

Edison Press Release

June 9, 2014

FINAL

FDA Grants Edison Pharmaceuticals' EPI-743 Orphan Status for Leigh Syndrome

EPI-743 in phase 2B development for Leigh syndrome

Mountain View, California; June 9, 2014. Edison Pharmaceuticals today announced that the FDA has granted Orphan Drug status to vatiquinone for the treatment of Leigh syndrome.

Vatiquinone is the International Nonproprietary Name (INN) for Edison's EPI-743. The INN is a unique international name issued by the World Health Organization, which is used to identify the active pharmacological ingredient in a drug. This is also known as the generic name.

EPI-743 is currently in phase 2B/3 development for the treatment of Leigh syndrome. A phase 2B randomized double-blind placebo-controlled trial in children with Leigh syndrome is fully enrolled in the United States, and a phase 2B/3 trial of EPI-743, which is being conducted in conjunction with Dainippon Sumitomo Pharma Co, Ltd, is ongoing in Japan.

Orphan designation was established as part of the Orphan Drug Act which was passed by the US Congress in 1983 to encourage the development of drugs for the treatment of rare, orphan diseases. The FDA grants orphan status to drugs that are being developed specifically to treat a rare condition and have shown potential benefit for the indication. Orphan designation affords several advantages including a more expedited drug approval process and an extended period of market exclusivity.

Edison Pharmaceuticals has previously received Orphan Designation from the FDA for the treatment of inherited respiratory chain diseases of the mitochondria and Friedreich's ataxia, and from the European Medicines Agency Committee on Orphan Products and Japanese Ministry of Health, Labor and Welfare for the treatment of Leigh syndrome.

Leigh Syndrome

Leigh syndrome is an inherited lethal, progressive, predominately pediatric, neuromuscular disorder for which there are no approved treatments. It is the most common pediatric inherited mitochondrial disease. Initially described in 1951, the hallmarks of the disease include bilateral necrosis (death) of central nervous system regions responsible for the control of breathing and other neurologic functions. Leigh syndrome belongs to a large family of disorders identified as "mitochondrial disease." The disorders share as a common biochemical feature defects in cellular energy metabolism.

EPI-743

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

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EPI-743 is an orally bioavailable small molecule being developed by Edison Pharmaceuticals for inherited mitochondrial diseases, and is a member of the para-benzoquinone class of drugs. The mechanism of action of EPI-743 involves: i) augmenting the synthesis of glutathione; ii) optimizing metabolic control; iii) enhancing the expression of genetic elements critical for cellular management of oxidative stress; and iv) acting at the mitochondria to regulate electron transport. The utility of EPI-743 is also being explored in other inherited respiratory chain disorders.

Edison Pharmaceuticals

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

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