Defining Clinical Endpoints in LGMD

Status: Recruiting

Eligibility Criteria

Age: Not specified

This study is NOT accepting healthy

Healthy Volunteers: volunteers

Inclusion Criteria:

- 4 to 65 years of age - diagnosis of Muscular Dystrophy with weakness in either a limb-girdle pattern, or in a arm or leg - confirmed mutation in ANO5, CAPN3, DYSF, DNAJB6 or SGCA-G.

Exclusion Criteria:

- bleeding disorder, platelet count less than 50,000, or currently taking an anticoagulant. - women who are pregnant - other illness that would interfere clinical trial (study staff will review)

Conditions & Interventions

Conditions:
Rare Diseases
Keywords:
Limb Girdle Muscular Dystrophy, Limb-Girdle Muscular Dystrophy (LGMD), Muscular Dystrophy

More Information

Description: Limb Girdle Muscular Dystrophy comprise a group of disorders made up of over 30 mutations which share a common phenotype of progressive weakness of the shoulder and hip girdle muscles. While the individual genetic mutations are rare, as a cohort, LGMDs are one of the four most common muscular dystrophies. The overall goal of project 1 is to define the key phenotypes as measured by standard clinical outcome assessments (COAs) for limb girdle muscular dystrophies (LGMD) to hasten therapeutic development.
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