NEWS RELEASE

bridgebio pharma announces positive phase 2 data for limb-girdle muscular dystrophy type 2i (lgmd2i)

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- 43% increase in the ratio of glycosylated alpha-dystroglycan (αDG) to total αDG from baseline were measured across all three dosing cohorts, signifying the oral therapy has the potential to address both the root cause of LGMD2i and drive functional improvements for patients
- Average of 70% reduction in creatine kinase (CK), a key marker of muscle breakdown, after 90 days of treatment and average 77% reduction after 180 days
- Increase in velocity in the 10-meter walk test (10MWT) at day 90 and 180, which is an improvement over the decline in velocity seen in the natural history data
- If the development program is successful, BridgeBio believes BBP-418 could be the first approved therapy for the treatment of patients with LGMD2i
- BridgeBio to host investor call on March 14, 2022 at 8:00 AM ET

PALO ALTO, Calif., March 14, 2022 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (Nasdaq: BBIO) (“BridgeBio” or the “Company”), a commercial-stage biopharmaceutical company focused on genetic diseases and cancers, and its affiliate company ML Bio Solutions, today announced positive data from the Phase 2 study of BBP-418 in patients with limb-girdle muscular dystrophy type 2i (LGMD2i). The results are featured in a poster at the Muscular Dystrophy Association (MDA) 2022 Annual Meeting, taking place in Nashville, Tennessee on March 13 – 16, 2022.

BridgeBio believes these initial results indicate the potential for BBP-418 to increase glycosylation of αDG and drive functional improvements for patients, as well as reduce creatine kinase, a key marker of muscle breakdown. Furthermore, the 90- and 180-day data show improvements on walk tests from baseline, which the Company believes suggests a potential impact on clinical function and on the rate of disease progression.

“To date, people with LGMD2i have no approved disease-modifying treatment options. Many of these patients see their quality of life deteriorate rapidly and lose their functional independence, including their ability to walk. Our preliminary trial data holds promise for this unmet patient need as our investigational therapy is shown to be generally well-tolerated and to improve several key markers associated with a patient’s decline,” said Douglas Sproule, M.D., M.Sc., chief medical officer of ML Bio Solutions. ML Bio Solutions, based in Charlotte, North Carolina, is the affiliate company of BridgeBio that is focused on developing BBP-418 for LGMD2i.

BridgeBio plans to engage with regulatory health bodies in 2022 to discuss potential paths to approval and intends to initiate a Phase 3 clinical trial in the second half of the year. At MDA 2022, BridgeBio is also presenting Phase 1 trial data of BBP-418 in healthy volunteers to support its LGMD2i program. The Phase 1 study demonstrated broad tolerability across a wide range of dosing, including doses beyond expected therapeutic range. No dose limiting toxicity was observed.

The Phase 2 trial enrolled 14 participants, including both ambulatory and non-ambulatory patients with LGMD2i. The open-label study is designed to explore the safety and tolerability, feasibility, and usefulness of selected clinical efficacy and pharmacodynamic (PD) assessment of patients with LGMD2i receiving ascending doses of BBP-418 across three cohorts. Based on the data observed after 90 and 180 days of treatment, BridgeBio observed:

- Participants showed an average 0.21 or 43% increase in the ratio of glycosylated αDG to total αDG, signifying that the oral therapy has the potential to address both the root cause of LGMD2i and drive functional improvements for patients
- Participants showed statistically significant declines in all cohorts for CK, of 70% at day 90 for all cohorts and 77% at day 180 for cohorts 1 and 2. 11 of 12 participants received at least 50% reduction in CK with 75% of participants reaching 2x the normal range, suggesting a reduction in muscle breakdown
- All cohorts demonstrated a 0.08 m/sec (3%) increase in 10MWT velocity at day 90 and 0.12 m/sec (4%) increase at day 180 for cohorts 1 and 2. This result is encouraging in correlating the positive biomarker changes to potential clinical outcomes
- The 10MWTs were measured at six months and compare favorably to natural history data where the same patients demonstrated a decline of 0.12 m/sec in the 10MWT in the 6-months prior to enrollment in the Phase 2 study
- BBP-418 was well-tolerated across a wide range of dose levels with no treatment-related serious adverse events, dose limiting toxicities or discontinuations observed

“The positive results from the Phase 2 study exceed expectations and are incredibly exciting as they demonstrate

consistent improvements in key markers of muscle function and support further study," said Amy Harper, M.D., professor in the department of neurology at Virginia Commonwealth University (VCU) and primary investigator of the Phase 2 clinical trial in LGMD2i. "The close collaboration with BridgeBio and ML Bio Solutions on this trial has provided patient focused data that addresses a serious unmet need in a rare disease."

