NEWS RELEASE

bridgebio pharma's ml bio solutions announces dosing of first subject in phase 1 clinical trial of bbp-418 for limb girdle muscular dystrophy type 2i (lgmd2i)

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- ML Bio Solutions has initiated a Phase 1 clinical trial of BBP-418 in healthy volunteers
- ML Bio Solutions' sponsored lead-in study is enrolling patients with LGMD2i across twelve sites in the U.S. and internationally, in collaboration with the LGMD-GRASP consortium

SAN FRANCISCO, June 11, 2020 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (Nasdaq: BBIO) affiliate ML Bio Solutions today announced that the first subject has been dosed in the Phase 1 clinical trial of BBP-418, an orally-administered small molecule therapy being evaluated for the treatment of limb girdle muscular dystrophy type 2i (LGMD2i). The Phase 1 clinical trial is designed to assess safety, tolerability, pharmacokinetics and food effect of BBP-418 in healthy volunteers. In collaboration with the GRASP-LGMD consortium based at Virginia Commonwealth University, which is led by Nicholas Johnson, M.D., M.S.C.I., ML Bio Solutions is also enrolling a LGMD2i lead-in study across multiple centers in the U.S. and Europe. ML Bio Solutions also announced that the U.S. Food and Drug Administration (FDA) approved its Investigational New Drug (IND) application for BBP-418 in March 2020.

"The FDA's acceptance of the investigational new drug application for BBP-418 marks a turning point for patients with LGMD2i, who currently have no targeted treatment options. BBP-418 has demonstrated efficacy in both mild and severe animal models of LGMD2i, and the start of the Phase 1 trial brings us one step closer to translating its potential to change the course of this incurable and debilitating disease," said Uma Sinha, Ph.D., chief scientific officer of ML Bio Solutions and BridgeBio.

LGMD2i is an inherited muscular dystrophy that is associated with two mutant copies of a gene called fukutin-related protein (FKRP). The FKRP enzyme enables the proper functioning of a fully glycosylated alpha-dystroglycan (α-DG) protein on muscle cells. While fully glycosylated α-DG anchors the inside and the outside of cells to act as a "shock-absorber," hypo-glycosylated α-DG – such as occurs in LGMD2i and fukutinopathies – leads to muscle cells that cannot withstand the stress of normal cellular activities. ML Bio Solutions' oral therapy BBP-418 is designed to enhance the function of enzymes like FKRP in order to restore glycosylation levels of α-DG protein and thereby recover its shock absorber functionality. The hope is that BBP-418 is ultimately able to change the prognosis of patients with LGMD2i – who experience progressive loss of skeletal, respiratory and cardiac muscle strength that leads to loss of walking, need for ventilatory support and even death from heart failure – and who today have only palliative treatment options. BBP-418 has previously been granted Orphan Drug Designation for LGMD2i by the FDA.

At this pivotal juncture, ML Bio Solutions has appointed a chief medical officer, Douglas Sproule, M.D. M.Sc., to lead clinical development as well as regulatory strategy and affairs for BBP-418. Dr. Sproule is a trained pediatric neuro-muscular specialist who worked most recently as vice president, clinical development and medical affairs and spinal muscular atrophy (SMA) therapeutic area head at AveXis.

"It is a privilege to join ML Bio Solutions as it rapidly advances an oral medicine with the potential to treat patients with LGMD2i as well as other alpha-dystroglycanopathies in the U.S., European Union and rest of world. Data from the ongoing lead-in and Phase 1 studies, guidance from our world-class scientific and clinical advisors, and feedback from the FDA and other regulatory authorities will inform and accelerate development of BBP-418 to address a critical unmet need for new treatment options in LGMD2i," said Dr. Sproule.

"The potential of a disease-modifying treatment from ML Bio Solutions for LGMD2i represents a beacon of hope for our community. We stand ready to provide guidance about the needs and experiences of patients and caregivers to inform development of BBP-418 to ensure it can have the maximal potential impact and benefit for patients," said Kathryn Bryant, CEO and founder of the Speak Foundation, a patient-led organization for people with limb girdle muscular dystrophy.

About GRASP-LGMD (Genetic Resolution and Assessments Solving Phenotypes in LGMD) Consortium

The GRASP-LGMD consortium assembles an international team of neuromuscular specialists, basic scientists, physical therapists, geneticists, informaticians, and patient advocates to address issues related to: diagnostics; outcome measure development, patient engagement; and therapeutic development, to advance the state of LGMD research and readiness to support translation of science into therapeutic development. For more information visit: **http://www.grasp-lgmd.org**

About the ML Bio Solutions-sponsored GRASP Consortium lead-in study:

A lead-in study is currently enrolling patients with a confirmed genetic diagnosis of LGMD2i across 12 centers in the U.S. and Europe to collect data on clinical outcome assessments as well as disease specific muscle biomarkers and MR imaging biomarkers that can be used to inform future planned drug study trials in LGMD2i. For more information visit: **https://clinicaltrials.gov/ct2/show/NCT04202627** OR contact **info@mlbiosolutions.com**

About ML Bio Solutions

ML Bio Solutions, an affiliate of BridgeBio Pharma, is a biotechnology company focused on developing a small molecule as an oral substrate supplementation therapy for LGMD2i. ML Bio Solutions is led by a team of veteran biotechnology executives, and together with patients and physicians, aims to bring safe, effective treatments to market as quickly as possible. For more information, visit **mlbiosolutions.com.**

About BridgeBio Pharma

BridgeBio is a team of experienced drug discoverers, developers and innovators working to create life-altering medicines that target well-characterized genetic diseases at their source. BridgeBio was founded in 2015 to identify and advance transformative medicines to treat patients who suffer from Mendelian diseases, which are diseases that arise from defects in a single gene, and cancers with clear genetic drivers. BridgeBio's pipeline of over 20 development programs includes product candidates ranging from early discovery to late-stage development. For more information, visit **bridgebio.com.**

BridgeBio Pharma 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' Phase 1 clinical trial 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, and the timing and success of initial top-line Phase 1 date of BBP-418, 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 Phase 1 clinical trial of BBP-418 for the treatment of LDMD2i, the continuing success of ML Bio Solutions' collaboration with the GRASP-LGMD consortium based at Virginia Commonwealth University, 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 treat LGMD2i, as well as those risks set forth in the Risk Factors section of BridgeBio Pharma's most recent Quarterly Report on Form 10-Q and BridgeBio Pharma's other SEC filings. Moreover, ML Bio Solutions 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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