NEWS RELEASE

BridgeBio Reports Positive Phase 3 Results for Small Molecule BBP-418 in LGMD2I/R9 FORTIFY Study

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- All primary and secondary interim analysis endpoints in FORTIFY Phase 3 study successfully achieved with well-tolerated safety profile consistent with the Company's prior studies

- Primary interim analysis endpoint, glycosylated αDG, significantly increased by 1.8x change from baseline at 3 months ($p<0.0001$), and improvements were sustained at 12 months ($p<0.0001$) in BBP-418 treated individuals versus placebo

- Average reduction in serum CK, a marker of muscle damage, of 82% change from baseline and statistically significant difference versus placebo ($p<0.0001$) in BBP-418 treated individuals at 12 months

- BBP-418 treated individuals had statistically significant, clinically meaningful improvements at 12 months in all key clinical endpoints studied, including:

  - Ambulatory function (100MTT): Increase in velocity of 0.14 m/s from baseline and 0.27 m/s versus placebo ($p<0.0001$)
  - Pulmonary function (FVC): Increase of ~3% predicted volume from baseline and a difference of ~5% predicted volume versus placebo ($p=0.0071$)

- Company intends to file an NDA for approval with the FDA in first half of 2026

PALO ALTO, Calif., Oct. 27, 2025 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (Nasdaq: BBIO) ("BridgeBio" or the "Company"), a new type of biopharmaceutical company focused on genetic diseases, today reports positive topline

results from FORTIFY, the Company's Phase 3 pivotal study of BBP-418 in individuals living with limb-girdle muscular dystrophy type 2I/R9 (LGMD2I/R9). BridgeBio will host an investor call on October 27, 2025 at 8:00 am ET to discuss these results.

"I'm very excited to see that treatment with BBP-418 was associated with clinically meaningful improvements in motor and pulmonary function, along with robust restoration of αDG glycosylation. This is such an important result for individuals living with LGMD2I/R9, which is a progressive muscular dystrophy. The resulting weakness often leads loss of ambulation, need for respiratory support, and need for heart failure medications," said Katherine Mathews, M.D., Professor of Pediatrics and Neurology at the University of Iowa's Roy J. and Lucille A. Carver College of Medicine. "To date, there has been no specific treatment. These results bring enormous hope that BBP-418 might change the disease course."

"Living with LGMD2I/R9 is a daily negotiation with limits, and I won't pretend it's easy. I'm fiercely independent, but I'm not alone– our community lifts me, and it finally feels like science is catching up to our hope, so we can look ahead with real possibility." said Dan Pope, an individual living with LGMD2I/R9 and Vice President and Advocacy Director of the CureLGMD2i Foundation.

FORTIFY is a randomized, double-blind, placebo-controlled Phase 3 study, evaluating the efficacy and safety of BBP-418, an investigational small molecule oral therapy in development for the treatment of individuals living with LGMD2I/R9.

Key results from the planned interim analysis at 12 months include the following:

- Highly statistically significant increase of 1.8x change from baseline (~17% of control, $p<0.0001$) in the primary interim analysis endpoint of glycosylated alpha-dystroglycan (αDG) observed in the BBP-418 treated group compared to approximately no change in the placebo group from baseline to 3 months
- Highly statistically significant increases in glycosylated αDG were sustained at 12 months in the BBP-418 treated group ($p<0.0001$)
- Substantial and highly statistically significant increases in glycosylated αDG were observed both in individuals with the L276I homozygous genotype and other FKRP genotypes
- Average reduction in serum creatine kinase (CK), a marker of muscle breakdown, of 82% change from baseline ($p<0.0001$) in BBP-418 treated individuals
- BBP-418 treated individuals had statistically significant, clinically meaningful improvements at 12 months in all key clinical endpoints studied, including:
    - Ambulatory function (100MTT): Increase in velocity of 0.14 m/s from baseline and 0.27 m/s versus placebo ($p<0.0001$)
    - Pulmonary function (FVC): Increase of ~3% predicted volume from baseline and a difference of ~5%

predicted volume versus placebo (p=0.0071)

