

Friedreich's Ataxia Research at Clinilabs

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CLINILABS
DRUG DEVELOPMENT CORPORATION



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Participants are needed for a Phase 1 research study to test an investigational medication (DT-216) in individuals with Friedreich's Ataxia.

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, Phase 1, study of DT-216. The initial single ascending dose part of the Phase 1 has completed recruitment and we are now beginning the Multiple Ascending Dose (MAD) study. Enrollment will be in cohorts (typically 5-8 people). The first cohort will be enrolled and tested at the first dose, then there will be a safety review, then typically several weeks later the next cohort will be enrolled to test the next dose. In the MAD study subjects will be treated with a once a week dose over 3 weeks, with regular safety assessments after dosing. The study treatment period includes 3 non-consecutive inpatient stays for a total of 10 nights/11 days. Maximum duration of study participation is approximately 10.5 weeks.

You May Be Eligible if You:

- Are male or female between the ages 18 to 55 years old (inclusive) at screening
- Are able to perform basic daily care, such as feeding yourself and personal hygiene, with minimal assistance
- Can sit up with thighs together and arms crossed without requiring support on more than 2 sides
- Have genetically confirmed diagnosis of Friedreich's Ataxia with homozygous GAA repeat expansions

Compensation for time, travel, and participation will be provided for qualified participants. Individuals who are not local to the research facility will receive transportation and accommodations. For more information, please call (212) 994-4567.