Friedreich's Ataxia Research

Participants are needed for a Phase 2 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 FA, GAA repeat expansions on each gene

-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)

-Have not used omaveloxolone in the last 30 days and agree not to receive omaveloxolone treatment for the duration of the study

Quick Facts

1 telehealth visit

3 remote visits by visiting nurse

Commitment to being at the study site & nearby hotel for 32 days

Total duration up to 93 days

Travel Reimbursement

Compensation for Participating

Clinical research site is located in Eatontown, NJ

Contact Us today at (212)994-4567 or at getinvolved@clinilabs.com to Learn More.

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CLIN-1601-200

CTI-1601, an investigational 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 help determine the 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 25ft 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 physically contributing to the transfer in some way
   -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. Male subject with QTcF > 450 msec or Female subject with QTcF > 470 msec
4. Subject with screening echocardiogram ejection fraction <45%
5. Subject requires use of amiodarone
6. Subject use of investigational drug (other than CTI-1601) or device within 90 days prior to screening
7. Subject use of daily biotin supplementation that exceeds 30 mgc/day, either as part of a multivitamin or as a standalone supplement, within 7 days prior to dosing and throughout the study
8. Omaveloxolone use within 30 days prior to screening and agree not to receive omaveloxolone treatment for duration of the study

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

Study Schedule and Time Commitment

One telehealth visit and three home nurse visits, 17 days/16 night inpatient stay, five outpatient follow-up visits where you will be housed locally and return to the study site every other day for five visits, followed by a 5 day/ 4 night inpatient stay. Upon discharge, home nurse follow-up visits 7 days and 30 days after the last dose of study drug. Total duration up to 93 days. Visits will be conducted at the clinical research unit in Eatontown, NJ. In the clinical research unit during your impatient stay, you will have your own bedroom, an accessible bathroom, and activities. A caregiver is also welcome 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.

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