USF HEALTH

Making Life Better
through Research, Education & Healthcare.

The USF Ataxia Research Center is pleased to announce that enrollment has opened for a phase 2B EPI-743 trial in Friedreich's ataxia patients ("Safety and Efficacy Study of EPI-743 on Visual Function in Patients with Friedreich's Ataxia."). 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. Enrollment is expected to open soon at other sites including the Children's Hospital of Philadelphia, PA and the University of California, Los Angeles, CA.

The primary endpoint of the trial is visual function, with secondary endpoints including neurological and neuromuscular function and disease-relevant biomarkers.

Patients with Friedreich's ataxia who are interested in participating in the study at the Florida site should contact the USF Ataxia Research Center at 813-974-5909. To be eligible for participation, genetic confirmation of Friedreich's Ataxia is required. Additional eligibility details are available at http://clinicaltrials.gov/ct2/show/NCT01728064.

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. For more information about FA, please visit http://curefa.org/.

Morsani College of Medicine • Department of Neurology
University of South Florida, 12901 Bruce B. Downs Blvd., MDC-55, Tampa, FL 33612
Phone (813) 974-5909 Fax (813) 974-8032