

RESEARCH TRIAL FOR PATIENTS WITH FRIEDREICH'S ATAXIA

Safety and Efficacy Study of EPI-743 (Vincerinone™) on Visual Function in Patients with Friedreich's Ataxia

What is the Study?

The purpose of this study is to determine whether EPI-743 is effective in improving vision in Friedreich's ataxia. Research studies indicate that there is a problem with energy production in the nervous system of people with Friedreich's ataxia, which may lead to loss of vision. The drug EPI-743 could potentially prevent or reverse this vision loss.

This is a multisite clinical study funded by the Edison Pharmaceuticals, Inc.. Dr. Susan Perlman is the Principle Investigator at University of California, Los Angeles. Up to 20 study subjects will be enrolled at UCLA.

What is EPI-743?

EPI-743 is an Investigational drug. Investigational means that the study drug has not been approved by the US Food and Drug Administration (FDA) and its use is experimental. EPI-743 is under development for the treatment of symptoms associated with inherited mitochondrial diseases, including Friedreich's Ataxia. The mitochondria are the parts of the cells in the body that convert food energy into fuel for cell function. Mitochondrial diseases are a group of conditions in which the cells of the body are not able to use energy effectively. EPI-743 is chemically similar to the commercially available, vitamin-like substance Coenzyme Q10, but is thought to be more potent in helping cells use energy.

Who is Eligible?

Male or female volunteers, aged 18 to 45 years, diagnosed with Friedreich's Ataxia with a positive gene test and in otherwise good health.

How Long Does the Study Last?

This study involves 8 clinic visits and 6 phone calls over 13 months. Blood will be drawn and an electrocardiogram (ECG) performed for safety checks 8 times over the 13 month period of the study. An echocardiogram (ECHO) will be done at the beginning, middle, and end of the study.

What is Involved in Participation?

If you qualify for participation, you will be randomly selected to receive either low or high dose study medication or placebo (identical capsule with no active drug in it) every day for 6 months. After 6 months, if you have been on placebo, you will be randomly re-assigned to either low or high dose study drug for the final 6 months of the study, so everyone in the study will get "real drug" for at least 6 months. At each visit, you will see a doctor, be asked about current illnesses and changes in medications, have your General, Neurological, and Visual exam checked, and complete surveys about how you are feeling. The length of each visit will be from 2 ½ up to 7 hours, depending on the number of tests done at each visit. Two visits will require an overnight stay for blood drawing the next day. There is no cost for participation, and you will receive reimbursement for travel, lodging, and food expenses up to \$300 per clinic visit with an additional \$200 for the two overnight visits.

What Side Effects have been Observed in Connection with EPI-743?

No serious side effects have been confirmed with use of EPI-743.

Where Can I Get More Information?

To determine if you or a family member is eligible to participate in this study and for more information, please contact Dr. Susan Perlman or Maria Casado at (310)-206-8153.

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