

BBP-418 Demonstrates Potential to Be Disease Modifying Therapy, Restoring Cardiac and Disease Markers to Unaffected Levels

2026-10-05

- In exploratory analyses at Month 12, among individuals with LGMD2I/R9 (FKRP-related) with elevated HS troponin I at baseline, 100% treated with BBP-418 returned to the normal range by Month 12 versus 40% in the placebo arm ($p=0.0248$); HS troponin I, a blood marker of heart muscle injury, declined more with BBP-418 than placebo (LS mean difference -17.8 ng/L; 95% CI: -35.1 to -0.5; $p=0.0443$)

- 54% of BBP-418-treated individuals had stable or improved LVEF at Month 12 versus 25% in the placebo arm ($p=0.0262$)

- In data presented separately, BBP-418 treatment of LGMD2I/R9 in FORTIFY increased mean glycosylated α DG by Month 3 to the levels of asymptomatic heterozygous FKRP carriers, who do not exhibit disease; this result was sustained through Month 12

- Taken together, these findings support the potential of BBP-418 to act as a disease modifying therapy by addressing the condition at its source

- BBP-418 is under FDA Priority Review with a PDUFA target action date of November 27, 2026

PALO ALTO, Calif., Oct. 05, 2026 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (Nasdaq: BBIO) ("BridgeBio" or the "Company"), a biopharmaceutical company focused on developing medicines for genetic conditions, today presented new exploratory cardiac data from the 12-month interim analysis of FORTIFY, the Phase 3 clinical trial of oral BBP-418 in individuals living with limb-girdle muscular dystrophy type 2I/R9 (LGMD2I/R9) (FKRP-related). The data were presented in an oral presentation at the 31st annual Congress of the World Muscle Society in Hiroshima,

Japan by Volker Straub, M.D., Ph.D. of Newcastle University, UK.

"Cardiac involvement is a major driver of morbidity and mortality in LGMD2I/R9, which is why it remains one of the greatest concerns for people living with the condition, families and treating physicians. Historically, we have been able to monitor the heart and manage complications, but we have not been able to address the underlying cause of the disease," said Dr. Straub. "What stands out in this analysis is how consistent it is: high-sensitivity troponin I fell and, in those who entered the study with elevated levels, it normalized; ejection fraction stabilized; and this happened alongside walking farther and breathing better. It's encouraging to see that the therapeutic approach seems to work across the whole muscular system, including the heart."

Cardiomyopathy affects approximately 30% of individuals with LGMD2I/R9 and approximately 60% of those with other FKRPs, and is progressive, with an annual loss of approximately 0.4% of left ventricular ejection fraction (LVEF). In FORTIFY, 22.9% of participants (25/109) had elevated high-sensitivity (HS) troponin I at baseline and 17.1% (18/105) had an LVEF below 50%. Exploratory cardiac findings at Month 12 showed:

- HS troponin I is a blood test used to detect injury to heart muscle. 100% of the participants treated with BBP-418 returned to the normal range for HS troponin I by Month 12 versus 40% in the placebo arm ($p=0.0248$); HS troponin I declined more with BBP-418 than placebo (LS mean difference -17.8 ng/L; 95% CI: -35.1 to -0.5 ; $p=0.0443$)
- At Month 12, 54% of participants treated with BBP-418 had stable or improved LVEF, a measurement of how much blood the heart's main chamber pushes out with each beat, compared with 25% of those on placebo ($p=0.0262$)
- Both HS troponin I and LVEF will continue to be monitored in the ongoing FORTIFY study

A consistent pattern across the FORTIFY interim analysis is the proportion of treated individuals reaching normal or near-normal levels on markers of the disease e.g., 38% of patients whose CK reached normal levels. In addition to the HS troponin I finding, in which 100% of evaluable BBP-418-treated participants with elevated levels at baseline returned to the normal range by Month 12, separate data presented at WMS 2026 by Anne Katrine Kjemsø Jensen, a medical student in the laboratory of John Vissing M.D., D.M.Sci., Copenhagen Neuromuscular Center of Rigshospitalet, DK, quantified α DG glycosylation in 34 asymptomatic heterozygous FKRPs. Results showed:

