Open-label pilot study of interferon gamma-1b (Actimmune™)
for the treatment of Friedreich Ataxia

June 10, 2013

The Children's Hospital of Philadelphia (CHOP) is recruiting children with Friedreich ataxia (FRDA) in the United States for a Phase II dose-escalation, open-label clinical trial studying the safety and effects of IFN-γ (interferon gamma-1b, Actimmune™) in FRDA. This study is funded by the Friedreich Ataxia Research Alliance (FARA). This study is being conducted only at the Children's Hospital of Philadelphia.

We are looking for patients between the ages of 5 and 17 years who have Friedreich ataxia.

To participate, you must:

- Have genetic confirmation of your FA (must have two GAA repeat expansions only)
- Be able to tolerate injections under the skin
- Be willing to maintain stable doses of all medications, vitamins, and supplements for 30 days prior to study entry and for the duration of the study.

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 of the study.

About the study:

- Participation in the study is for about 4 to 5 months, with a schedule as follows:
  - Screening: visit to be done within 30 days of baseline and initiation of treatment with IFN-γ.
  - Treatment phase. This is a dose-escalation study, with no placebo group. Medication will be administered 3 times per week for 8 weeks.
    - Weeks 1 and 2: all participants take 10 mcg/m2 of study medication (administered 3x/week)
    - Weeks 3 and 4: all participants take 25 mcg/m2 of study medication (administered 3x/week)
    - Weeks 5-8: all participants take 50 mcg/m2 of study medication (administered 3x/week)
  - Follow-up phase: All subjects will be asked to return to CHOP 7 and 28 days after the end of treatment with IFN-γ.
- Your child may benefit by finding some improvement in his/her condition while on the study drug. All subjects would be on active study medication rather than placebo and thus all participants would have the potential to notice some benefit in symptoms. Your child and other participants may make an important contribution to advancing the understanding and treatment of FRDA.

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

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Thank you for your ongoing support of clinical research in Friedreich ataxia and the Friedreich Ataxia Research Alliance (FARA).