



January 2021

Re: MLB-01-001 Biomarker Development in LGMD2I

Dear Friend,

I am writing to provide you with information about the LGMD 2I Natural History study noted above that is actively recruiting patients at multiple sites across the US. This is a natural history study for individuals with LGMD2I, all of whom may be eligible to participate in the subsequent phase 3 trials of BBP-418 (Ribitol), an oral medication for LGMD2I.

Enrolling in the MLB-01-001 trial will involve your participation for 12 months, during which time you will be required to attend 5 study visits. You must meet the following criteria to be eligible for participation:

- Males and females ages 10-65
- Genetic confirmation of LGMD2I
- Clinically affected, defined as weakness on bedside evaluation in either a limb-girdle pattern, or in a distal extremity
- A 10-meter walk time of ≥ 4 seconds (A certain number of wheelchair-users will also be eligible to participate.)
- No other illnesses that would interfere with the ability to undergo safe testing

The study does not involve a treatment. The objective is to identify appropriate assessments (endpoints) that will be used in the clinical trial of BBP-418 (Ribitol). These may include:

- Fine needle muscle biopsy
- Muscle MRI of the lower extremities
- Potential clinical trial endpoints such as: 10-meter walk test, 100-meter walk test, 4- stair climb test, timed up and go test, and the Modified North Star Assessment for Limb Girdle Muscular Dystrophy
- Pulmonary (lung) function testing
- Assessment of quality of life and activities of daily living using several patient-reported outcomes

If you have additional questions regarding the study, please contact Brittany Holmberg at Brittney.Holmberg@vcuhealth.org or 804-552-0014. Or visit www.mlbiosolutions.com for a FAQ document and full listing of study sites. Due to COVID restrictions, remote enrollment may be available and in-person visits will observe strict COVID precautions. All study costs are covered.

Thank you for your attention and best regards,

Douglas Sproule, MD
Medical Director, ML BioSolutions

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Principal Investigator