

# Call for Patients with Friedreich ataxia Phase I Trial with VP-20629

July 16, 2013

The Children's Hospital of Philadelphia (CHOP) is recruiting patients with Friedreich ataxia (FA) in the United States for a Phase I clinical trial studying the safety and effects of VP-20629 (indole-3-propionic acid, an antioxidant compound) in FA. This study is sponsored by ViroPharma, Inc.

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 45 years who have Friedreich ataxia.***

## **To participate, you must:**

- Have genetic confirmation of your FA (must have two GAA repeats only);
- Be willing to abstain from alcohol within two days of your inpatient stay and throughout your time on study drug. Be willing to limit caffeine intake during similar time course.
- Be willing to discontinue taking Idebenone, coenzyme Q10, and other dietary supplements or vitamins within one week before beginning this study drug and throughout your participation in the study
- **Be willing to spend 3-10 days in an inpatient unit at CHOP**

## **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 about 5 to 7 weeks.
- The dose-escalation study has 2 main designs:
  - Single dose of VP-20629 or placebo, blinded treatment phase: participants are assigned to one of several single dose groups or matching placebo. These individuals would have three visits at CHOP, one of which is a 3-day inpatient stay at the hospital.
  - Multiple doses of VP-20629 or placebo, blinded treatment phase: participants are assigned to one of several dose groups (doses are to be given multiple times) or matching placebo. These individuals would have three visits at CHOP, one of which is a 10-day inpatient stay at the hospital.
- Once you enroll, your travel costs to CHOP will be reimbursed up to a specific amount as allowed by ViroPharma, 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.