

AS Community Update

Ultragenyx's Angelman program updates and global community engagements are being shared to honor your ongoing request for information.

Research on Angelman Syndrome and Development on an Investigational ASO by Ultragenyx, Continues

Ph I/II Plain Language Summary Available

In January, we [posted](#) the Plain Language Summary of the Phase I/II clinical trial of the safety and tolerability of an investigational ASO in kids with Angelman syndrome.

Ph II Study (Aurora) in Active Enrollment



Per clinicaltrials.gov Ultragenyx has 22 planned locations, with enrollment already active at 9 sites. We are engaging in discussions to be able to enroll in the US, Argentina, Brazil, France, Israel, Italy, Portugal and the UK.

Expectations Remain for Ph III Study (Aspire)



ASPIRE study completion continues to be expected in the second half of 2026 and Ultragenyx expects to report data as soon as possible upon study completion.



Ultragenyx Commemorated International Angelman Day!

The Ultragenyx team took time to pause, connect and [reflect](#) on what it means to contribute to research in Angelman syndrome, the moments that first sparked our passion and how partnering with families continue to guide and motivate us.

We're thankful for the AS community and remain committed to honor, serve and work with the community.



Advocacy in Action

We are encouraged by the continued commitment to rare diseases, including Angelman syndrome, seen in the passage of key provisions in Federal US FY26 funding bills. We remain focused on what matters most, delivering meaningful therapies to patients and families who can't afford to wait.

The Mikaela Naylor Give Kids a Chance Act has been signed into law, reinstating the Rare Pediatric Disease Priority Review Voucher (PRV) Program

Looking Ahead in 2026

As we continue to advance an ASO under development by Ultragenyx and plan for the next stages, we are honored to continue this journey together. Your partnership will remain essential as we:

- **Generate insights** that inform transparent and responsible communications
- **Foster understanding** of AS through clear and accessible education
- **Champion the Community Voice** so that future program decisions reflect what matters most to those affected by AS.
- **Explore opportunities** to address unmet education and information needs.

Community Engagement

As part of our commitment to patient-focused drug development, we work closely with leaders from advocacy organizations and individuals impacted by Angelman syndrome. These partnerships provide important opportunities for our team to listen to and learn from the community and help to ensure we understand what is most important to patients and families. Our work is strengthened by understanding disease impact, unmet needs and challenges, and using this information to inform our strategies and decision-making.

We welcome community questions - please reach out to patientadvocacy@ultragenyx.com.

We will continue to share timely information as the program progresses.

As a reminder, the safety and efficacy of Ultragenyx's investigational drug candidate has not yet been established and it has not been approved by any regulatory agencies.