April 17, 2024

Dear leaders of FAST,

We hope this letter finds you well.

We'd like to share an update on GTX-102, our development program for Angelman syndrome, and new interim data that was presented this week at the American Academy of Neurology annual meeting (AAN).

In total, the Phase 1/2 Study has enrolled and treated 74 patients in the "Dose-escalation" and "Expansion" Cohorts. The aim of the Dose-escalation Cohorts is to determine the best dosing regimen for the expansion phase of the Study, and the Expansion Cohorts will further evaluate the safety and efficacy of treatment at that specified dose. The new data being presented this week focus on the first six months of treatment in the Expansion Cohorts and demonstrate encouraging trends across several aspects of development affected by Angelman syndrome, reinforcing our confidence in the clinical development program to move forward into our next phase.

We are now preparing for interactions with the FDA and other health authorities to discuss our findings from the Phase 1/2 trial and the Phase 3 clinical program. While timelines are not fully within our control, as we will require inputs from the regulatory agencies, we aim to initiate a placebo-controlled, global, Phase 3 study by the end of 2024 in individuals with Angelman syndrome with a confirmed gene deletion. We will also explore the opportunity to evaluate GTX-102 in patients with other genotypes and age groups in the future.

At Ultragenyx, we are committed to partnering with the Angelman syndrome community to learn as much as we can and to keeping you updated so members of your community can make important care decisions for their families. We want to thank the patients and families enrolled in the GTX-102 Phase 1/2 study for their trust and investment in our clinical trial, as well as you and the larger AS community for your partnership, patience, and support as we continue our research and plans for a global Phase 3 program.

Sincerely,

Elizabeth Maia
Global Patient Advocacy & Patient Engagement
Ultragenyx