Message to the Friedreich Ataxia Community

July 10, 2025

Dear Advocacy Partners and FA community members,

We are pleased to share that LX2006, our investigational gene therapy for Friedreich ataxia cardiomyopathy, has been granted Breakthrough Therapy designation by the U.S. Food and Drug Administration (FDA). LX2006 was also selected for participation in the FDA's Chemistry, Manufacturing, and Controls Development and Readiness Pilot (CDRP) program.

The full press release can be found here on the Lexeo website.

Highlights include:

- The U.S. Food and Drug administration (FDA) has granted Breakthrough Therapy designation to LX2006 based on clinical evidence generated on both cardiac and neurologic measures of Friedreich ataxia (FA). This designation is intended to accelerate the development and review of investigational therapies that aim to treat serious or life-threatening diseases, and where preliminary clinical evidence indicates that the therapy may demonstrate substantial improvement over available alternatives.
- This designation reflects the strength of the clinical evidence generated to date on LX2006. Interim data showed clinically significant improvements in cardiac biomarkers and in both cardiac and neurological functional measures, with increased frataxin expression observed in all participants with cardiac biopsies done at three months after treatment.
- LX2006 was also selected for the FDA CDRP program, which facilitates faster manufacturing development for promising investigational therapies with the goal of bringing earlier patient access to therapies in areas of high unmet need.
- Lexeo is currently enrolling a prospective natural history study, CLARITY-FA, which will serve as an external control arm for the registrational study. The registrational study,

with LX2006 as interventional study drug, is expected to begin early 2026. You can find more information on these trials here: https://trials.lexeotx.com/

We thank our study investigators and the courageous trial participants and caregivers who have helped us achieve this incredible milestone. We appreciate the Friedreich ataxia community's partnership as we work to help advance research in FA.

The Lexeo Team