A Study of SGT-212 Gene Therapy in Friedreich's Ataxia

NCT07180355

Status	RECRUITING
Phase	Phase 1
Sponsor	Solid Biosciences Inc.
Enrollment	10 participants

Key Eligibility Criteria

Inclusion (6)

- Has history of FA symptom onset d25 years of age
- Has a clinical and genetic diagnosis of FA
- Has a staging score of e1 but \<6 on the Friedreich's Ataxia Rating Scale (FARS) Functional Disability Staging Score
- Is willing to agree to the following rules for use of omaveloxolone (Skyclarys):
- For a candidate who is currently taking omaveloxolone, has been on a stable dose for 12 weeks, expects to continue taking omaveloxolone at that dose throughout the study, and is willing to stop taking omaveloxolone at the direction of the Investigator or Sponsor's Medical Monitor if evidence of transaminitis or synthetic liver dysfunction is detected during the study

... and 1 more (see full listing online)

Exclusion (11)

- Antibodies against adeno-associated virus serotype 9 (AAV9)
- Has a modified FARS (mFARS) score \<20
- Has a body weight d25 kilogram (kg) or has body mass index (BMI) e33 kg/m\^2
- Has a contraindication to endomyocardial biopsy (EMB) or cardiac catheterization
- Is unable to undergo cardiac and brain MRI with contrast, including hypersensitivity to gadolinium contrast agent, presence of a non-MRI-compatible cardiac pacemaker, presence of a non-MRI-compatible implantable cardiac defibrillator, or physical condition (e.g., contractures)

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

Locations (3 total)

The University of California, Los Angeles (UCLA), Los Angeles, California, United States
The Ohio State University, Columbus, Ohio, United States
The Children's Hospital of Philadelphia (CHOP), Philadelphia, Pennsylvania, United States