Edison News Release
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Edison Pharmaceuticals Announces Positive Results from Phase 2 Placebo-Controlled EPI-743 Leigh Syndrome Trial

EPI-743 demonstrates marked improvement in disease morbidity endpoints

Mountain View, California; June 23, 2016. Edison Pharmaceuticals today announced positive results from the long-term extension trial of the EPI-743 placebo-controlled Leigh syndrome study being conducted in the United States.

In an analysis of the "Long-Term Safety and Efficacy Evaluation of EPI-743 in Children with Leigh Syndrome" (NCT02352896) study, EPI-743 treatment resulted in a marked decrease in the number of pediatric subjects being hospitalized and the overall number of hospitalizations - two key aspects of disease morbidity - over a 2.5-year period. Standard disease metrics of morbidity including hospitalization and serious adverse events were pre-specified as endpoints to capture drug effect given patient heterogeneity and the absence of validated endpoints.

The US EPI-743 Leigh syndrome trial included a six-month placebo-controlled phase, followed by a 30-month extension phase to assess long-term drug safety and impact on disease morbidity. During the extension phase, all subjects received treatment with EPI-743. The study enrolled 35 subjects at four study sites: Lucile Packard Children's Hospital, Stanford University Medical Center – Palo Alto, California; Akron Children's Hospital – Akron, Ohio; Seattle Children's Hospital – Seattle, Washington; and Texas Children's Hospital, Baylor University – Houston, Texas.

In the initial six-month placebo-controlled phase (NCT01721733), treatment with EPI-743 was associated with fewer subjects requiring hospitalizations and experiencing serious adverse events as compared with subjects who received placebo (11.8% vs 42.8%). The recently completed analysis included subject data from study entry to month 30. Changes in number of hospitalizations and serious adverse events over time were analyzed in six-month blocks following study enrollment. Over the long-term period of EPI-743 treatment, there was a decrease in number of hospitalizations (from 17 to 5), serious adverse events (from 23 to 8), and number of individual subjects hospitalized (from 8 to 4). In addition to the effect on disease morbidity, the 30-month analysis also demonstrated that EPI-743 was safe and well-tolerated with no drug-related serious adverse events or toxicities.

"These results confirm the treatment benefits we have observed in the EPI-743 compassionate use program—that children and adults with mitochondrial disease treated with EPI-743 tend to have symptomatic improvement and fewer intercurrent illnesses and hospitalizations—a significant departure from the understood natural history of mitochondrial disease," said Bruce H. Cohen, MD, FAAN, Professor of Pediatrics and Director of NeuroDevelopmental Science Center, Akron Children's Hospital and study Principal Investigator. "The reduction in number of subjects being hospitalized over a two-year period of time provides objective and clear evidence of EPI-743 impact on disease progression."

"These data provide objective and quantitative evidence of EPI-743 treatment effect on an important aspect of Leigh syndrome– disease morbidity," said Matthew Klein, MD, Edison's Chief Medical Officer. "In addition, these data also highlight the need to conduct clinical studies of sufficient duration to capture unambiguous drug effect on neuromuscular diseases that are heterogeneous, rare, and complex, such as Leigh syndrome."

EPI-743 has previously been awarded orphan designation for Leigh syndrome in the United States, Japan, and European Union.

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 death of central nervous system regions responsible for the control of breathing and coordination of movement. Leigh syndrome is a member of a larger family of diseases called mitochondrial disease, which share as a common biochemical mechanism 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 targets oxidoreductase enzymes essential for redox control of metabolism and sulfur-hydride antioxidant synthesis.

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