- Asymptomatic carriers showed significantly reduced glycosylation versus healthy controls, thereby establishing a reference point for the level associated with normal muscle function (34% of control)
- In FORTIFY, BBP-418 treatment increased mean glycosylated α DG above carrier levels by Month 12, suggesting that BBP-418 can restore glycosylated α DG to asymptomatic levels
- While many therapies in progressive neuromuscular disease aim to slow decline, BBP-418 was developed to target LGMD2I/R9 at its source, and the pattern of biomarker normalization observed to date, alongside

functional improvement from baseline rather than stabilization, is consistent with the potential for BBP-418 to be a disease modifying therapy

In the Phase 3 FORTIFY trial, BBP-418 met all primary and secondary endpoints at the pre-specified 12-month interim analysis, showing treated individuals improving while placebo recipients declined across every key measure. These results were presented as a late-breaking oral presentation at the 2026 MDA Clinical and Scientific Conference, which are available [here](#).

BridgeBio believes BBP-418 is positioned to become the first and only approved therapy for individuals living with LGMD2I/R9, addressing a significant unmet need in this disease and potentially representing the first approval of a therapy for any form of LGMD. Approximately 7,000 individuals currently live with LGMD2I/R9 and other addressable α -dystroglycanopathies in the U.S. and Europe. BBP-418 is under FDA Priority Review with a PDUFA target action date of November 27, 2026. The Company is also engaging regulatory agencies to identify an expedited path to approval for BBP-418 in Europe.

BBP-418 has previously received Orphan Drug, Fast Track, and Rare Pediatric Disease Designations from the FDA and Orphan Drug Designation from the European Medicines Agency (EMA). BBP-418 received Priority Review from the FDA, highlighting the potential for BBP-418 to address unmet need in LGMD2I/R9. Consistent with Rare Pediatric Designation from the FDA, if BBP-418 is approved, BridgeBio may qualify for a Priority Review Voucher. The Company intends to initiate clinical studies of BBP-418 in LGMD2I/R9 for individuals less than 12 years of age in the first half of 2027 and in LGMD2M/R13 and LGMD2U/R20 in the near future.

About Limb-Girdle Muscular Dystrophy Type 2I/R9 (LGMD2I/R9)

LGMD2I/R9 is a monogenic autosomal recessive disease caused by partial loss of function mutations in the fukutin-related protein (FKRP) gene, and FKRP mutations impair glycosylation of alpha-dystroglycan (α DG), a protein associated with stabilizing muscle cells. Clinical manifestations typically present as a skeletal myopathy affecting the lower and then upper limbs, which is commonly later accompanied by pulmonary muscle and cardiac muscle involvement. Individuals who harbor a homozygous L276I genotype typically develop disease manifestations during late childhood with progression to loss of independent ambulation (25%), assisted ventilation (10%), and cardiomyopathy (30%) in adulthood. Cardiomyopathy is progressive, with an annual loss of 0.4% of left ventricular ejection fraction (LVEF). Individuals with other FKRP genotypes typically have an earlier childhood onset with a more severe clinical course, rapid loss of mobility by 20 years of age, more frequent cardiac involvement (60%), and eventual pulmonary decline by 30 years of age in nearly all cases.

About BBP-418

BBP-418 is an investigational oral glycosylation substrate therapy with potential to be the first and only therapy for LGMD2I/R9. BBP-418 is designed to saturate the partially functional FKRP enzyme with substrate thereby enhancing

residual FKRP function and restoring glycosylation of αDG. Through restoration of αDG glycosylation, BBP-418 may stabilize or improve muscle function, including gross motor, ambulatory, and cardiopulmonary function.

About BridgeBio

BridgeBio exists to develop transformative medicines for genetic conditions. Millions of people worldwide living with genetic conditions lack treatment options, often because drug development for small patient populations can be commercially challenging. We aim to bridge the gap between advancements in genetic science and meaningful medicines for underserved patient populations. Our decentralized, hub-and-spoke model is designed for speed, precision, and scalability. Autonomous and empowered teams focus on individual conditions, while a central hub provides the clinical, regulatory, and commercial capabilities needed to bring innovation to market. For more information, visit bridgebio.com and follow us on [LinkedIn](#), [X](#), [Facebook](#), [Instagram](#), [YouTube](#), and [TikTok](#).

