An Open-Label Study of CTI-1601 in Subjects With Friedreich's Ataxia

NCT06447025

Status	RECRUITING
Phase	Phase 2
Sponsor	Larimar Therapeutics, Inc.
Enrollment	85 participants

Key Eligibility Criteria

Inclusion (5)

- Subjects with FRDA who have or have not previously completed participation in a study of CTI-1601 are eligible to participate in this study unless the subject experienced one or more of the following in a previous CTI-1601 study: a) serious adverse event (SAE) related to study drug; b) significant AE, defined as Grade 3 or higher according to the Common Terminology Criteria for Adverse Events (CTCAE), version 5.0 (or higher), related to study drug; c) some other event, related to participation in a previous study with CTI-1601, that supports the exclusion of the subject from participating in this study as determined by the Sponsor (i.e., an AE considered clinically significant by the Sponsor regardless of whether it met SAE criteria and regardless of CTCAE grade); d) Withdraw from participation in a previous study of CTI-1601 for any reason.
- Subject has a HbA1c less than or equal to 7.0%.
- Subject must demonstrate sufficient dexterity and visual acuity to prepare and self-administer SC injections of CTI-1601 QD or is able to identify a caregiver who will be trained and committed to prepare and administer the daily injections.
- If subject is taking permitted concomitant medication(s), subject must have been on a stable dose and frequency of medication(s) over the past 28 days prior to the initiation of Screening; however, subjects taking niacin and resveratrol must have been on a stable dose and frequency for 90 days prior to the initiation of Screening
- \- Subjects who are currently receiving omaveloxolone or intend to receive omaveloxolone are permitted in the study but must either receive CTI-1601 for 3 months prior to their first dose of omaveloxolone or receive omaveloxolone for 3 months prior to their first dose of CTI-1601.

Exclusion (11)

- Subjects who are confirmed as compound heterozygous (GAA repeat expansion on only one allele) for FRDA.
- Subject has any condition, disease, or situation, including a cardiac condition or disease, that in the opinion of the PI, could confound the results of the study or put the subject at undue risk, making participation inadvisable.
- Subject used any investigational drug (other than CTI-1601) or device within 90 days prior to Screening.
- Subject requires use of amiodarone.
- Subject used erythropoietin, etravirine, or gamma interferon within 90 days prior to Screening.

... and 6 more (see full listing online)

Locations (8 total)

University of California Los Angeles, Los Angeles, California, United States
Fixel Institute for Neurological Disease, University of Florida Health, Gainesville, Florida, United States
Morsani Center for Advanced Health Care, University of South Florida Health, Tampa, Florida, United States
... and 5 more locations