

Edison Pharmaceuticals Announces FDA Grants EPI-743 Orphan Drug Designation

Designation covers inherited respiratory chain diseases of the mitochondria

Mountain View, California. June 8, 2011. Edison Pharmaceuticals, Inc. announced today the United States Food and Drug Administration has granted orphan drug designation to EPI-743 for treatment of inherited mitochondrial respiratory chain diseases. Today, these mitochondrial diseases affect an estimated 60,000 individuals.

The Orphan Drug Act, passed in 1982, provides incentives for companies to pursue treatments for diseases affecting fewer than 200,000 individuals in the United States. Such designation provides an accelerated review and a seven-year period of exclusivity upon FDA approval.

The FDA granted orphan drug designation to Edison's EPI-743 based on review of the application, which included clinical and pre-clinical data demonstrating a favorable efficacy and safety profile.

About Inherited Mitochondrial Disease

Inherited mitochondrial diseases are genetic disorders that share as a common link defects in how cells make and regulate energy. They can arise through defects in genes located in the nucleus or mitochondria. An estimated 2,000 defects in nuclear DNA and 200 defects in mitochondrial DNA have been identified that are pathogenic¹.

Inherited mitochondrial diseases are clinically diverse and poorly understood. They can affect virtually any organ system in the body. As the brain and muscle tissue use an extraordinary amount of energy, they are dramatically affected by these disorders. Mitochondrial diseases often present with central nervous system manifestations, but they also result in a variety of other significant clinical signs that include diabetes, heart failure, liver failure, deafness, blindness, renal insufficiency, and muscle weakness and fatigue.

The incidence of mitochondrial disease is estimated at 1-5 in 10,000. However, this number may underestimate the true number of people with mitochondrial disease. While new genetic screening tools have made detection of mitochondrial disease possible, the majority of individuals with a clinical diagnosis of mitochondrial disease may not have a confirmatory genetic diagnosis. Some estimates place the number of clinically diagnosed mitochondrial disease patients without a genetic diagnosis— so called mitochondrial syndromes— at 10 times the incidence of genetically defined disease.

Science today is at the frontier of mitochondrial medicine. Certain diseases that previously lacked a known cause are now being defined as mitochondrial in etiology. It is possible that in the future mitochondrial disease will be implicated in additional diseases, such as in the biology of cancer.

Edison Pharmaceuticals

Edison Pharmaceuticals is a patient- and physician-founded company devoted to developing new medicines that will 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

¹ http://www.ninds.nih.gov/news_and_events/proceedings/20090629_mitochondrial.htm

and highly debilitating. Edison's specialized skill set spans the discovery, development, and translation of redox-based drugs.

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