Summer 2026

# AS Community Update

*GTX-102 program updates and global community engagement highlights are being shared to honor your ongoing request for information.*

## Investigational Clinical Trial Program Overview

**Phase I/II:** More than 70 participants have been treated with GTX-102 in the Phase 1/2 study, and the majority are continuing in the long-term extension study. GTX-102 has not been approved by the FDA or any other regulatory authority. Updated data from the study are expected to be presented at a future scientific meeting.

**Phase II (AURORA):** Enrollment is ongoing, with completion expected in the second half of 2026. All Ultragenyx clinical trial sites are activated with more information available on clinicaltrials.gov.

**Ph III Aspire:** Phase 3 data are still expected in the second half of 2026.

## New Research Leverages Global Caregiver Perspectives to Demonstrate that AS Impacts all Aspects of Family Life

Seven caregivers (five parents and two siblings) of adults (age 18-37) living with AS from around the world were recruited to share their perspectives on Angelman syndrome. Caregivers reported that their individuals with AS required constant supervision and support for basic activities of daily living. Sleep disturbances were noted as one of the most difficult symptoms to manage. Caregivers also described having to give up careers working outside the home to care for their individuals when they were young, limiting earning potential. Most caregivers never returned to the workforce, even as their individuals aged into adulthood.

Caregivers needed to find adult day care, transition from pediatric to adult healthcare, and make legal provisions to manage money and care decisions on behalf of their adults with AS. Most individuals with AS were covered under a complex combination of government-funded programs and private insurance that varied based on where the family lives.


## Community Corner

- **The U.S. FDA** has launched "Rare Connections," a quarterly newsletter from the Rare Disease Innovation Hub designed to keep the rare disease community informed and connected with the latest updates across the FDA. *Anyone can sign up for the newsletter*.
- **ASGCT** developed a creative way to share the impact of genetic medicine. The cell and gene therapy-themed coloring book can be downloaded for free.

## Angelman Caregiver Leadership Council Sheds Light on Living with AS

- Ultragenyx hosted an Angelman Syndrome (AS) Caregiver Leadership Council (CLC) with caregivers from across the globe.
- Participants shared perspectives on the biggest hurdles to navigating the health care system with AS, and the impact AS has on the whole family.
- A phrase raised and salient among caregivers was “consistently inconsistent” capturing the unique ways children with Angelman syndrome may learn, grow and experience the world.
- Insights from discussions like these help deepen understanding of the lived experience with AS and shape how we assess for potential treatment responses in trials.

## Research and Development Corner: It Takes a Village

- Developing new therapies requires hundreds of people working together behind the scenes.
- In this issue, we’d like to spotlight the Endpoint Development and Strategy team. This team is responsible for designing and validating patient-reported outcomes (PROs), clinician-reported outcomes, and functional performance measures for rare diseases.
- Paired with perspectives of the community, this team helps develop and evaluate measures and analyses, such as the Multi-Domain Responder Index (MDRI), that can capture changes that are meaningful to people living with rare diseases and their families.

**As appropriate we will continue to share timely information as the program progresses.**

*As a reminder, the safety and efficacy of GTX102 have not yet been established and it has not been approved by any regulatory agencies.*