Friedreich's Ataxia Research

Participants are needed for a Phase I research study to test an investigational drug in individuals with Friedreich's ataxia.

You May Be Eligible if You:

- Are 18 years of age or older
- Have genetically confirmed Friedreich's Ataxia, homozygous GAA repeat expansions, with repeat sizing.
- Are able to traverse 25 feet with or without an assistive device
- Are able to perform basic daily care
- Are able to sit upright with thighs together and arms crossed without requiring support on more than two sides
- Are able to transfer from bed to chair either independently or with minimal assistance while still physically contributing to the transfer in some way (e.g. able to partially support your weight)

Quick Facts

1 telehealth visit
3 remote visits by visiting nurse
1 in-patient stay up to 22 days/21 nights

Total duration up to 71 days

Travel Reimbursement

Compensation for Participating

Clinical research site is located in Eatontown, NJ

CLIN-1601-102

CTI-1601, a drug being developed by Larimar Therapeutics, uses a carrier protein to deliver frataxin to the mitochondria in cells. Frataxin is the protein deficient in individuals with Friedreich's ataxia. Your participation in this study of CTI-1601 will determine safety and tolerability of this investigational drug.

Key Inclusion Criteria

1. Genetically confirmed FRDA diagnosis, homozygous GAA repeat expansion with repeat sizing on diagnostic report.
2. Subject is male or female, 18 years of age or older at screening.
3. mFARS_neuro score ≥ 20 and be able to traverse 25 ft with or without some assistive device (cane, walker, self-propelled wheelchair) and:
   - Sit upright (thighs together, arms crossed) without support on more than 2 sides
   - Transfer from bed to chair either independently or with minimal assistance while still physicially contributing to the transfer in some way (e.g. able to partially support weight)
   - Perform basic daily care such as feeding and personal hygeine, with minimal assistance
4. Weight greater than 40 kg

Key Exclusion Criteria

1. Compound heterozygous (GAA repeat expansion on only one allele) for FRDA.
2. Subject used erythropoietin, etravirine, or gamma interferon within 90 days prior to screening
3. Subject with clinically significant arrhythmia on ECG or evidence of predisposition to significant ventricular arrhythmia on ECG, or evidence of active and unstable coronary artery disease
4. Male subject with QTcF > 450 msec or Female subject with QTcF > 470 msec
5. Subject with screening echocardiogram ejection fraction < 45%
6. Subject requires use of amiodarone
7. Subject use of investigational drug (other than CTI-1601) or device within 90 days prior to screening.
8. Subject use of daily biotin supplementation that exceeds 30 mcg/day, either as part of a multivitamin or as a standalone supplement, within 7 days prior to dosing and throughout the study.
9. History of aspiration, aspiration pneumonia, or recurrent episodes of pneumonia (great than or equal to 2 episodes of pneumonia) within the last 12 months.

There will be additional inclusion and exclusion criteria evaluated at the time of initial screening.

Study Schedule and Time Commitment

This study will be up to 71 days in duration with 1 telehealth visit, 3 home nurse visits, and 1 in-patient stay at the clinical research unit in Eatontown, NJ of up to 22 days/21 nights. In the clinical research unit during your inpatient stay, you will have your own bedroom, an accessible bathroom, and activities. A caregiver is also welcomed to accompany you during your participation. CTI-1601 will be given by injection, and monitoring of response to CTI-1601 will include periodic blood draws.

Contact Us Today
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www.clinilabs.com/volunteers

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