

Clinical Course Of Disease In Participants With FA-CM

Status: RECRUITING

Eligibility Criteria

Sex: ALL

Age: 6 years and over

Healthy Volunteers: This study is NOT accepting healthy volunteers

Inclusion Criteria:

- Male or female, ages ≥ 6 years at the time of signing the informed consent (and assent, if applicable).
- Diagnosis of FA, based on clinical phenotype and genotype (GAA expansion on both alleles or compound heterozygous), with onset of FA occurring at ≤ 25 years of age
- Confirmed left ventricular hypertrophy (LVH)
- Left ventricular ejection fraction $\geq 30\%$

Exclusion Criteria:

- Presence of other form(s) of CM contributing to heart failure (HF), clinically significant cardiac anatomic abnormality or congenital cardiac malformation, clinically significant coronary artery, uncorrected, hemodynamically significant primary structural valvular disease not due to CM
- Currently receiving intermittent or continuous intravenous (IV) inotrope infusion, presence of a ventricular assist device, or history of prior heart transplantation
- Contraindication to cMRI, participants < 12 years of age who cannot complete the cMRI without sedation will instead undergo ECHOs and are exempt from this criterion.
- Prior organ transplantation
- Initiation of cardiac resynchronization therapy (CRT) within 6 months prior to screening.
- History of prior gene transfer or cell therapy.
- Poorly controlled diabetes (hemoglobin A1c $\geq 8\%$)
- Active hematologic or solid organ malignancy

Conditions & Interventions

Conditions:

Friedreich Ataxia, Cardiomyopathy

Keywords:

Friedreich Ataxia, FA-CM, Cardiomyopathy, FA, Cardiac Disease

More Information

Description:

Characteristics and clinical course of disease In participants with cardiomyopathy associated with Friedreich Ataxia (CLARITY-FA)

Contact(s): Jaynee.Bartsch@cchmc.org

Principal Investigator:

Principal Investigator ID:

Phase:

IRB Number:

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