



Message to the Friedreich Ataxia Community

Dear NAF community,

Yesterday we shared a press release that includes some updates about our investigational gene therapy candidate, LX2006, for the treatment of Friedreich ataxia cardiomyopathy. We are pleased to share some exciting developments on our program, which you can read below.

You can read the full press release [here](#) on the Lexeo website.

Highlights include:

- The FDA has agreed with our plan to include pediatric patients, including both children and adolescents, in the pivotal study
- The FDA has also agreed that as part of our pivotal program, we will not be required to have a placebo arm in the pivotal study. Data from a separate natural history study (CLARITY-FA) will be used to compare against the pivotal study
- Based on improvements observed in cardiac measures in the Phase 1/2 studies, protein expression in the planned pivotal trial will be measured based on any increase from the starting level. This highlights the FDA's increased understanding of FA and the potential connections between any increase in frataxin and clinical benefit
- We plan to share new clinical data from our Phase 1/2 trial for LX2006 in mid-2025

Our CLARITY-FA natural history study (LX2006-02) is expected to begin enrollment in the US in April. The goal of this natural history study is to learn about how heart disease develops and worsens in individuals with FA. Information gained from participation in this study will provide valuable data about heart disease in FA and will help advance our gene therapy research.

We appreciate our study investigators and the courageous trial participants and caregivers who have helped us get to this point. Lexeo is dedicated to advancing

therapies that target the root causes of conditions with few existing treatment options, striving to transform patient care and improve lives.

We will keep this community updated as we continue our work with regulatory authorities to design and implement clinical studies, as well as enroll participants in ongoing and future trials. For more information or any questions, please contact clinicaltrials@lexeotx.com.

Thank you for your partnership and working with Lexeo to help address the unmet need in Friedreich ataxia cardiomyopathy.

The Lexeo Team