A RANDOMIZED, DOUBLE-BLIND, CONTROLLED, PHASE 2/3 STUDY TO ASSESS EFFICACY, LONG TERM SAFETY AND TOLERABILITY OF RT001 IN SUBJECTS WITH FRIEDREICH'S ATAXIA

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The Children's Hospital of Philadelphia (CHOP) is recruiting children and adults with Friedreich Ataxia (FA) for a Phase 2/3 study assessing efficacy, long-term safety and tolerability of RT001-006 and will include approximately 60 patients at about 10 sites in the United States.

We are looking for patients between the ages of 12 and 50 years who have FA.

To participate, you must:

- Have genetic confirmation of your FA (can have two expanded GAA repeats or one expanded repeat plus a point mutation)
- Have been diagnosed with FA at age 25 years or younger
- Be able to ride an exercise bike
- Be able to walk 25 feet within one minute, with or without the use of an assisted device
- Be willing to make dietary modifications, keep a diet log, and participate in scheduled phone calls with a dietician
- 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 disease or uncontrolled diabetes
- Have previously participated in the Retrotope phase I/II study in FA
- Have a history of substance abuse or severe mental illness within the last 2 years
- Have a history of cancer or malignancy other than basal cell carcinoma
- Be pregnant, planning a pregnancy, or breastfeeding
- Take any fish oil or oil-based supplements for the duration of the study
- Have used another investigational study medication within 30 days prior to starting study drug

About the study:

- Participation in the study is about 13 months (11 months of treatment, a 30-day phone call follow up and up to a 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 RT001-006 or placebo. Study drug will be administered orally as a loading dose of 9 capsules per day for the first month (3 capsules taken with each meal), followed by a maintenance dose of 6 capsules per day (3 capsules each taken with breakfast and dinner) for the remainder of the study. Doses will be adjusted as needed for tolerability. Participants will need to discontinue all oil-based supplements during the study. There will be clinic visits at Day 1, Day 120 (approx. 4 months on drug), and Day 240 (approx. 8 months on drug), and Day 330 (11 months on drug)

will mark the end of treatment. There will be several mandatory phone calls between visits, both with study staff and with a diet coach

Follow-up phase: All subjects will be asked to participate in a phone call follow up within 30 days after the end of treatment.

- 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 up to $1000 per patient per visit will be available

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

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