

## Call for Patients with Friedreich Ataxia Phase 1/2 Clinical Trial with Elamipretide

The Children's Hospital of Philadelphia (CHOP) is recruiting patients with Friedreich ataxia (FRDA) in the United States for a Phase 1/2 clinical trial studying the safety and effects of Elamipretide in the treatment of advanced stage FRDA symptoms, specifically vision loss and cardiac disease in both adults and older children. This study is being funded by the Friedreich's Ataxia Research Alliance (FARA) and the study drug sponsor is Stealth Biotherapeutics (SBT).

This study will include up to 18 patients to be enrolled at the Children's Hospital of Philadelphia.

### To participate, you must:

- Be 16 years of age or older
- Have genetic confirmation of your FRDA (point mutations allowed)
- Have been diagnosed with FRDA at age 18 years old or younger
- Have vision loss and/or cardiac disease
- Be willing to administer a daily injection (shot)
- Not be actively enrolled in any clinical trial

### In addition, you must NOT:

- Have any clinically relevant medical or surgical condition that could interfere with the administration of study drug, or compromise your safety or well-being
- Be pregnant, planning a pregnancy, or breastfeeding
- Have a history of substance abuse
- Have any other active cause of optic neuropathy or cardiac disease outside of FRDA
- Have a history of an uncontrolled arrhythmia

Additional requirements apply and will be discussed with you by the study doctor.

The study drug and all study-related assessments will be provided at no cost. You may be reimbursed for study-related expenses, such as parking and meals up to \$500 per visit.

Call one of the CHOP study coordinators below to learn more about what study procedures are involved and if you may be eligible to participate.

**Courtney Cheek Park:** Phone: (267) 426-9667; Email: [parkcc@chop.edu](mailto:parkcc@chop.edu)

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Thank you for your ongoing support of clinical research in Friedreich ataxia.