



Going beyond every day.™

September 5, 2026

Dear Angelman syndrome Community,

As shared in our initial update, Ultragenyx continues to evaluate next steps for the apazunersen program in light of the topline findings from the Aspire Phase 3 study released earlier this week. First, we acknowledge how difficult and disappointing this news continues to be for families, advocates and everyone who has dedicated their time, effort and hope to advance a first-ever treatment to ease the daily burden of Angelman syndrome.

We understand the frustration and concern that exists and will provide timely updates as we move forward. Today, 9/5, we share the following:

- Ultragenyx is temporarily halting GTX-102 dosing across all active studies while we continue to review the data from the Aspire study, discuss the findings with investigators, and assess next steps for the apazunersen program.
- Investigators have been informed and will communicate with study caregivers and participants directly about how this will impact them.
- Please direct additional questions to patientinquiry@ultragenyx.com

We will share information as able and will continue to engage in community-focused forums transparently.

Although this outcome is not what any of us hoped for, we remain deeply appreciative of the opportunity to work alongside this community throughout this program. We hope that in time, the learnings will inform other development efforts across the field.

We are deeply grateful for your continued collaboration.