

Teleconference 6/16/11
Edison Pharmaceuticals
EPI-743

Introduction

Dr. Guy Miller is the founder of Edison Pharmaceuticals and has been dedicated to researching treatments for mitochondrial disease. He is joined by Dr. Greg Enns, an associate professor of pediatrics at Stanford University, to talk about the exciting new drug EPI-743. This drug is in its early stages of development, but has exhibited some promising effects in patients with severe mitochondrial diseases.

What is EPI-743?

EPI-743 is a molecule that is somewhat similar in structure to CoQ10, but it also has some basic chemical differences. For instance, CoQ10 has a long “tail” made of long chain fatty acids, and the “tail” of EPI-743 is significantly shorter. Dr. Miller and Dr. Enns report that there is a correlation between those who have had a positive response to CoQ10 and those who have a positive response to EPI-743, although they have also seen patients respond well to EPI-743 who have not responded to CoQ10.

The Breaking News About EPI-743

Two years ago, Edison was authorized by the United States Food and Drug Administration (FDA) to treat mitochondrial disease patients with EPI-743 under certain parameters. The patients treated had to meet two criteria: (1) they must have a genetically confirmed mitochondrial disease and (2) they must be within 90 days of end-of-life care. This process is *not* a clinical trial. It is an “expanded access investigational new drug protocol”. The intention of this protocol is not to gain drug approval, but instead to provide treatment to a patient whose physician finds the risk-benefit ratio to be warranted.

To date, Edison has treated 44 patients worldwide. Of these, the data for 7 patients has been analyzed and released. The FDA has recently allowed Edison to publicize information about EPI-743 and openly discuss the drug. They have also lifted the requirement that the patient must have a genetically confirmed mitochondrial disease. Although the patient must still be within 90 days of end-of-life care, Edison is able to treat patients who are “syndromic”.

What are the results of EPI-743 so far?

Although the data for only 7 patients has been analyzed so far, Dr. Miller and Dr. Enns are able to report some qualitative and quantitative results. In the analysis process, the “Newcastle Mitochondrial Disease Scale Assessment” is used. This assessment has four sections. In these patients, there have not been any significant changes in the first three sections (current function, degree of systemic involvement, and current clinical assessment). However, the doctors state that this assessment scale may not be sensitive enough for the 13 week time scale in which

the data was gathered. There were observable improvements in the fourth section (quality of life) of the assessment.

Outside of this official assessment, the doctors report that they have seen improvements in many patients, and some stabilization in patients whose health was rapidly declining. Symptoms such as strength, coordination, focus, attention, and sleep patterns have improved in many patients. The doctors have also used a SPECT scan to study images of patients' brains. By using a special dye, they have noted an increase in mitochondrial function in many parts of the brain after treatment with EPI-743. It is important to note that this drug is in its very early stages, and therefore it is so far difficult to generalize its effects

What types of mitochondrial disease has EPI-743 had an effect on?

EPI-743 has been administered to children and adults with Leigh's Disease, MERRF, MELAS, Kearns Sayre Syndrome, and Alpers' Disease, among others. This drug is still in its experimental stages, so it is impossible to say who can benefit the most.

Are there any side effects?

No significant drug-related effects have been documented with EPI-743.

What's next for EPI-743?

The ultimate goal for this drug is to make it available to treat anyone with mitochondrial disease. The data from the initial patients will need to be analyzed over the next few weeks. After that, Edison will meet with the FDA to agree upon protocols for clinical trials, which will commence as soon as possible. MitoAction will be keeping up to date with all of the latest news about EPI-743 here:

www.mitoaction.org/epi743