



Going beyond every day.™

Ultragenyx
Novato, CA 94949

September 15 2023

Dear members of the Angelman Syndrome community,

First and foremost, thank you for your patience and support as we continue to work through a series of critically important queries regarding eligibility in our current and future trials for Angelman Syndrome patients.

We wanted to share with you the following update on our program and reassure you that we are committed to providing timely updates to the Angelman community so families can make informed decisions regarding participation in a trial.

While our current Phase 1/2 study is evaluating GTX-102 in pediatric patients (aged 4 to 17 at screening) with UBE3A gene deletions, we do plan to evaluate the safety and efficacy of GTX-102 in patients younger than 4 years of age, as well as those with non-deletion-type Angelman Syndrome (i.e., mutations, imprinting center defects, uniparental disomy, mosaicism). We are actively exploring options and will inform the Angelman Syndrome community when we have more details about the design and timing.

With respect to individuals previously treated with other ASOs, we are gathering the information necessary to determine how and when they could be treated with another investigational product. We do not know how long this process will take but will keep the Angelman Syndrome community updated. We realize that this is not a complete answer, especially for individuals and families who may have been participating in the rugonersen (RO7248824) trial.

The safety of individuals living with Angelman Syndrome is of the utmost importance to Ultragenyx so we need complete and accurate information that will best inform our science and development strategy. We recommend families work with their physicians to discuss their individual care plans and options moving forward.

At Ultragenyx we are committed to partnering with you to learn as much as we can and keeping the AS community updated so they can make important care decisions for their families.

Thank you for all you do,

Sincerely,

A handwritten signature in black ink that reads "Eric Crombez".

Eric Crombez, MD
Chief Medical Officer
Ultragenyx