

Call for Patients with Friedreich's ataxia Phase 2 Trial with TAK-831

November 2017

The Children's Hospital of Philadelphia is recruiting patients with Friedreich's ataxia (FA) in the United States for a Phase 2 clinical trial studying the safety and effects of TAK-831 in FA. This study is sponsored by Takeda Pharmaceuticals.

This study includes several sites around the United States, including the Children's Hospital of Philadelphia.

We are looking for patients between the ages of 18 and 55 years who have Friedreich ataxia.

To participate, you must:

- Have genetic confirmation of your FA;
- Be able to complete the 9-hole peg test in under 150 seconds (2.5 minutes)
- Be willing to keep your medications stable throughout 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.
- Approximately 65 patients will be enrolled across all sites
- You will be given either placebo, a low dose of TAK-831, or a high dose of TAK-831 and will remain on the same dose during all 12 weeks of treatment. There is a 60% chance of receiving active medication.
- All individuals in the study would be asked to complete 5 visits to the Children's Hospital of Philadelphia, including one 2-day visit at the beginning of treatment, and several phone calls.
- You will be asked to attend clinic in the early morning for most of the study visits
- Once you enroll, your travel costs to the Children's Hospital of Philadelphia will be reimbursed up to a certain amount as allowed by Takeda Pharmaceuticals.
- 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 the study coordinators below to learn more about what study procedures are involved and if you may be eligible to participate.

McKenzie Wells from CHOP/ Tel.: (267) 426-9608; Email: wellsm@email.chop.edu

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