Information About a Clinical Research Study for Children with Friedreich Ataxia (FA)

About Friedreich Ataxia

Friedreich ataxia (FA) is a rare genetic disease that mainly affects the nervous system and the heart. FA causes difficulty with walking and speaking, and also a loss of sensation in the arms and legs. There are no FA-specific treatments and no cure currently available.

About Clinical Research Studies

Clinical research studies test medicines and medical devices under controlled conditions before they are made available to the general public. Studies are carefully put together and monitored, and participant safety is the priority.

About This Research Study

This clinical research study is being conducted at the Children's Hospital of Philadelphia and will evaluate the effectiveness and safety of a study drug. It will be an open-label study in which all study participants receive the study drug (no placebo). The study drug might help decrease the progression of FA symptoms, and information gained from the study may help others with FA in the future.

About the Study Drug

The study drug, vatiquinone, targets an enzyme associated with FA. It is an oral solution that your child will take three times a day during the treatment part of the study. It has not been approved for treating FA.

What is Involved in This Study?

If your child qualifies and agrees to take part, they will be in this study for about one and a half years. You will visit the study site for medical assessments, and also speak by telephone with the study doctor or a member of the study staff to talk about your child's health.

During the treatment part of the study, your child will take the study drug three times a day with food containing at least 25% fat.

Who Can Be in This Study?

For this study, we are looking for children under 7 years of age who:

- Are diagnosed with FA confirmed by genetic testing
- Have not participated in any other interventional clinical trial in the past 2 months

Additional requirements apply, which the study doctor will discuss with you.

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Study Stages

There are three stages in this study:

Screening

You will visit the study site for a series of tests such as a physical exam, heart tests (echo and EKG), and blood tests to see if your child meets the requirements to participate in this study.

Treatment (about 17 months)

If your child qualifies and would like to participate, you will visit the study site 8 times and speak with the study staff by telephone 11 times in between visits.

Follow-Up

After your child stops taking the study drug, the study doctor or a member of the study staff will contact you by phone to talk about your child's health. The study doctor may decide to have your child come to the study site for additional medical assessments.

Informed Consent

Before any medical assessments take place, you will read and sign an informed consent document. By signing, you are stating that the study details have been explained to you, your questions have been answered, and you agree to have your child participate. This document is not a contract, and you are free to stop your child's participation in the study for any reason at any time.

Is There a Cost to Participate?

The study drug and all study-related assessments will be provided at no cost.

Travel expenses, along with study-related expenses (e.g., meals) for the child and a parent/caregiver will be reimbursed.

For more information, including possible risks and benefits of participating, please contact:

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