BridgeBio Forward-Looking Statements

This press release contains forward-looking statements. Statements in this press release may include statements that are not historical facts and are considered forward-looking within the meaning of Section 27A of the Securities Act of 1933, as amended (the Securities Act), and Section 21E of the Securities Exchange Act of 1934, as amended (the Exchange Act), which are usually identified by the use of words such as “anticipates,” “believes,” “continues,” “estimates,” “expects,” “hopes,” “intends,” “may,” “plans,” “projects,” “remains,” “seeks,” “should,” “will,” and variations of such words or similar expressions. BridgeBio intends these forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act and Section 21E of the Exchange Act. These forward-looking statements include statements regarding the potential clinical significance and therapeutic implications of data regarding BBP-418, including the potential for BBP-418 to act as a disease-modifying therapy for LGMD2I/R9, restore or normalize disease biomarkers, and stabilize or improve cardiac, gross motor, ambulatory and pulmonary function; the potential for BBP-418 to become the first and only approved therapy for individuals living with LGMD2I/R9 and potentially the first approved therapy for any form of LGMD; the timing and likelihood of potential regulatory approval and commercialization of BBP-418, including the PDUFA target action date of November 27, 2026; BridgeBio’s interactions with regulatory authorities regarding a potential expedited path to approval for BBP-418 in Europe; BridgeBio’s potential eligibility to receive a Priority Review Voucher if BBP-418 is approved; the continued evaluation of cardiac and other clinical measures in the ongoing FORTIFY study; and BridgeBio’s plans to initiate additional clinical studies of BBP-418, including in individuals with LGMD2I/R9 less than 12 years of age and in individuals with LGMD2M/R13 and LGMD2U/R20. Although the Company believes that its plans, intentions, expectations and strategies as reflected in or suggested by those forward-looking statements are reasonable, the Company can give no assurance that the plans, intentions, expectations or strategies will be attained or achieved. Furthermore, actual results may differ materially from those described in the forward-looking statements and will be affected by a number of risks, uncertainties and assumptions, including, but not limited to, initial and ongoing data from the Company’s preclinical studies and

clinical trials not being indicative of final data; the exploratory and non-alpha-controlled nature of certain analyses described in this press release and the small sample sizes on which certain of those analyses are based; that observed biomarker changes, including normalization of HS troponin I, glycosylated αDG or other disease markers, may not be replicated in additional analyses or studies or predict or translate into long-term clinical benefit, sustained improvement in cardiac or other muscle function, or prevention of disease progression; that BBP-418 may not demonstrate the anticipated efficacy, safety, therapeutic benefit or disease-modifying potential in ongoing or future clinical studies; that the design, enrollment, conduct, timing and success of ongoing and planned clinical trials may differ from current expectations; that the FDA or other regulatory authorities may not approve BBP-418 on the anticipated timeline or at all, may not agree with BridgeBio's regulatory approval strategies, clinical trial designs, conduct or methodologies, or the sufficiency of data submitted, or may require additional data, studies or other information; that BBP-418 may not become the first and only approved therapy for LGMD2I/R9 or the first approved therapy for any form of LGMD; that BridgeBio may not identify or obtain an expedited path to approval for BBP-418 in Europe; that BridgeBio may not qualify for or receive a Priority Review Voucher; that plans to initiate additional clinical studies, including studies in individuals less than 12 years of age or in other forms of LGMD, may be delayed, modified or may not occur as expected; the potential size of the target patient populations BBP-418 is designed to treat not being as large as anticipated; the impacts of current macroeconomic and geopolitical events, including changing conditions from hostilities in Ukraine and the Middle East, increasing rates of inflation and changing interest rates, on business operations and expectations, as well as those risks set forth in the Risk Factors section of the Company's most recent Quarterly Report on Form 10-Q and Annual Report on Form 10-K and the Company's other filings with the U.S. Securities and Exchange Commission. Moreover, the Company operates in a very competitive and rapidly changing environment in which new risks emerge from time to time. These forward-looking statements are based upon the current expectations and beliefs of the Company's management as of the date of this press release, and are subject to certain risks and uncertainties that could cause actual results to differ materially from those described in the forward-looking statements. Except as required by applicable law, BridgeBio assumes no obligation to update publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

BridgeBio Media Contact

Kaitlyn Reilly, Director, Communications

contact@bridgebio.com

(650) 789-8220

BridgeBio Investor Contact

Kristen Kelleher, Director, Investor Relations

ir@bridgebio.com

Source: BridgeBio Pharma, Inc.