UNITED MITOCHONDRIAL DISEASE FOUNDATION®

HOPE. ENERGY. LIFE.

THE UMDF IS PROVIDING YOU THIS INFORMATION ABOUT THE FIRST EVER CLINICAL TRIAL FOR THE TREATMEND OF LEIGH SYNDROME. PLEASE READ THE INFORMATION BELOW AND PARTICIPATE IN THE VERY IMPORTANT TRIAL. ALL STUDY EXPENSES ARE COVERED INCLUIDING TRAVEL.

Dear UMDF Members:

We are actively recruiting children ages 1-12 years with Leigh syndrome for a trial of EPI-743. This is a randomized, double-blinded, and placebo-controlled trial using a novel drug, alpha-trocotrienol quinone (EPI-743).

During the first six months of the trial children are randomized to receive the active agent or placebo. During the next six months, those on placebo are randomized to one of two dosages of the active agent (and those on the active agent remain on the active agent). Neither the patient nor doctor will know during the first six months if the child is on EPI-743 or a placebo. A computer performs the randomization.

The Italian experience in children with genetically confirmed Leigh syndrome treated in an open label study is published and you can the basic information about this study by visiting http://www.ncbi.nlm.nih.gov/pubmed/23010433

Improvement in function was noted in 10 consecutive patients.

We are looking for candidates that can travel to one of the four centers. All study expenses are covered. We ask that you consider this trial. If you have any questions please feel free to contact any of us.

The major eligibility requirements include:
-Age 1-12 years
-Clinical diagnosis of Leigh Syndrome
-Confirmed Genetic mutation known to cause Leigh Syndrome
-MRI confirms Leigh syndrome
-Progression within the last 12 months but stable respiratory status and never trached

Full information about this trial with institutional contact information, inclusion and exclusion criteria as well as outcome measures is available at:

http://clinicaltrials.gov/ct2/show/NCT01721733?term=EPI-743&rank=2

Sincerely yours,

Bruce H. Cohen - Akron Children's Hospital
Greg Enns - Lucille Packard Children's Hospital - Stanford
Fernado Scaglia- Texas Children's Hospital -Baylor
Russ Saneto - Seattle Children's Hospital