Modulation of SERCA2a of Intra-Myocytic Calcium Trafficking in Cardiomyopathy Secondary to Duchenne Muscular Dystrophy

Status: RECRUITING

Eligibility Criteria

Sex: MALE
Age: 18 years and over
Healthy Volunteers: This study is NOT accepting healthy volunteers
Inclusion Criteria:

- Diagnosis of DMD with confirmatory genetic testing
- Cardiomyopathy with left ventricular scar in at least 3 of 16 segments
- Left ventricular ejection fraction < 40%
- Individualized, optimized cardiac medical therapy and glucocorticoid treatment for at least 12 months prior to enrollment
- Willing and able to provide informed consent

Exclusion Criteria:

- Abnormal blood pressure
- Non-DMD-related liver function test elevations
- Cystatin C ≥ 1.2 mg/L
- Thrombocytopenia
- Anemia
- Inadequate pulmonary function

Conditions & Interventions

Interventions:
GENETIC: SRD-001
Conditions:
DMD-Associated Dilated Cardiomyopathy

More Information

Description:
This research study is testing whether an experimental drug, called SRD-001, is safe and helps the weakened heart of patients with Duchenne muscular dystrophy (DMD) regain its ability to effectively pump blood to the rest of the body. SRD-001 is a form of gene therapy. The goal of SRD-001 gene therapy is to provide the heart muscle cells with extra copies of the SERCA2a gene so that they can produce more SERCA2a protein to help the heart muscle cells squeeze/contract better. Researchers will compare SRD-001 treated participants with no-treatment participants; all participants will continue to take their current heart medications. All participants will be followed very closely for 2 years and undergo cardiac magnetic resonance imaging of their heart at baseline, year 1 and year 2 along with assessment of upper limb function and lung function. After the 2 years of close follow-up, all participants will roll over into long-term follow-up where they will be called biannually for information on their current medical status.

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