

# Neurological Measures of Progression in Children with Friedreich Ataxia

The Children's Hospital of Philadelphia is recruiting children with Friedreich's Ataxia (FA) to participate in a three year trial to help advance clinical care, research, and therapeutic approaches in FA.

This study is funded by the Food and Drug Administration (FDA) and the Friedreich's Ataxia Research Alliance (FARA). It is a natural history study with the following objectives:

- Collect data twice a year to understand progression of FA and be able to quantify change in progression over time.
- Develop and validate outcome measures (timed walk tests, vision tests) that can be used in future clinical trials
- Make design and implementation of clinical trials more efficient and effective
- Collect biological samples such as cheek swabs and blood to identify and study biomarkers involved with the disease process and quantify the change in these markers over time
- Analyze disease progression in FA affected tissues using innovative and non-invasive measures

## You may be eligible to take part if:

- You have a confirmed diagnosis of Friedreich's ataxia
- You are between the ages of 2 and 18 years of age
- A parent/guardian gives permission for you to participate if you are under 18 years of age

CHOP will be enrolling 60 children to participate, while the University of Florida and the University of California Los Angeles (UCLA) will each enroll 20 children. A select number of participants at CHOP will be able to participate in two additional procedures to further our understanding of how FA affects certain tissues in the body. These procedures are: a Motor Evoked Potentials (MEP) Measurement and an MRI scan.

The study involves biannual visits to CHOP. Once you enroll your travel costs will be reimbursed up to \$500.

If you would like to participate in this study at the Children's Hospital of Philadelphia, please contact the CHOP Friedreich Ataxia Program coordinator below. The Principal Investigator at this site is Dr. David Lynch.

**Coordinator: Cassandra Strawser (215) 590-2314 [strawserc@email.chop.edu](mailto:strawserc@email.chop.edu)**

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