A Phase 3 Randomized, Placebo-controlled, Double-blind Study to Evaluate the Efficacy and Safety of BBP-418 (ribitol) in Patients with Limb Girdle Muscular Dystrophy 21 (LGMD21)

Status: Recruiting

Eligibility Criteria

Sex: Male or Female
Age Group: Not specified
This study is NOT accepting healthy volunteers

Inclusion Criteria:

- 12 to 60 years of age - genetically confirmed diagnosis of limb girdle muscular dystrophy - have clinical symptoms of weakness - weight at least 30 kg (66 lbs.) - willing to use a highly effective method of birth control until 12 weeks after last dose of study medication

Exclusion Criteria:

- any significant medical or mental health diagnosis including abnormal lab values (study staff will review) - surgery for scoliosis or other indication planned during the time of the study - use of ribose or other sugar alcohol-containing supplement within 90 days of staring the study - use of a systemic corticosteroid for the treatment of muscular dystrophy within 90 days of starting the study

Conditions & Interventions

Conditions:
Rare Diseases
Keywords:
Clinics and Surgery Center (CSC), Limb-Girdle Muscular Dystrophy Type 2I (LGMD2I), Muscular Dystrophy

More Information

Description: This study will use BBP-418 study drug in patients with LGMD to assess the clinical biomarkers, efficacy and safety of BBP-418 during the 36 months treatment phase.
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