



NEWS RELEASE

bridgebio pharma and affiliate ml bio solutions announce dosing of first patient in phase 2 trial of bbp-418 in limb girdle muscular dystrophy type 2i (lgmd2i)

2021-02-19

SAN FRANCISCO, Feb. 19, 2021 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (Nasdaq: BBIO), a clinical-stage biopharmaceutical company founded to discover, create, test and deliver meaningful medicines for patients with genetic diseases and cancers with clear genetic drivers, and affiliate ML Bio Solutions today announced that the first patient has been dosed in a Phase 2 trial of BBP-418 in patients with LGMD2i. BridgeBio and ML Bio's BBP-418 is the first-ever oral disease-modifying investigational treatment for LGMD2i. BBP-418 was granted Orphan Drug Designation for LGMD2i by the US Food and Drug Administration (FDA) in 2019, and for LGMD by the European Medicines Agency (EMA) in 2020.

LGMD2i is an inherited recessive muscular dystrophy caused by mutation of fukutin-related protein (FKRP). FKRP is an enzyme that adds a sugar molecule to a critical muscle cell structural protein called alpha-dystroglycan (α DG). Due to defective FKRP enzyme function, muscle cells of patients affected by LGMD2i lack the structural integrity that is provided by fully glycosylated α DG protein. This leads to chronic muscle injury and loss, muscle weakness and disability. Many affected patients ultimately lose their ability to walk, and some develop a need for ventilatory support or die from heart failure. Currently, there are no disease-modifying therapies for people with LGMD2i.

BBP-418 is designed to bypass the metabolic defect in LGMD2i by providing the FKRP enzyme with precursor sugar molecules that supplement the body's natural sugars used by FKRP to glycosylate the α DG protein on muscle cells. BBP-418 represents a novel substrate rescue approach with the potential to improve muscle strength and function.

The Phase 2 trial is expected to enroll up to 16 patients with a genetically-confirmed diagnosis of LGMD2i. In addition to safety, key efficacy measurements include changes in muscle α DG glycosylation levels, and changes in functional measures including 10 meter walk and North Star for Dysferlinopathy (NSAD).

“The start of our Phase 2 trial is a key milestone in our effort to develop a safe and efficacious therapy for patients with LGMD2i who lack treatment options,” said Dr. Douglas Sproule, M.D. M.Sc., Chief Medical Officer of ML Bio Solutions. “This trial launch is an outgrowth of our strong partnership with the GRASP-LGMD consortium based at Virginia Commonwealth University, where we are currently enrolling a lead-in study to define LGMD2i-specific biomarkers and functional endpoints. Our hope is that the ongoing lead-in study, together with early data from our Phase 2 trial, will hasten the successful execution of a future registrational trial and ultimately advance a meaningful medicine for patients in need.”

“We are pleased to continue our collaboration with ML Bio Solutions on the lead-in study that is currently enrolling and look forward to further extending it by launching the first-in-patient treatment study for BBP-418. Our clinical partnership is seeking to validate the biomarker assays and establish the registrational clinical outcome measures that are needed to bring BBP-418 to LGMD2i patients,” said Dr. Nicholas Johnson M.D. M.S.C.I., Associate Professor of Neurology and Chair of Research at Virginia Commonwealth University and Director of the GRASP-LGMD consortium.

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 ML Bio Solutions

ML Bio Solutions, an affiliate of BridgeBio Pharma based in a Charlotte, NC, 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 2 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 2 data 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 2 clinical trial of BBP-418 for the treatment of LGMD2i, 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 BioSolutions 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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Source: BridgeBio Pharma