



Going beyond every day.™

60 Leveroni Court
Novato, CA 94949

July 6, 2023

Dear members of the Angelman syndrome community,

We understand that it's been a difficult couple weeks for the Angelman community, which has led to a number of unanswered questions. Ultragenyx has received several queries and we want to reassure the community that our Phase 1 /2 study is not impacted, and that we are continuing to enroll and dose participants around the globe.

We have full confidence in our development program, GTX-102, our ASO for the potential treatment of Angelman syndrome, and the results we've observed in our Phase 1/2 study are encouraging. We are currently activating additional sites in the U.S. and other countries as we confirm the GTX-102 treatment regimen that will be used in the Phase 3 program. While the current Phase 1/2 study is evaluating GTX-102 in pediatric patients with UBE3A gene deletions, we do plan to evaluate GTX-102 in patients with other genotypes and age groups in the future.

There have been questions about eligibility in our current and future trials for individuals previously treated with other ASOs. While we would like to be able to provide a definitive answer, there is still information that we need to gather as a company, which will take some time. We realize that this is not a complete answer, especially for individuals and families who may have been participating in the rugonersen (RO7248824) trial. However, given that the safety of individuals living with Angelman syndrome is of the utmost importance to Ultragenyx, we want to ensure we have 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.

We remain committed to providing timely information to the Angelman community regarding our program, so families can make informed decisions regarding participation in a trial.

Sincerely,

DocuSigned by:

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

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Eric Crombez, M.D.

Executive Vice President and Chief Medical Officer

Ultragenyx

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