

Edison Pharmaceuticals Announces Results of EPI-A0001 Phase 2A Double Blind Placebo Controlled 28-day Clinical Trial in the Mitochondrial Disease– Friedreich’s Ataxia

EPI-A0001 improves neurological outcome endpoint

Mountain View, California. June 10, 2011. Edison Pharmaceuticals, Inc. announced today preliminary results obtained on a 28-day phase 2A clinical trial in Friedreich’s ataxia. While the primary endpoint of glucose disposition index (a measure of glucose handling by the body) did not show statistically significant improvement, EPI-A0001 did significantly improve neurological function as assessed by the Friedreich’s Ataxia Rating Scale (FARS). This double-blind placebo-controlled trial consisted of three arms: placebo, low dose, and high dose EPI-A0001. The improvement in the FARS was statistically significant in both the high and low dose groups in comparison to placebo. There were no differences in the rates of drug-related adverse events between the placebo group and each of the drug-treated groups.

EPI-A0001 has received orphan designation from FDA. On February 25, 2010, the FDA granted Fast Track designation to EPI-A0001 for Friedreich’s ataxia.

The results of this study are preliminary. Additional clinical trials will be required to determine the safety and effectiveness of EPI-A0001 for the proposed indication.

Treatment Information

Edison Pharmaceuticals

– EdisonPharma.com

Friedreich’s Ataxia Research Alliance

– CureFA.org

GoFAR

– FAGoFAR.org

United States Food and Drug Administration

– Clinicaltrials.gov

About Friedreich’s Ataxia

Friedreich’s ataxia is an autosomal nDNA inherited mitochondrial disease, affecting an estimated 50,000 individuals worldwide. Friedreich’s ataxia is caused by a defect in the gene frataxin, which is a 210 amino acid protein that participates in iron-sulfur (Fe-S) cluster protein assembly. As the majority of these Fe-S cluster proteins are localized to the respiratory chain in the mitochondria, patients with Friedreich’s ataxia present with “energy failure” symptoms including heart failure, ataxia, diabetes, and visual and hearing deficiencies. Friedreich’s ataxia is a highly debilitating and life-shortening disease. There are no FDA-approved drugs for Friedreich’s ataxia.

Edison Pharmaceuticals

Edison Pharmaceuticals is a patient- and physician-founded company devoted to developing new medicines to transform the lives of those diagnosed with rare and neglected diseases. Currently, the company is focused on



inherited mitochondrial diseases for which there are no approved drugs. These conditions are life-threatening and highly debilitating. Edison's specialized skill set spans the discovery, development, and translation of redox-based drugs.

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