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Dear Friedreich's Ataxia Community:

We are excited to share that we will be initiating a new clinical trial for vatiquinone for the treatment of individuals living with Friedreich's ataxia (FA) from 7 to 21 years of age. We expect this study to start enrolling in the third quarter of this year.

The study design was based on discussions with the FDA and, importantly, all participants will receive vatiquinone following enrollment—there will not be a placebo group. The purpose of this study is to collect additional data on the benefits of vatiquinone to support resubmission of an approval application to FDA. The primary endpoint will be change in the modified Friedreich's Ataxia Rating Scale (mFARS) score from baseline to 24 months. We plan to compare trial participant outcomes to a matched natural history control group drawn from the FACOMS disease registry, which is a robust and well-established source of FA natural history data.

We look forward to initiating this study at FA clinics worldwide within the next few months and will share additional details as soon as possible, including specific information about participating sites, eligibility criteria, and how to learn more about enrollment once the study is opened. We will also be participating in a webinar with FARA to discuss the study on June 22nd at 12:00 pm ET. Please reach out to patientengagement@ptcbio.com with any questions.

We are grateful to the FA community for your continued dedication and partnership.