



Message to the Friedreich Ataxia Community

April 8, 2025

Dear Advocacy Partners,

Yesterday, we were pleased to share positive interim clinical data for LX2006, our investigational gene therapy candidate for the treatment of Friedreich ataxia cardiomyopathy that is currently being studied in the Lexeo-sponsored SUNRISE-FA Phase 1/2 clinical trial and the Weill Cornell Medicine investigator-initiated Phase 1A trial. This update included data from 12 participants with at least 6 months of follow-up across the two trials.

We are thrilled to share this exciting update for our program, which we believe supports advancement towards a future marketing application.

The full press release can be found [here](#) on the Lexeo website.

Highlights include:

- Participants who began the trial with an abnormal or increased left ventricular mass index (LVMI) (n=6) experienced an average improvement of 25% within a year or less of treatment. LVMI is a measure of the size and thickness of the heart. An abnormally thick heart can lead to serious heart complications, and reducing LVMI can potentially improve health outcomes.
- Most participants demonstrated clear improvements in biomarkers that evaluate heart health and tests that measure overall physical function, including the mFARS scale.
- All participants in the SUNRISE-FA trial (n=8) experienced increases in frataxin levels in the heart at 3-months post treatment.
- Increase in frataxin expression and improvement in LVMI are consistent with FDA's expectations for the planned registrational study.
- LX2006 continues to be generally well tolerated by study participants. Last fall, Lexeo reported one participant had an adverse event of asymptomatic myocarditis, one year after dosing which was considered to be possibly related to the study treatment. This individual continues to be monitored.

Next steps for the LX2006 program:

We are excited to continue the next phase of our program.

- Lexeo plans to start our registrational study (LX2006-3) by early 2026. This will be an interventional trial (i.e., participants will be given the investigational gene therapy LX2006). We will provide updates to this community on all ongoing and future trials.
- Our CLARITY-FA natural history study (LX2006-02) is expected to begin enrollment in the US this spring. 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.
 - It is important to note that participants in the CLARITY-FA natural history study will not receive any investigational study drug (i.e., gene therapy).
- For more information or any questions, please contact clinicaltrials@lexeotx.com.

We thank our study investigators and the courageous trial participants and caregivers who have helped us get to this point. We appreciate your partnership as we work to help address the unmet need in Friedreich ataxia cardiomyopathy.

The Lexeo Team