- BBP-418 was well-tolerated with no new or unexpected safety findings observed
- Overall, interim analysis data are supportive of a favorable risk-benefit profile for BBP-418

“Our heartfelt thanks go out to the individuals in our study, their caregivers, investigators, and study staff who have actively participated in FORTIFY and continue to contribute to this pivotal research. The FORTIFY results reaffirm the power of targeting this genetic disease at its source with relentless focus and compassion,” said Douglas Sproule, M.D., M.Sc., Chief Medical Officer of ML Bio Solutions, a BridgeBio company developing BBP-418 for LGMD2I/R9. “For individuals living with LGMD2I/R9, a condition that slowly takes away the strength, breathing, and independence of an individual, each day matters. We are determined to move swiftly toward filing for approval, so that BBP-418, if approved, can become the first therapy to change the course of this devastating disease.”

The Company intends to engage the FDA later this year to discuss these data and plans for submission of a New Drug Application (NDA) for BBP-418 in the first half of 2026. Analyses of the full FORTIFY interim analysis data are ongoing, and the Company plans to present detailed results at a future medical meeting.

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). Consistent with the Rare Pediatric Designation from the FDA, if BBP-418 is approved, BridgeBio may qualify for a Priority Review Voucher.

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 (αDG), 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 failure by 30 years of age in nearly all cases.

About BridgeBio Pharma, Inc.
BridgeBio Pharma, Inc. (BridgeBio) is a new type of biopharmaceutical company founded to discover, create, test, and deliver transformative medicines to treat patients who suffer from genetic diseases. BridgeBio’s pipeline of development programs ranges from early science to advanced clinical trials. BridgeBio was founded in 2015, and its

team of experienced drug discoverers, developers and innovators are committed to applying advances in genetic medicine to help patients as quickly as possible. For more information visit **bridgebio.com** and follow us on **LinkedIn**, **X, Facebook**, **Instagram**, and **YouTube**.

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, or the negative of these terms or other comparable terminology are intended to identify forward-looking statements, though not all forward-looking statements necessarily contain these identifying words. We intend 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, including express or implied statements relating to the Phase 3 interim topline results for small molecule BBP-418 in the LGMD2I/R9 FORTIFY Study, the efficacy, safety and the clinical and therapeutic potential of BBP-418, the progress of our ongoing and planned clinical trials of BBP-418, our plans to submit a New Drug Application for BBP-418 to the FDA in the first half of 2026, and the statements regarding the potential clinical benefits of BBP-418 for patients in the quotes of Dr. Mathews and Dr. Sproule, reflect our current views about our plans, intentions, expectations and strategies, which are based on the information currently available to us and on assumptions we have made. Although we believe that our plans, intentions, expectations and strategies as reflected in or suggested by those forward-looking statements are reasonable, we 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 our clinical trials not being indicative of final data, the design and success of ongoing and planned clinical trials, difficulties with enrollment in our clinical trials, adverse events that may be encountered in our clinical trials, the FDA or other regulatory agencies not agreeing with our regulatory approval strategies, components of our filings, such as clinical trial designs, conduct and methodologies, or the sufficiency of data submitted, the potential inability to obtain regulatory approvals in a timely manner or at all, potential manufacturing and supply chain interruptions, adverse effects on healthcare systems, disruption of the global economy, the impacts of current macroeconomic and geopolitical events, including hostilities in Ukraine and in Israel and the Gaza Strip, increasing rates of inflation and rising interest rates, on our business operations and expectations, as well as those risks set forth in the Risk Factors section of our most recent Annual Report on Form 10-K and our other subsequent filings with the U.S. Securities and Exchange Commission. Except as required by applicable law, we assume no obligation to update publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

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