



# PRESS RELEASE

## **EPI-743 Phase 2B Friedreich Ataxia clinical trial to be initiated**

*Friedreich's Ataxia Research Alliance encourages patients to join registry*

Downingtown, PA, November 15, 2012- The Friedreich's Ataxia Research Alliance (FARA) today announced that final preparations are being made for a phase 2B EPI-743 trial in Friedreich's ataxia patients. This study will be a 6-month placebo-controlled multicenter trial, with a six-month extension phase where all subjects will receive EPI-743.

The sponsor of the trial is Edison Pharmaceuticals, Inc. and the study's lead investigator is Theresa Zesiewicz, MD, University of South Florida - Tampa, FL. Co-investigators include Dr. David Lynch at the Children's Hospital of Philadelphia, PA and Dr. Susan Perlman at the University of California, Los Angeles, CA The study is entitled "Safety and Efficacy Study of EPI-743 on Visual Function in Patients with Friedreich's Ataxia." The primary endpoint of the trial is visual function, with secondary endpoints including neurological and neuromuscular function and disease-relevant biomarkers.

Recruitment is expected to begin in the next 45 days. Details concerning study eligibility will be available on the FARA website, [ClinicalTrials.gov](http://ClinicalTrials.gov), and at [health.usf.edu/medicine/neurology/ataxia](http://health.usf.edu/medicine/neurology/ataxia). FARA will also provide detailed information to all Friedreich's ataxia patients in the FARA Patient Registry who may be eligible based on the study criteria.

"We are tremendously excited about the encouraging results Edison Pharma's team has obtained with EPI-743 in mitochondrial disease, and about the promising prospects for this upcoming multicenter Friedreich's ataxia phase 2B study. We strongly encourage all Friedreich's ataxia patients interested in participating to take the steps above to prepare for this important trial," said FARA President Ron Bartek.

### **FARA Registry**

If you have Friedreich's ataxia and are interested in participating in this or other Friedreich's ataxia clinical trials, please make sure you are enrolled in the FARA Patient Registry ([www.curefa.org/registry](http://www.curefa.org/registry)) and that your contact information and other data in the Registry are up-to-date. If you have been tested and genetically confirmed as having Friedreich's ataxia, please have ready a copy of the confirming lab report. If you have not been genetically confirmed with Friedreich's ataxia and are interested in participating in Friedreich's ataxia clinical trials, please consider being tested. If you are a physician that sees Friedreich's ataxia patients, please inform them of this upcoming trial and encourage

them to take these preparatory steps.

**EPI-743**

EPI-743 (alpha-tocotrienol quinone) is an orally absorbed small molecule that targets improvement of mitochondrial function. It has been granted orphan product status in the United States and Europe, and has been administered to over 120 patients with various mitochondrial diseases for over 50,000 treatment days with no serious drug-related adverse events. Recent results have been published on EPI-743 in one mitochondrial disease- Leigh syndrome - with encouraging results. Equally encouraging data have been reported on the sister molecule of EPI-743, EPI-A0001, in a double-blind placebo-controlled trial in Friedreich's ataxia.

**About FA**

Friedreich's ataxia is a rare, degenerative, life-shortening neuro-muscular disorder that affects children and adults, and involves the loss of strength and coordination usually leading to wheelchair use; diminished vision, hearing and speech; scoliosis (curvature of the spine); increased risk of diabetes; and a life-threatening heart condition. There are no FDA-approved treatments.

**About FARA**

The Friedreich's Ataxia Research Alliance (FARA) is a 501(c)(3), non-profit, charitable organization dedicated to accelerating research leading to treatments and a cure for Friedreich's ataxia. [www.CureFA.org](http://www.CureFA.org)

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