

May 20, 2014

Edison Pharmaceuticals' EPI-743 Granted Orphan Designation for Leigh Syndrome in Japan

EPI-743 in phase 2B/3 development for Leigh syndrome

Mountain View, California; May 2014. Edison Pharmaceuticals today announced that the Japanese Ministry of Health, Labor and Welfare (MHLW) has granted Orphan Designation to EPI-743 for the treatment of Leigh syndrome.

EPI-743 is currently in phase 2B 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.

The Japanese MHLW grants orphan status to drugs that are being developed specifically to treat rare conditions that are serious and difficult to treat and that have shown potential benefit for the indication. Orphan designation in Japan affords several advantages including a more expedited drug approval process as well as a priority consultation with MHLW, the Japanese Pharmaceuticals and Medical Devices Agency (PMDA), and the Japanese National Institute of Biomedical Innovation (NIBIO).

Edison Pharmaceuticals has previously received Orphan Designation from the United States Food and Drug Administration for the treatment of inherited respiratory chain diseases of the mitochondria and from the European Medicines Agency Committee on Orphan Products 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. 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." These disorders share as a common biochemical feature defects in cellular energy metabolism.

EPI-743

EPI-743 is an orally bioavailable small molecule being developed by Edison Pharmaceuticals for the treatment of Leigh syndrome and other inherited mitochondrial diseases. EPI-743 is a



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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.

Edison Pharmaceuticals

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

Dainippon Sumitomo Pharma Co., Ltd.

Dainippon Sumitomo Pharma Co., Ltd. (DSP) is a multi-billion dollar, top-ten listed pharmaceutical company in Japan with a diverse portfolio of pharmaceutical products. DSP aims to produce innovative pharmaceutical products in the psychiatry and neurology, which is designated as a key therapeutic area, as well as oncology. DSP has about 7,000 employees worldwide. Additional information about DSP is available through its corporate website at www.ds-pharma.com.

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