



NEWS RELEASE

# bridgebio pharma surpasses interim analysis enrollment target and receives u.s. fda rare pediatric disease designation for bbp-418, a potential treatment for limb-girdle muscular dystrophy type 2i/r9 (lgmd2i/r9)

2024-06-18

- BridgeBio has surpassed its interim analysis enrollment target for its Phase 3 FORTIFY study of BBP-418 in individuals living with LGMD2I/R9, with top-line results from the interim analysis expected in 2025
- Recent Type C interactions with U.S. Food and Drug Administration (FDA) focused on the validated glycosylated alpha-dystroglycan (αDG) bioassay and our interim analysis plans reinforce BridgeBio's belief that there is potential to pursue Accelerated Approval for BBP-418
- Rare Pediatric Disease Designation for BBP-418 highlights that LGMD2I/R9 is a rare disease with serious manifestations, which primarily impacts children, and if BBP-418 is approved, BridgeBio may qualify for a Priority Review Voucher

PALO ALTO, Calif., June 18, 2024 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (Nasdaq: BBIO) ("BridgeBio" or the "Company"), a commercial-stage biopharmaceutical company focused on genetic diseases, today announced it has surpassed its interim analysis enrollment target and expects topline interim data from its Phase 3 registrational study (FORTIFY) in individuals with LGMD2I/R9 in 2025.

FORTIFY is a randomized, double-blind, placebo-controlled Phase 3 study evaluating the safety and efficacy of BBP-

418, an investigational oral therapy in development for the treatment of individuals living with LGMD2I/R9. The study includes a planned interim analysis at 12 months focused on assessing glycosylated  $\alpha$ DG as a surrogate endpoint to support Accelerated Approval. The primary endpoint to be evaluated at 36 months is the North Star Assessment (NSAD) for limb-girdle type muscular dystrophies and is designed to provide confirmatory clinical data. BridgeBio is continuing to enroll FORTIFY in the U.S., UK, Europe, and Australia. More information about the ongoing Phase 3 clinical trial of BBP-418 (NCT05775848) can be found [here](#) on clinicaltrials.gov.

Multiple encouraging discussions with the FDA in 2024, focused on the validated glycosylated  $\alpha$ DG bioassay and our interim analysis plans, continue to support the Company's plan to pursue Accelerated Approval for BBP-418 based on the use of glycosylated  $\alpha$ DG levels as a surrogate endpoint in LGMD2I/R9. Furthermore, the Agency indicated that the Company's approach to measure glycosylated  $\alpha$ DG levels via a novel, validated muscle tissue-based bioassay appears reasonable. A peer-reviewed manuscript providing details on the novel, multiplexed, fluorescence-based Western Blot assay was recently published in the **Journal of Muscle Research and Cell Motility**.

"Reduced glycosylated  $\alpha$ DG is the primary molecular driver of LGMD2I/R9, a serious, progressively debilitating disease that weakens the muscles causing many affected people to become fully dependent on a caregiver, while also threatening their cardiac and respiratory function," said Douglas Sproule, M.D., M.Sc., chief medical officer of ML Bio Solutions, a BridgeBio affiliate that is focused on developing BBP-418 for LGMD2I/R9. "We hope to continue to serve individuals through expeditious enrollment of the Phase 3 FORTIFY study and look forward to continuing to partner with the FDA to accelerate development of a potential new therapeutic option for people living with LGMD2I/R9."

The Company also announced that the FDA has granted Rare Pediatric Disease Designation (RPDD) for BBP-418 in the treatment of LGMD2I/R9, recognizing the rarity of this disease is characterized by serious manifestations primarily affecting children. If BBP-418 is approved, BridgeBio may qualify for a Priority Review Voucher based on receipt of the RPDD. A Priority Review Voucher can be applied to another therapy in the Company's pipeline for a shorter timeline during the review process of a New Drug Application or can be sold and transferred to another company looking to receive priority review for one of its applications.

#### 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 ( $\alpha$ 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 respiratory 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 FKR genotype 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 respiratory failure by 30 years of age in nearly all cases.

About BridgeBio Pharma, Inc.

BridgeBio Pharma, Inc. (BridgeBio) is a commercial-stage 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](https://www.bridgebio.com) and follow us on [LinkedIn](#) and [Twitter](#).

BridgeBio Pharma, Inc. Forward-Looking Statements

This press release contains forward-looking statements. Statements BridgeBio makes 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, including statements relating to the clinical and therapeutic and market potential of BridgeBio's programs and product candidates, including BBP-418 for the treatment of LGMD2I/R9, the drug design and the potential benefits of BBP-418, including its potential to address serious unmet need for patients with LGMD2I/R9, the potential and the opportunity to pursue Accelerated Approval Pathway for BBP-418 in LGMD2I/R9 in the U.S., the potential BridgeBio may qualify for a Priority Review Voucher based on receipt of the RPDD, the expected timeline of announcing the topline data from the interim analysis of FORTIFY in individuals with LGMD2I/R9 in 2025, and the statements regarding the potential benefit of our clinical trial or of our product candidate in the quotes of Dr. Sproule; the progress, timeline and success of BridgeBio's ongoing and planned clinical trials of BBP-418, the timeline of evaluation of the NSAD and secondary endpoints at 36 months, and the expectation of providing confirmatory clinical data from such results, the plans of engaging with regulatory authorities, including the collaboration and interaction with the FDA to address the challenges associated with LGMD2I/R9 drug development, among others, reflect BridgeBio's current views about its plans, intentions, expectations, strategies and prospects, which are based on the information currently available to BridgeBio and on assumptions BridgeBio has made. Although BridgeBio believes that its plans, intentions, expectations, strategies and prospects as reflected in or suggested by those forward-looking

statements are reasonable, BridgeBio 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, BridgeBio's ability to continue and complete its ongoing and planned clinical trials of BBP-418 for the treatment of LGMD2I/R9, initial and ongoing data from its 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 BridgeBio's regulatory approval strategies, components of our filings, such as clinical trial designs, conduct and methodologies, or the sufficiency of data submitted, potential adverse impacts due to global health emergencies, including delays in regulatory review, manufacturing and supply chain interruptions, adverse effects on healthcare systems and disruption of the global economy, the impacts of current macroeconomic and geopolitical events, including changing conditions from 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 BridgeBio's most recent Annual Report on Form 10-K, and BridgeBio's other filings with the U.S. Securities and Exchange Commission. Moreover, BridgeBio 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 BridgeBio'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, we assume no obligation to update publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

BridgeBio Contact:

Vikram Bali

**[contact@bridgebio.com](mailto:contact@bridgebio.com)**

(650)-789-8220