Edison Pharmaceuticals Announces Initiation of EPI-743 Pearson Syndrome Clinical Trial

*FDA approves IND for clinical trial in ultra rare orphan mitochondrial disease*

Mountain View, California; March 17, 2014. Edison Pharmaceuticals today announced the initiation of a phase 2 study entitled “A Phase 2 Safety and Efficacy Study of EPI-743 (Vincerinone\textsuperscript{TM}) in Children with Pearson Syndrome.” The Investigative New Drug application (IND) was approved by the Food and Drug Administration, Office of Hematology and Oncology Products.

The trial is a subject-controlled study design lasting 12 months in which all subjects will receive EPI-743. The primary endpoint of the study is the incidence of episodes of sepsis, metabolic crisis, and hepatic failure. Secondary endpoints include transfusion avoidance and other disease-relevant endocrine and neurological outcome assessments. Given the rarity of Pearson syndrome, the study is a single arm design, and is being conducted at multiple sites worldwide.

Historically, mitochondrial disease has been described as a group of neuromuscular diseases. This description connotes the energy derangements associated with brain and muscle function. However, today our knowledge of both the molecular and genetic basis of the diseases has significantly grown. It is now established that genetic defects in either nuclear or mitochondrial derived DNA are responsible for a myriad of clinical “mitochondrial” diseases that can affect virtually every organ system. Pearson syndrome is one such example.

Pearson syndrome has an estimated prevalence of less than 1:1,000,000. It is typically diagnosed in infancy, though it can be diagnosed in neonates. Its clinical hallmarks are transfusion-dependent anemia, neutropenia, and pancreatic dysfunction. Given the notable hematologic derangements associated with the syndrome, Edison has submitted the Pearson syndrome IND to the Office of Hematology and Oncology Products. This allows Edison to utilize the division’s expertise in the development of drugs for diseases with significant hematological manifestations.

“Pearson syndrome is a very rare mitochondrial disease with a devastating clinical outcome,” stated Mathew Klein, MD, FACS, Chief Medical Officer, Edison Pharmaceuticals. “We are working with our clinical investigator team worldwide to accelerate enrollment in this trial and to systematically explore whether EPI-743 can offer benefit for this patient population.”

The FDA has previously granted orphan status to EPI-743 for the treatment of inherited respiratory chain diseases, as well as for Friedreich’s ataxia. Edison is currently conducting a number of phase 2A and 2B trials in various mitochondrial disease indications. A complete list of ongoing trials can be found on www.clinicaltrials.gov.
Pearson syndrome

Pearson syndrome is an ultra-rare, fatal mitochondrial disease characterized by sideroblastic anemia, pancytopenia and pancreatic dysfunction. Pearson syndrome results from a mitochondrial deletion, spanning a range of mitochondrial genes. Hematologic features are often present at birth and include severe, transfusion-dependent macrocytic anemia as well as neutropenia and thrombocytopenia. There is no treatment for Pearson syndrome and most patients die in infancy or early childhood secondary to metabolic disorders or infections. A subset of patients survive past age three and develop a neurological syndrome– resembling Kearns-Sayre syndrome– characterized by ophthalmologic and neuromuscular impairments.

EPI-743

EPI-743 is an orally bioavailable small molecule being developed by Edison Pharmaceuticals for the treatment of children and adults with 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, which is essential for the control of oxidative stress. EPI-743 is in phase 2 clinical development for the treatment of inherited respiratory chain disorders. Double-blind placebo-controlled trials 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.

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