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Update to the Friedreich's Ataxia Community:

We are pleased to announce that the U.S. Food and Drug Administration (FDA) has accepted for review the New Drug Application (NDA) for vatiquinone for the treatment of children and adults 6 years and older living with Friedreich's ataxia (FA). The application has been granted priority review, which means that FDA plans to make a decision on the application within six months, compared to the standard 10-month review period. The target action date (also referred to as PDUFA date) is Aug. 19, 2025.

The granting of priority review underscores the significant need for additional treatments for individuals, particularly children living with FA. If approved, vatiquinone will be the first therapy available for children and offers an additional treatment for adults living with FA.

The vatiquinone NDA is based on data from the placebo-controlled MOVE-FA study as well as results from two long-term studies including pediatric and adult FA patients. Data from these three studies demonstrate significant, lasting and clinically meaningful evidence of slowing disease progression on key aspects of disease. In addition, these studies demonstrate that vatiquinone is safe and well tolerated in all age groups, including children.

Our team is in the process of determining the timing of approval applications to other countries where there are individuals living with FA who could benefit from vatiquinone. We will have more information on specific plans over the next several months.

We're proud to take this significant step forward together, as the community has provided unwavering support throughout the development of vatiquinone. On January 21, 2025, prior to the NDA acceptance, our CEO, Dr. Matthew Klein, joined Jen Farmer, CEO of the Friedreich's Ataxia Research Alliance (FARA), to provide an update on the development program. You can view the webinar recording here to learn more about vatiquinone and the journey to reach this milestone: https://bit.ly/40SOjko.

We extend our heartfelt appreciation for your continued support, and we look forward to sharing further updates as the regulatory review progresses.