Call for Patients with Friedreich ataxia
Phase 2 Trial with RTA 408

October 2014

The Children's Hospital of Philadelphia (CHOP) is recruiting patients with Friedreich ataxia (FA) in the United States for a Phase 2 clinical trial studying the safety and effects of RTA 408 (a semi-synthetic triterpenoid, a Nrf2 activator with antioxidant properties) in FA. This study is sponsored by Reata Pharmaceuticals, Inc.

This study will include up to 52 patients, to be enrolled at one of several sites around the United States including the Children's Hospital of Philadelphia.

We are looking for patients between the ages of 18 and 40 years who have Friedreich ataxia.

To participate, you must:

- Have genetic confirmation of your FA;
- Be willing to maintain a consistent exercise routine and stable medication doses throughout the study;
- Be willing to discontinue taking all antioxidant supplements and vitamins, or any other medication intended to treat Friedreich's ataxia, before beginning this study drug and throughout your participation in the study;
- Use an acceptable form of contraception throughout the study.

In addition, you must NOT:

- Have any clinically relevant medical or surgical condition that could interfere with the administration of study drug, or compromise your safety or well-being
- Be pregnant, planning a pregnancy, or breastfeeding.

About the study:

- Participation in the study is for up to 5 months, including 12 weeks of treatment.
- The study has 3 different study groups:
  - Group 1: dose-escalation, placebo-controlled: participants are assigned to receive 2.5 mg or placebo of RTA 408 for 2 weeks, and dose is increased to 5 mg RTA 408 or placebo for remaining 10 weeks.
  - Group 2: 10 mg dose, placebo-controlled: participants are assigned to receive 10 mg RTA 408 or placebo for 12 weeks. Groups 1 and 2 will not include patients under 18 years old.
  - Group 3: parallel doses, placebo-controlled: participants are assigned to receive placebo, 2.5 mg, or 10 mg of RTA 408 for 12 weeks.
- All individuals in the study would be asked to complete 8 visits to CHOP, including one 2-day visit, and several phone calls. For one visit, there is an option for a home health nurse to perform study procedures at your home instead of traveling to Philadelphia. The first five visits, including the optional home visit, take place within the first six weeks of the study. Participation in more than one study group is not permitted.
- Once you enroll, your travel costs to CHOP may be reimbursed up to a specific amount as allowed by Reata Pharmaceuticals, Inc.
- You may not directly benefit from participating in this study, but you and other participants may make an important contribution to advancing the understanding and treatment of FA.

Call one of the study coordinators below to learn more about what study procedures are involved and if you may be eligible to participate.

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Thank you for your ongoing support of clinical research in Friedreich ataxia.