

Call for Patients with Friedreich ataxia Phase II Trial with EPI-743

February 6, 2012

The Children's Hospital of Philadelphia (CHOP) is recruiting patients with Friedreich ataxia (FA) in the United States for a Phase II clinical trial studying the effects of EPI-743 (alpha-tocotrienol quinone, a compound similar to Coenzyme Q10) on visual function in FA. This study is sponsored by Edison Pharmaceuticals, Inc.

CHOP is one of three sites in the United States conducting this trial. The other two sites are at the University of South Florida (Tampa, FL) and the University of California at Los Angeles (CA).

We are looking for patients between the ages of 18 and 45 years who have Friedreich ataxia.

To participate, you must:

- Have genetic confirmation of your FA;
- Be willing to discontinue taking idebenone, coenzyme Q10, and Vitamin E one month before enrolling in the study through the end of the study.

In addition, you must NOT:

- be on certain prescription medications for blood pressure or cholesterol;
- have serious cardiovascular disease that requires medication or a medical device;
- be planning surgery during the study and up to a month afterward;
- be pregnant, planning a pregnancy, or breastfeeding.

About the study:

- Participation in the study is for a maximum of 393 days.
- The study has 2 phases:
 - 6 month blinded treatment phase: participants are randomly assigned to placebo, low dose, or high dose of EPI-743. Those who successfully complete this phase without serious side effects can participate in the
 - 6 month optional extension phase: all participants will be on active agent (low or high dose of EPI-743) though all subjects will be blinded to which dose they are receiving
- In all, you may have a total of 7 one-day visits to CHOP and 2 two-day visits to CHOP. All visits include overnight stays in Philadelphia. You will also have at least 6 phone calls to check for side effects while taking the study drug.
- Once you enroll, your travel costs to CHOP will be reimbursed up to a specific amount as allowed by Edison Pharmaceuticals, Inc.
- You may not directly benefit from participating in this study, but you and other participants may make an important contribution to advancing the understanding and treatment of FA.

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

Karlla W. Brigatti from CHOP/ Tel.: (267) 426-9608; Email: brigatti@email.chop.edu

Lauren Seyer from CHOP/ Tel.: (267) 426-9738; Email: seyerl@email.chop.edu

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