With approximately 7,000 patients in the U.S. and European Union with potentially treatable mutations, LGMD2i is an inherited autosomal recessive muscular dystrophy caused by the mutation of fukutin-related protein (FKRP), which results in hypoglycosylation of αDG. BBP-418 is designed to allow the muscle cell to properly glycosylate αDG, allowing αDG to function normally and potentially resulting in improved muscle strength and function for patients. If the development program is successful, the Company believes BBP-418 could be the first approved therapy for the treatment of patients with LGMD2i.

Focused execution is BridgeBio's top priority as it advances its pipeline of high-quality programs to help patients as quickly as possible. The Company remains dedicated to strategically developing and delivering transformative medicines for genetic diseases and cancers with unmet need.

Webcast Information

BridgeBio will host an investor call and simultaneous webcast to share updates on the Phase 2 data for limb-girdle muscular dystrophy type 2i on March 14, 2022 at 8:00 AM ET. To access this call, dial 800-379-2666 and enter conference ID 3562087. A link to the webcast may be accessed from the event calendar page of BridgeBio's website at **https://investor.bridgebio.com/**. A replay of the conference call and webcast will be archived on the Company's website and will be available for at least 30 days following the event.

About Limb-girdle Muscular Dystrophy Type 2i (LGMD2i)

LGMD2i is a monogenic autosomal recessive disease caused by partial loss of function mutations in the FKRP gene, and these FKRP mutations impair glycosylation of α-DG, a protein associated with stabilizing muscle cells. LGMD2i is a monogenic autosomal recessive disease caused by partial loss of function mutations in the FKRP gene, and these FKRP mutations impair glycosylation of 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 respiratory muscle and cardiac muscle involvement. Patients who harbor a homozygous genotype typically develop disease manifestations during late childhood with progression to loss of independent ambulation (25%), assisted ventilation (5%), and cardiomyopathy (10%) in adulthood. Cardiomyopathy is progressive, with an annual loss of 0.4% of left ventricular ejection fraction (LVEF). Patients with heterozygous genotypes have an earlier childhood onset with a more severe clinical course, rapid loss of mobility by 20 years of age, more frequent cardiac involvement (25%), 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 and cancers with clear genetic drivers. BridgeBio's pipeline of over 30 development programs ranges from early science to advanced clinical trials, and its commercial organization is focused on delivering the company's first two approved therapies. 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** and **Twitter**.

BridgeBio Pharma, Inc. Forward-Looking Statements

This press release contains forward-looking statements. Statements we make 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," "estimates," "expects," "intends," "may," "plans," "projects," "seeks," "should," "will," and variations of such words or similar expressions. 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 and are making this statement for purposes of complying with those safe harbor provisions. These forward-looking statements, including statements relating to the timing and success of ML Bio Solutions' clinical trials of BBP-418 for the treatment of LGMD2i, expectations, plans and prospects regarding ML Bio Solutions' regulatory approval process for BBP-418, the ability of BBP-418 to treat LGMD2i in humans, the potential for BBP-418 to be the first approved therapy for the treatment of LGMD2i and the timing and success of BridgeBio's clinical trials and development pipeline, reflect our current views about our plans, intentions, expectations, strategies and prospects, which are based on the information currently available to us and on assumptions we have made. Although we believe that our plans, intentions, expectations, strategies and prospects 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, ML Bio Solutions' ability to continue and complete its clinical trials of BBP-418 for the treatment of LGMD2i, past data from preclinical studies not being indicative of future data from clinical trials, ML Bio Solutions' ability to advance BBP-418 in clinical development according to its plans, the ability of BBP-418 to be the first approved therapy for the treatment of patients with LGMD2i, BridgeBio's ability to advance its clinical trials and development pipeline, the success of BridgeBio's approved drugs, as well as those risks set forth in the Risk Factors section of BridgeBio Pharma's Annual Report on Form 10-K for the year ended December 31, 2021, and BridgeBio Pharma's other SEC filings. Moreover, BridgeBio operates in a very competitive and rapidly changing environment in which new risks emerge from time to time. 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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