

Call for Patients with Friedreich's Ataxia Phase IIa Trial with A0001

December 8, 2009

Dear FARA Patient Registry Participant:

We are pleased to announce today that The Children's Hospital of Philadelphia (CHOP) is recruiting patients with Friedreich's Ataxia (FA) in the United States for a Phase II clinical research trial examining the effects of A0001 (alpha-tocopherolquinone, a compound similar to Coenzyme Q10) on mitochondrial function, specifically on the way glucose is converted to energy, which is often impaired in individuals with FA. This study is sponsored by Penwest Pharmaceutical Co.

We are looking for patients between the ages of 18 and 60 years who have a genetic diagnosis of Friedreich's Ataxia.

To participate, you must:

- Have a genetically confirmed diagnosis of FA;
- Have no personal history of diabetes BUT you must have a positive oral glucose tolerance test (as determined by specific testing at your research screening visit);
- Be able to eat foods containing milk and wheat products;
- Not be taking anticoagulants (blood thinners) or statins (medications to help lower cholesterol);
- Not be planning any surgery during the study and up to a month after you have finished the last dose of study medication;
- Not be pregnant, planning a pregnancy during the study and for a month afterward, or breastfeeding.

About the study:

- Participation in the study is for a maximum of 56 days;
- You will have a total of 4 visits to CHOP (including two overnight stays in a hotel near the hospital) and 7 home-based visits, specifically for blood draws. At your first visit, you will have a blood test to determine if you are eligible to participate in the study. If you are eligible to participate, you will be treated with study medication or placebo (an inactive pill with no drug in it) beginning at your baseline visit a few weeks later and continuing to your last visit 28 days later.
- There are 3 possible treatments: 2 different doses of A0001 or a placebo.
- Once you enroll, your travel costs to CHOP will be fully covered, including meals and accommodations. You will be responsible for any travel costs incurred for your screening visit.

The purpose of the study is to investigate the effect of A0001 on mitochondrial function in subjects with FA. You will not directly benefit from participating in this study, but you and other study participants may make an important contribution to advancing the understanding and treatment of Friedreich ataxia.

For more information about this study and to find out how to take part, please contact one of the following:

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Thank you for your ongoing support of FARA and clinical research in Friedreich's Ataxia.

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Expiration Date: N/A