FRIEDREICH'S ATAXIA
RESEARCH

Nomlabofusp, a drug being developed by Larimar Therapeutics, Inc., is a recombinant fusion protein intended to deliver human frataxin into the mitochondria of patients with Friedreich's ataxia. Frataxin is the protein deficient in individuals with Friedreich's ataxia. Your/your child's participation in this study on nomlabofusp will help determine the safety and tolerability of this investigational drug.

Key Inclusion Criteria:

1. Participant has confirmed diagnosis of Friedreich's ataxia via genetic testing.
2. Male or female participants 2 to 17 years of age at screening and weighs ≥ 22.0 lbs.
3. Participant must be able to travel a distance of 25 feet with or without some assistive device (e.g., cane, walker, crutches, self-propelled wheelchair) and meet the following requirements:
    a. Be able to sit upright with thighs together and arms crossed without requiring support on more than 2 sides;
    b. Be able to transfer from bed to chair independently or with assistance if, in the opinion of the investigator, the degree of physical disability does not result in undue risk to the participant while participating in the study; and
    c. Perform basic age-appropriate daily care, such as feeding themselves and personal hygiene, with minimal assistance.

Key Exclusion Criteria:

1. Participants who are confirmed as compound heterozygous (GAA repeat expansion on only 1 allele) for Friedreich's ataxia.
2. Participant used erythropoietin, etravirine, or gamma interferon within 90 days prior to screening
3. Participant has a screening/baseline ECHO ejection fraction < 45%.
4. Participant requires use of amiodarone
5. Participant used an investigational drug or device within 90 days of screening.
6. Participant used omaveloxolone for the last 30 days or plans to receive omaveloxolone treatment during the study.

Study Schedule and Time Commitment

This study will be up to 75 days in duration. The study will require one remote visit, one home visit during the screening period (up to 35 days in duration), and daily treatment visits over a 7 day period at the study site, Uncommon Cures in Chevy Chase, MD followed by two home visits during the 30-day follow-up period. Study drug will be given by injection, and monitoring of response to study drug will include periodic blood draws.

Contact us today at (240)858-4938/ (240)858-4912 or
L-FA@uncommoncures.com to learn more!