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Update to the Friedreich's Ataxia Community:

We recently shared the disappointing news that the U.S. Food and Drug Administration (FDA) has issued a Complete Response Letter (CRL) related to the New Drug Application (NDA) for vatiquinone for the treatment of children and adults living with Friedreich's ataxia. In the letter, the FDA stated that substantial evidence of efficacy was not demonstrated for vatiquinone and that an additional clinical trial would be needed before resubmitting the NDA for consideration of approval. We plan to meet with the FDA as soon as possible to discuss potential steps to address the issues raised in their letter.

This news is incredibly disappointing for PTC and the FA community. We believe the data collected to date from our clinical studies demonstrate that vatiquinone could provide a safe and effective therapy that could fulfill the significant need for children under the age of 16 living with FA as well as provide an additional treatment option for adults.

While we work with FDA to understand the next steps for vatiquinone, we will continue all ongoing vatiquinone studies and continue to provide vatiquinone for all those participating in the studies for the foreseeable future.

We appreciate the unwavering support of the entire FA community and will continue to provide updates as they become available.