FARA Friedreich's Ataxia Research Alliance

PRESS RELEASE

FDA Grants Fast Track Status to Edison Pharmaceuticals' EPI-743 for Friedreich's Ataxia

EPI-743 gains key regulatory endorsement

Downingtown, PA, March 17, 2014. Edison Pharmaceuticals announced today that the U.S. Food and Drug Administration (FDA) has granted Fast Track Status to EPI-743 for the treatment of Friedreich's ataxia (http://www.prnewswire.com/news-releases/fda-awards-fast-track-status-to-edison-pharmaceuticals-epi-743-for-friedreichs-ataxia-250558901.html). This status will further accelerate the clinical development of EPI-743 now under way in two phase 2b clinical trials at three sites in the Friedreich's Ataxia Research Alliance (FARA) Collaborative Clinical Research Network.

When the FDA grants the Fast Track designation, the Agency encourages early and more frequent meetings and communications with the drug company throughout the drug development and review process to ensure that any questions and issues are resolved quickly. With this status, the drug may also benefit from "rolling review," which permits the drug company to submit completed portions of its application for approval (New Drug Application) for immediate review by the FDA, instead of waiting for the entire application to be completed. These multiple benefits are intended to result in substantially faster drug approval and earlier treatment access by patients.

The phase 2b clinical trial of EPI-743 in Friedreich's ataxia adults has been under way since early 2013 at the University of South Florida, the Children's Hospital of Philadelphia and the University of California in Los Angeles. Details of this trial and of a study of EPI-743 in Friedreich's ataxia patients with a rare point-mutation genotype can be seen at clinicaltrials.gov.

"Fast Track designation allows Edison to work hand in hand with the FDA, expediting EPI-743 approval," said Guy Miller, MD, PhD, Chairman and CEO of Edison Pharmaceuticals. "In partnership with FARA, physicians/scientists, care providers, and patients/families worldwide, Edison is committed to bringing forward the first FDA approved treatment for Friedriech's ataxia."

"We are excited about the FDA granting Fast Track status to EPI-743 for Friedreich's ataxia and are hopeful that this will hasten the day when Friedreich's patients have their first approved treatment," said Ron Bartek, FARA co-founder and President. "FARA has long been in active partnership with our friends at Edison and, with them, have worked closely with our colleagues at the FDA and the National Institutes of Health in advancing 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 enabled us to recruit the participants for these EPI-743 clinical trials in a matter of a few hours."

About Friedreich's Ataxia
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 currently no effective 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. Please visit our website at http://www.curefa.org.

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