Friedreich's Ataxia Research

DTX-216-101

CLINILABS
DRUG DEVELOPMENT CORPORATION

Friedreich's Ataxia Research at Clinilabs Drug Development Corporation

Participants are needed for a Phase 1a, Single Ascending Dose, clinical trial to test an investigational medication (DT-216) in individuals with Friedreich's Ataxia.

Quick Facts:

- Participation time: ~2 months
- Treatment period includes an inpatient stay for 10 nights/11 days
- Compensation up to $2,000 for participants and $1,400 for caregivers
- Travel will be provided at no cost
- Clinical research site is located in Eatontown, NJ

You May Be Eligible if You Are:

- Male or female, 18 to 55 years old (inclusive) at screening
- Genetically confirmed diagnosis of FA with homozygous GAA repeat expansions
- Weigh between 90 and 200lbs (approximately)
- Can sit upright with thighs together and arms crossed without requiring support on more than two sides
- Can perform basic daily care such as feeding yourself and basic personal hygiene with minimal assistance
- Must have completed full COVID-19 vaccination, including booster, at least 4 weeks before treatment
- Willing to avoid use of alcohol & marijuana at least 2 days before check in to Clinilabs

www.clinilabs.com/volunteers
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DT-216 is a new molecule to activate transcription of the FXN gene and restore production of the frataxin protein. More specifically, it is a GeneTAC™ gene targeted chimera small molecule designed to specifically target the GAA repeat expansion mutation and restore FXN gene expression. This is a first-in-human, randomized, double-blind placebo-controlled study designed to evaluate single ascending doses of DT-216 when administered intravenously in adult patients with FA. The key outcomes of this study are to evaluate safety and dose of DT-216.

Key Exclusion Criteria:

- Any current medical condition or medication that, in the opinion of the investigator, puts the participant at risk or precludes participant from completing the study protocol.
- Liver disease or hepatic impairment
- Reduced kidney function
- Clinically significant cardiac disease
- History of drug abuse (any illicit drug use) or a history of alcohol abuse within one year of screening
- Has taken any excluded medication
- Positive result for hepatitis B, hepatitis C or HIV
- Has a history of suicidal behavior within 6 months prior to screening
- Recent or current participation in any interventional trial and recombinant protein or gene replacement therapy trials.

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

Study Schedule & Time Commitment:

The study duration is approximately 6 months. Each participant will be enrolled for up to 8 weeks with a screening period up to 4 weeks before treatment. During the treatment period, there will be an inpatient visit that is 10 nights/11 days at the clinical research unit in Eatontown, NJ. In the unit during your inpatient stay, you will have your own bedroom, an accessible bathroom and area for activities and light workouts. A caregiver is welcome to accompany you during your participation.

Contact us at (212) 994-4567 or Visit clinilabs.com/volunteers