FARA Friedreich's Ataxia Research Alliance

PRESS RELEASE

Edison Pharmaceuticals' EPI-743 Granted FDA Orphan Drug Status for Friedreich's Ataxia

EPI-743 phase 2b Friedreich's ataxia trial fully enrolled

Downingtown, PA, February 4, 2014. Edison Pharmaceuticals today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug status to vatiquinone (EPI-743) for the treatment of Friedreich's ataxia (http://www.prnewswire.com/news-releases/fda-grants-edison-pharmaceuticals-epi-743-orphan-status-for-friedreichs-ataxia-243439351.html). Orphan status brings additional momentum and acceleration to the clinical development of EPI-743 now underway at three sites in the Friedreich's Ataxia Research Alliance (FARA) Collaborative Clinical Research Network.

The FDA's Orphan Drug Designation program was established by the Orphan Drug Act of 1983 to provide further incentives for the biopharmaceuticals industry to develop therapies for rare diseases. The FDA can grant this status to a drug that is being developed specifically to treat a rare condition and that has shown potential benefit for that disorder. Among the incentives is an extension of the period of market exclusivity, partial reimbursement of development costs via tax benefits, waiver of prescription drug user fees, and expedited review of applications for drug approval (within six months of application).

"The FDA's granting of orphan designation underscores the promise that EPI-743 has for the treatment of Friedreich's ataxia," stated Guy Miller, MD, PhD, Chairman & CEO, Edison Pharmaceuticals.

The phase 2b clinical trial of EPI-743 in Friedreich's ataxia began in early 2013 at the University of South Florida, the Children's Hospital of Philadelphia, and the University of California in Los Angeles. The study is now fully enrolled. Details of this trial and of a study of EPI-743 in Friedreich's ataxia patients with a rare point-mutation genotype can be found at www.clinicaltrials.gov.

"This is an important step forward in moving EPI-743 towards approval for the treatment of Friedreich's ataxia," said Ron Bartek, FARA co-founder and President. "FARA has been working closely with Edison, the FDA and National Institutes of Health to advance EPI-743 to this very promising stage of development. We are grateful to Edison, our government partners, the dedicated teams of our Collaborative Clinical Research Network, and to the patients and patient families that have enabled us to recruit the 60 EPI-743 clinical trial participants in a matter of hours."

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☐

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