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Update to the Friedreich's Ataxia Community:

PTC recently announced that, as expected, we have submitted the New Drug Application (NDA) to the U.S. FDA for vatiquinone. This submission is an important step in our path to bring forward a potential therapy for the treatment of both children and adults living with FA. If approved, vatiquinone would be the first and only therapy for children affected by FA.

The vatiquinone NDA is based on data from the placebo-controlled MOVE-FA study as well as results from two long-term studies. Data from these three studies demonstrated significant and clinically meaningful evidence of slowing disease progression over many years. Importantly, these studies demonstrated that vatiquinone was safe and well tolerated across all age groups studied, including children.

This milestone would not be possible without the support from the entire FA community, including researchers, clinicians, advocates, and most importantly, people living with FA. We have been working with the community for many years to advance vatiquinone towards approval and we are very proud to have reached this important milestone. We thank you for your ongoing support to help bring this transformative treatment to those that may benefit from it.

Our CEO, Dr. Matthew Klein will be joining Jen Farmer, CEO of FARA on a webinar on January 21, 2025 to discuss the vatiquinone program and the approval application. More details on the event will be provided by FARA in the coming weeks.

We thank you again for your support and look forward to connecting in the New Year.