NEWS RELEASE

bridgebio pharma presents 12-month results from phase 2 study of bbp-418 in limb-girdle muscular dystrophy type 2i (lgmd2i)

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- Novel assay developed to assess the extent of alpha-dystroglycan (αDG) glycosylation, the core pathology of LDMD2i, from muscle biopsy samples
- Increase in the ratio of glycosylated αDG to total αDG from baseline towards normal levels, suggesting that the investigational oral therapy, BBP-418, has the potential to address the root cause of LGMD2i
- Large, sustained reduction in creatine kinase (CK) (>75%), a key marker of muscle breakdown, after 12 months of treatment
- Improvements in key functional measures, the north star assessment for dysferlinopathy (NSAD) and 10-meter walk test (10MWT), observed after 12 months of treatment with BBP-418
- If successful, BridgeBio believes BBP-418 could be the first approved therapy for the treatment of patients with LGMD2i

PALO ALTO, Calif., Oct. 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 reported 12-month data from the Phase 2 study of BBP-418 in patients with limb-girdle muscular dystrophy type 2i (LGMD2i). These updated results were presented in an **oral presentation** at the 27th International Hybrid Annual Congress of the World Muscle Society (WMS), taking place in

Halifax, Canada on October 11 – 15, 2022.

These additional Phase 2 results continue to support the potential for BBP-418 to restore glycosylation of alpha-dystroglycan (αDG), reduce muscle damage, and preserve motor function for patients over time, as demonstrated by the sustained increase in the ratio of glycosylated-αDG over total αDG, reduction in creatine kinase, improvements in NSAD scores, and improvements in 10MWT velocity observed over a 12-month period.

“LGMD2i dramatically impacts individuals living with the disease, fundamentally robbing people of their independence. As the disease progresses, individuals with LGMD2i lose the ability to function unaided, eventually becoming wheelchair-dependent and requiring ventilation assistance, the timing of which is dependent on the severity of their disease. Our Phase 2 data show our investigational therapy continues to be well-tolerated and may have the potential to improve or slow clinical decline associated with the disease,” 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.

BridgeBio and ML Bio Solutions continue to engage with regulatory agencies and intend to initiate a Phase 3 clinical trial in 2023. BridgeBio also presented data in a **poster** at WMS 2022 showcasing development of a novel bioassay to detect the extent of αDG glycosylation relative to total αDG from muscle biopsy samples. Given impaired glycosylation of αDG directly leads to the development of muscular dystrophy in LGMD2i patients, this novel assay was designed to assess the core pathology of the disease. “We believe this is the first time an increase in αDG has been demonstrated in LGMD2i patients as a result of therapy, and that these data are supportive of the therapeutic potential of BBP-418 to treat the disease at its source,” said Uma Sinha, Ph.D., Chief Scientific Officer of BridgeBio.

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, 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 after 12 months of treatment, BridgeBio observed:

- Increased glycosylation of αDG in all dose cohorts, with an average increase in αDG ratio of +0.21 at day 90
- Greater than 75% reduction in CK sustained over 12 monthsImprovements from baseline in NSAD (0.95) & 10MWT velocity (0.09 m/s) at 12 months
- No treatment-related SAEs or dose limiting toxicities with 12 months of treatment with BBP-418

“The continued positive data from this study are encouraging. In addition to being well-tolerated, the consistent improvements observed in both important biomarkers of muscle function as well as in clinical endpoints form a compelling proof of concept for BBP-418 in patients with LGMD2i,” 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. “These patients represent a serious unmet need, and it is exciting to see this treatment progress towards a Phase 3 study that may allow it to serve that need.”

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) gene, 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 the improvement or preservation of motor 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.

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 αDG, 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 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 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, including the intent to initiate a Phase 3 clinical trial in 2023, the development of a novel bioassay to detect the extent of αDG glycosylation relative to total αDG from muscle biopsy samples, 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, BridgeBio's ability to advance its clinical trials and development pipeline, 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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