



Going beyond every day.™

September 16, 2026

Dear Angelman syndrome Community,

We are following up with an important update on the apazunersen (GTX-102) program after our initial communication on September 4, 2026. After carefully considering next steps in light of topline findings from the *Aspire* Phase 3 study released earlier this month, Ultragenyx has made the difficult decision to discontinue the GTX-102 program. Due to the failure to demonstrate clinical efficacy in the Phase 3 study, despite the beneficial effects some patients have seen in the trials, the current overall clinical benefit observed just does not support continuation of the trials. Knowing all that we know about Angelman syndrome and what we have learned during these studies it is heartbreaking, but we are left with no way to proceed with development with this result.

Study investigators were informed of the decision earlier this week to ensure families could be informed directly before we shared this news with the Angelman syndrome community. We are deeply grateful to, and humbled by, the families who participated in these studies, as well as the advocacy organizations, investigators, study teams, and broader Angelman syndrome community whose dedication, engagement and commitment has advanced research in this field.

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 also recognize the community's desire to better understand these results and are exploring pathways to share the data more broadly with the hope it can support future learnings for the field.

A summary of key takeaways is below. Please direct additional questions to patientinquiry@ultragenyx.com.

- The GTX-102 studies, *Aspire*, *Aurora* and the Long-Term Extension (LTE) study will officially close on October 30, 2026.
- Investigators have been informed and are actively communicating with study caregivers and participants directly about what this means for them.
- Ultragenyx continues to explore pathways to share the data, including community forums, and will do so in partnership with FAST and the Angelman Syndrome Foundation.
- Ultragenyx and FAST are also discussing finding additional ways to support the community during this difficult transition.