Long-term Follow-up of the NIH Friedreich Ataxia Idebenone Study Cohort

March 2017

The Children's Hospital of Philadelphia (CHOP) is recruiting patients with Friedreich ataxia (FRDA) who previously participated in the 2005-06 phase 2 clinical trial of Idebenone for a new follow-up study. The purpose of the new study is to better understand how the cardiac phenotype of this specific group of patients has changed since the trial. There are no interventions or research visits in this study. The research team will mainly analyze previous medical records. If you (or your child) participated in the 2005-06 NIH clinical trial of Idebenone, you are invited to contact Dr. Lin and her study team to learn more about this research project. This study is funded by the Friedreich Ataxia Research Alliance (FARA) and is taking place only at CHOP.

We are looking for patients with Friedreich ataxia who previously participated in the 2005-06 phase 2 clinical trial of Idebenone (05-N-0245).

To participate, you must:

- Have genetic confirmation of FA
- Be one of the 48 individuals that were subjects in the Idebenone clinical trial that took place at the NIH in 2005-06.

About the study:

- There are NO INTERVENTIONS and NO RESEARCH VISITS involved in this study. You will not need to make a special trip to see the research staff. All activities will be completed by mail, email, facsimile, and/or phone.
- If you consent to this study, the research team will ask for your permission to obtain recent medical records from your physicians including your primary care provider and your cardiologist.
- The research team will use data from the former clinical trial of Idebenone, 05-N-0245.
- If you are a participant in the natural history study of Friedreich ataxia, the cardiac biomarker and cardiac MRI study in Friedreich ataxia, the exercise stress test study in Friedreich ataxia, and/or the cardiomyopathy records study in Friedreich ataxia, the research team will examine data from your most recent study visit.
- The purpose of collecting this information is to compare how you are now with how you were when you enrolled in the clinical trial of Idebenone in 2005-06.
- The study does not provide any direct benefit. By participating, you may make an important contribution to advancing the understanding of FA.

To learn more about the study, contact the below study coordinator:
Felice Wilson from CHOP/ Tel.: (267) 426-2995; Email: wilsonf@email.chop.edu

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

Kimberly Lin, MD
The Children's Hospital of Philadelphia
CHOP Main Building, 8NW86
34th & Civic Center Blvd.
Philadelphia, Pa. 19104