



NEWS RELEASE

## bridgebio pharma expands its research into neuromuscular disease to include limb-girdle muscular dystrophy type 2 (lgmd2i) with new subsidiary ml bio solutions

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Provides BridgeBio with leading scientific expertise to develop oral small molecule therapy for LGMD2i  
CHARLOTTE, N.C. and SAN FRANCISCO, Sept. 05, 2019 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (NASDAQ: BBIO) today announced ML Bio Solutions as a new subsidiary dedicated to developing BBP-418, a substrate supplementation therapy for the treatment of Limb-Girdle Muscular Dystrophy Type 2i (LGMD2i). BridgeBio is committing R&D expertise to advance this small molecule oral therapy towards patients and intends to begin a natural history study in the second half of 2019, with interventional trials to initiate as soon as possible thereafter, subject to IND approval. The McColl Lockwood Laboratory for Muscular Dystrophy Research at Atrium Health will continue to support the program alongside BridgeBio.

LGMD2i is an autosomal recessive disease wherein loss-of-function mutations in the FKRP gene lead to decreased glycosylation of alpha-dystroglycan ( $\alpha$ -DG). Properly-glycosylated  $\alpha$ -DG anchors muscle cells to proteins outside of the cell in the extracellular matrix (ECM) as well as shock absorber proteins inside of the muscle cells, such as dystrophin. In LGMD2i,  $\alpha$ -DG has a defective sugar backbone, and thus cannot effectively bind these proteins to stabilize the muscle cell, leading to muscle cell fragility and damage under stress. Damaged and degenerating muscle cells can result in global loss of muscle strength. LGMD2i is a progressive and debilitating disease that affects an estimated 7,000 patients across the US and EU, with more affected globally. It is associated with loss of mobility and ultimately respiratory and cardiac failure, and there is presently no approved treatment.

ML Bio Solutions' compound BBP-418 aims to bypass the core metabolic defect in LGMD2i by providing a pro-drug [to the other enzymes in the pathway] that can be metabolized into the missing substrate in the  $\alpha$ -DG sugar backbone. This therapeutic approach originates from the discovery and translational work of scientific co-founder Dr. Qi Long Lu, director of the McColl-Lockwood Laboratory for Muscular Dystrophy Research at Atrium Health, **as published in a Nature Communications article in 2018**. A brief overview of the program can be found in the **investor section of the BridgeBio website**.

"By combining the scientific leadership of the McColl-Lockwood Laboratory with the rare disease development expertise at BridgeBio, we believe we can accelerate the path to making a safe and effective disease-modifying treatment available to LGMD2i patients as soon as possible," said Luther Lockwood, chairman of the board and President of ML Bio Solutions.

"BBP-418 is a novel approach that targets LGMD2i at the source of disease, and exemplifies the BridgeBio mission of creating life-altering medicines by pairing well-understood genetically- driven phenotypes with treatments whose mechanisms target the sources of disease," said Uma Sinha, Ph.D, chief scientific officer of BridgeBio Pharma. "We are thrilled to partner with the foremost experts in the field to explore the translation of these important discoveries into clinical impact for patients who currently lack effective treatment options."

#### About ML Bio Solutions

ML Bio Solutions, a subsidiary of BridgeBio Pharma based in 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.

#### 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 15 development programs includes product candidates ranging from early discovery to late-stage development.

#### Forward-Looking Statements

This press release contains forward-looking statements. Statements we make in this press release may include statements which are not historical facts and are considered forward-looking within the meaning of Section 27A of

the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, 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 Securities 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 clinical and therapeutic potential of BBP-418, our intention to initiate a natural history study and our plans to initiate clinical development of BBP-418, and the timing of these events, 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 variety of risks and factors that are beyond our control including, without limitation, our ability to execute our clinical and regulatory strategy on the timelines we anticipate, as well as those set forth in the Risk Factors section of BridgeBio Pharma Inc.’s most recent Quarterly Report on Form 10-Q and our other SEC filings. Except as required by 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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