Webinar Summary: Larimar Therapeutics Nomlabofusp Clinical Program Update

This is a summary of the informational webinar with Larimar Therapeutics that occurred on June 24, 2025. During this webinar Larimar's Chief Medical Officer, Russell Clayton, DO, provided an update on the nomlabofusp clinical trial program.

What is nomlabofusp?

Nomlabofusp (previously called CTI-1601) is a protein replacement therapy designed to address the deficiency of frataxin in FA. It is administered by subcutaneous (under the skin) injection.

Upcoming clinical trial developments:

- Open label study: In July of 2025, the open label study will start enrolling individuals over the age of 12 who participated in the Phase 1 pediatric study. In addition, in the near future the open label study will be open to patients with FA who have not previously participated in a nomlabofusp trial and who will not qualify for the future global phase 3 trial.
    - Criteria related to omaveloxolone: individuals in the open label study must complete at least three months of nomlabofusp before starting omaveloxolone. Individuals already taking omaveloxolone must have received omaveloxolone for at least three months prior to receiving nomlabofusp in the open label study.
- Phase 1 pediatric dosing study: The 12- to 17-year-old cohort completed dosing and these participants are now enrolling in the open label study. The start of the 2-to-11-year cohort is on pause as Larimar works to ensure that everything is in place to allow these participants to immediately enter into the open label study at the completion of the dosing study. Larimar hopes to provide more information about the timing for the start of this cohort next month.
- Phase 3 study: Global Phase 3 study activities are ongoing. Sites in the US, Canada, UK, Europe, and Australia have been identified and are being qualified. Larimar expects recruiting to begin later this year. This will be a double blind, placebo-controlled study with one-to-one randomization. This study will enroll 100 to 150 participants with FA who are ambulatory. The study initially will enroll participants between the ages of 12 and 40 years. The lower age limit will be dropped to two years of age once Larimar has collected sufficient data from pediatric participants in the open label study.