

Edison Press Release

March 17, 2014

Rev. FINAL

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

EPI-743 in two phase 2 development studies for Friedreich's ataxia

Mountain View, California; March 17, 2014. Edison Pharmaceuticals today announced that the United States Food and Drug Administration granted Fast Track designation to EPI-743, the company's lead drug, for the treatment of Friedreich's ataxia. EPI-743 is being developed for pediatric and adult mitochondrial disease, including Friedreich's ataxia.

EPI-743 has successfully completed phase 1 and multiple phase 2 studies. In these studies, EPI-743 has been demonstrated to be safe and well tolerated. Two phase 2 trials in patients with Friedreich's ataxia are ongoing. A phase 2B randomized double-blind placebo-controlled trial in adults with Friedreich's ataxia is fully enrolled. It is expected to be completed in Q3, 2014. In addition, EPI-743 is being evaluated in a rare Friedreich's ataxia genetic subtype— patients with a point mutation in the gene encoding frataxin. This single-arm subject-controlled trial is also fully enrolled. Details of these trials can be found on clinicaltrials.gov.

“Fast Track designation will facilitate Edison's clinical development of EPI-743 for patients with Friedreich's ataxia,” said Guy Miller, MD, PhD, Chairman and CEO of Edison Pharmaceuticals. “We are fully committed to delivering the first approved drug for this highly debilitating and lethal disease for which there are no FDA-approved therapies.”

The FDA's Fast Track program is designed to facilitate the development of drugs that have demonstrated potential to treat diseases that are serious, life threatening, and for which there is an unmet medical need. Fast Track provides a number of benefits, including the ability to meet and communicate more frequently with the FDA to discuss drug development plans, as well as eligibility for accelerated approval. Drugs with Fast Track designation may also receive “rolling review” from the FDA. This permits a drug company to submit completed portions of a New Drug Application (NDA) for immediate review before the entire application is completed.

“We greet with tremendous enthusiasm the FDA's issuance of Fast Track designation to EPI-743 for Edison's Friedreich's ataxia development program,” stated Mr. Ron Bartek, President, Friedreich's Ataxia Research Alliance (FARA) and Chairman of the Board of the National Organization for Rare Disorders. “This demonstrates the FDA's commitment to the accelerated development of promising drugs for diseases which have no treatments.”

The FDA has previously granted Orphan designation status to EPI-743 for the treatment of Friedreich's ataxia.

The Edison logo consists of the word "edison" in a lowercase, blue, sans-serif font. The letters are closely spaced and have a clean, modern appearance.

Edison Press Release

March 17, 2014

Rev. FINAL

Friedreich's Ataxia

Friedreich's ataxia is an autosomal recessive nuclear DNA inherited mitochondrial disease, which affects an estimated 1 in 50,000 individuals in the United States and Europe. Friedreich's ataxia is caused by a defect in the gene frataxin. Frataxin encodes a 210 amino acid protein that participates in iron-sulfur (Fe-S) cluster protein assembly. Because the majority of these Fe-S cluster proteins are localized in the respiratory chain in the mitochondria, patients with Friedreich's ataxia present with "energy failure" symptoms, including ataxia, muscle weakness, heart failure, diabetes, and visual, speech and hearing deficiencies. Friedreich's ataxia is a highly debilitating and life-shortening disease and is a member of a larger family of diseases -- called mitochondrial disease -- that share as a common biochemical mechanism defects in cellular energy metabolism. There are no FDA-approved treatments for Friedreich's ataxia.

EPI-743

EPI-743 is an orally bioavailable small molecule being developed by Edison Pharmaceuticals for the treatment of Friedreich's ataxia and other inherited mitochondrial diseases. EPI-743 is a member of the para-benzoquinone class of drugs. Through a redox-based mechanism, EPI-743 augments endogenous glutathione biosynthesis-- essential for the control of oxidative stress. Double-blind placebo-controlled trials of EPI-743 are ongoing for the following indications: Friedreich's ataxia, Leigh syndrome, Cobalamin C defect, and Undiagnosed Disorders of Oxidation-Reduction.

Edison Pharmaceuticals

Edison Pharmaceuticals is a specialty pharmaceutical company dedicated to developing treatments for children and adults with mitochondrial diseases.

Edison Information

Edison Pharmaceuticals, Inc.

350 North Bernardo Avenue

Mountain View, CA 94043

info@edisonpharma.com

edisonpharma.com

###