First-in-human Friedreich's Ataxia Gene Therapy Study

This Phase 1/2 study investigating a single-dose gene therapy for cardiomyopathy associated with Friedreich's Ataxia and recruitment is currently ongoing for eligible participants

What is this study about?

The main goal is to learn about the safety and tolerability of 2 different doses of an investigational gene therapy in participants with cardiomyopathy associated with Friedreich's Ataxia. All eligible participants will receive one of the two doses of gene therapy; there will no placebo in this study. A placebo is therapy that has no active properties.

This investigational gene therapy is specifically designed to deliver a normal copy of the human FXN (Frataxin) gene to heart muscle cells (and other cells in the body). This normal FXN gene may result in the production of increased amounts of FXN protein, which could then stabilize and/or improve the function and structure of the heart muscle cells. This potential improvement in the function of heart muscle cells may lead to improvement in the cardiomyopathy associated with Friedreich's Ataxia.

To learn more about gene therapy, please visit: https://curefa.org/trial

You may be eligible if you:

- Are a male or female 18 - 40 years of age
- Have a confirmed diagnosis of Friedreich's Ataxia, with onset of disease before 25 years of age
- Have evidence of cardiomyopathy
- Are willing and able to undergo cardiac biopsies and MRIs

There are additional eligibility criteria that will be assessed and discussed with you by the study doctor at the time of screening.

Participation involves:

- Study visits over approximately 5 year period:
  - The screening period will take up to 3 months
  - There will be 16 office visits during the first year following the investigational therapy administration. After that, there will be a long-term follow up period consisting of an additional 10 office visits over the next 4 years.
- Immediately after the investigational gene therapy is given, participants will need to stay in the hospital for at least two nights
- Participants will also need to reside close to the study site for up to 4 weeks after the investigational gene therapy is given

Quick facts:

- The investigational gene therapy and all study-related assessments will be provided at no cost
- You may be reimbursed for study-related expenses, such as parking, meals, and other reasonable accommodations
- Travel and accommodations will be arranged for participants in the study

If you are interested, please visit ClinicalTrials.gov to see where this study is being conducted and who to contact for more information about participating in the study.