

**Phase III, double-blind, placebo-controlled study of interferon gamma-1b (ACTIMMUNE<sup>®</sup>) for the treatment of Friedreich's Ataxia**

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The Children's Hospital of Philadelphia (CHOP) is recruiting children and young adults with Friedreich's Ataxia (FA) for a Phase III, double-blind, placebo-controlled clinical trial studying the safety, efficacy, and pharmacokinetics (PK) of IFN- $\gamma$  (interferon gamma-1b, ACTIMMUNE) in FA. This study is funded by Horizon Pharma Ireland, Ltd. and will include approximately 90 patients at four sites in the United States.

***We are looking for patients between the ages of 10 and 25 years who have FA.***

**To participate, you must:**

- Have genetic confirmation of your FA (must have two expanded GAA repeats)
- Be able to tolerate injections under the skin
- Be able to walk 25 feet with or without the use of an assisted device
- Be willing to comply with all study procedures and scheduled protocol visits

**In addition, you must NOT:**

- Have any clinically relevant medical condition that could interfere with the administration of study drug, or compromise your safety or well-being, including clinically significant cardiac, liver, or kidney disease
- Have a history of substance abuse
- Be pregnant, planning a pregnancy, or breastfeeding
- Have used another investigational study medication within 30 days prior to entering the study.

**About the study:**

- Participation in the study is about 6 months (6 months treatment and up to 1 month screening period), with a schedule as follows:

Screening: visit to be done within 30 days of start of treatment with study drug.

Treatment phase: This study includes a placebo group. Participants will be randomized to either ACTIMMUNE or placebo. Study drug will be administered 3 times per week for 26 weeks via injections under the skin. Doses will be escalated each week for the first four weeks until a stable dose is reached. Participants will maintain their stable dose for the remainder of the study. During the treatment phase, there will be clinic visits at weeks 4, 13, and 26, and several phone calls between visits. You may be confined to the study hospital for 24 hours at the Week 4 visit to obtain serial blood samples for PK analyses.

Follow-up phase: All subjects will be asked to return to CHOP within 2 weeks after the end of treatment. A 6-month open label extension study in which all patients will receive ACTIMMUNE will be offered to any patient completing the 6-month double-blind study.

- You or your child may not directly benefit from participating in this study, but you/your child and other participants may make an important contribution to advancing the understanding and treatment of FA.
- Reimbursement for reasonable study-related expenses including travel will be available to you/your child and one parent or caregiver, if applicable.

To learn more about the study, contact one of the study coordinators below:

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