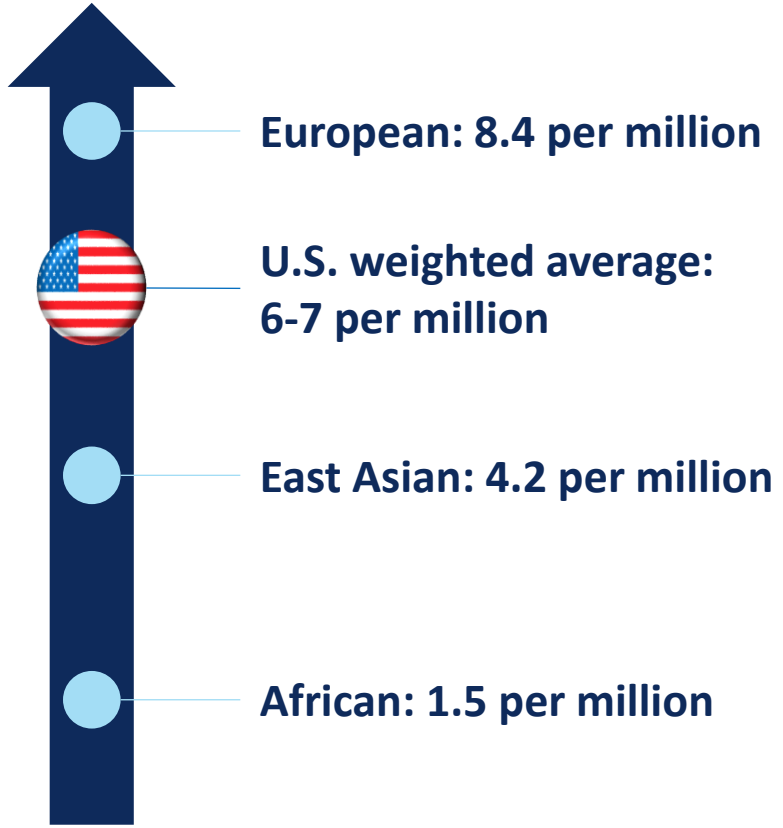
Summary of LGMD2I/R9 prevalence rate, by ancestry

Estimating prevalence for limb-girdle muscular dystrophy based on public sequencing databases

Wei Liu, BSc[1], Sander Pajusalu, MD, PhD[2,3,4], Nicole J. Lake, MSc, PhD[2,5], Geyu Zhou, BSc[1], Nilah Ioannidis, MPhil, PhD[6,7], Plavi Mittal, PhD[6,8], Nicholas E. Johnson, Msc, MD[9], Conrad C. Weihl, MD, PhD[10], Bradley A. Williams, PhD[6], Douglas E. Albrecht, PhD[6], Laura E. Rufibach, PhD[6] and Monkol Lek, BE, PhD[2]

Population	LGMD2I/R9 prevalence per million
African/African American	1.5
Ashkenazi Jewish	0.03
East Asian	4.2
European	8.4

Est. U.S. prevalence rate



Note: Prevalence rates exclude potentially addressable LGMDs caused by mutations in ISPD and FKTN
Source: Liu W et al, Genet Med, 2019

Natural history studies and highly engaged patient advocacy organizations accelerated Ph. 3 enrollment and provide support for patient identification

Natural history studies[1]

U.S.

University of Iowa

Clinical Trial Readiness for Dystroglycanopathies[2]
(N>100)

MLB-01-001[3]
(N=96)

Europe

University Hospital of North Norway

LGMD2I/R9 in Norway[4]
(N=106)

Genethon

NatHx of LGMD2I/R9[5]
(N=52; ambulatory patients only)

Patient advocacy organizations[1]

(N=850; includes >200 in U.S.)[6]

Highly powered Ph. 3 FORTIFY study (N=112) enrolled more quickly than expected

[1] Not exhaustive; [2] https://clinicaltrials.gov/study/NCT00313677; [3] https://clinicaltrials.gov/study/NCT04202627; [4] https://clinicaltrials.gov/study/NCT03930628; [5] https://clinicaltrials.gov/study/NCT03842878; [6] As of May 2025

Several tailwinds in the LGMD landscape may further accelerate market development in advance of a potential U.S. launch of BBP-418 in LGMD2I/R9

Sponsored genetic testing programs

- Established complimentary testing programs in U.S.

Approved ICD-10 code

- ICD-10 code specific to LGMD2I/R9 approved, going into effect Oct. 1, 2025

Potential approvals in other LGMDs to drive diagnosis

- Approval for gene therapy in LGMD2E/R4 possible as early as 1H 2026

BBP-418 has **first-in-class** market opportunity in LGMD2I/R9 while benefiting from **tailwinds** in broader LGMD market landscape supporting **patient identification**

Q&A