WINTER 2026

GSDIa Community Update

DTX401 program updates and global community engagement highlights are being shared to honor your ongoing request for information.

Regulatory Update

In February, Ultragenyx announced the U.S. FDA granted Priority Review for the Biologics License Application (BLA) for DTX401, an investigational gene therapy for GSDIa, and set a decision date of August 23, 2026. Priority Review does not mean approval, and FDA will evaluate the totality of the data before making a decision. We shared a social graphic explaining this news and outlining the next steps in the FDA review process. You can view the graphic here. We will continue to share updates with you throughout this process as we are able.

Sharing Research Results

In January, our team was invited to share an encore (repeat) presentation of the "Results from a Pivotal Phase 3 Double-blind Placebo-controlled Trial of DTX401 for the Treatment of Individuals with GSDIa" at the 32nd Annual Meeting of the Japan Society of Gene and Cell Therapy. These data were originally presented by a DTX401 Principal Investigator at the International Congress of Inborn Errors of Metabolism 2025 in Kyoto, Japan last September.

In February, our team encored two posters:

- "Nutritional Changes after an AAV8-mediated Liver-directed Gene Therapy in Participants with GSDIa: Results from a Phase 3 Pivotal Trial" at Washington University Medicine's Rare Disease Day. These data were originally presented by a metabolic dietitian at the 42nd Annual Meeting of the Southeastern Regional Genetics Group in Asheville, North Carolina last July.
- "Results from a Pivotal Phase 3 Double-blind Placebo-controlled Trial of DTX401 for the Treatment of Individuals with GSDIa" at the 2026 Hospital for Sick Children Rare Disease Day in Toronto, Canada. These data were originally presented by a DTX401 Principal Investigator at the International Congress of Inborn Errors of Metabolism 2025 in Kyoto, Japan last September.

In March, our team shared presentations at the 2026 ACMG Annual Clinical Genetics Meeting in Baltimore, Maryland:

- "Long-term Efficacy and Safety of DTX401, an AAV8-mediated, Liver-directed Gene Therapy, for the Treatment of GSDIa: Phase 1/2 End-of-Study Results" The Phase 1/2 primary (main) results were published in a scientific manuscript in 2025, and this is the first time these end-of-study (final) results were orally presented at a scientific congress.
- "Burden of Navigating the Healthcare System on People and Families Living with GSDIa and Their Care Team" A collaboration with patients, caregivers and clinicians in the US and UK, this is an encore presentation that was originally presented at the 2025 Association for Glycogen Storage Disease (ASGD) Conference.
- "A Locus-specific Database of G6PC1 Gene Variants Associated with GSDIa" This database, which supports the timely and accurate diagnosis of GSDIa, was originally presented at the International Congress of Inborn Errors of Metabolism 2025 in Kyoto, Japan last September.

We will continue to share timely information as the program progresses.
As a reminder, the safety and efficacy of DTX401 have not yet been established and it has not been approved by any regulatory agencies.

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Ultragenyx Stands with the Rare Disease Community During Rare Disease Week

Throughout Rare Disease Week and Rare Disease Day, Ultragenyx joined patients, caregivers, advocates, clinicians, researchers, and policymakers around the world to advance conversations that drive meaningful progress for people living with rare diseases. From advocacy meetings to global reflections on Rare Disease Day, our team reinforced our shared commitment to transforming urgency into action so more families have answers and more patients have the options they need. Learn more about how we spent the week advocating across the globe here.

New Opportunity to Share Your Experiences with Ultragenyx

As part of our ongoing commitment to patient-focused drug development, we actively engage with the global GSDIa community of patient advocacy leaders, individuals living with GSDIa, and caregivers of children diagnosed with GSDIa. Through a variety of engagement activities, such as advisory boards, interviews, and leadership councils, we seek to better understand the lived experiences, priorities, and unmet needs among individuals and the broader community. These insights help guide our strategic priorities and inform decision-making as we work to address what matters most to patients and their families.

As one example of these efforts, in February we announced plans to host a virtual advisory board to gather perspectives directly from adults (18+) living with GSDIa and caregivers of children (8+) living with GSDIa who reside in the European Union (EU), United Kingdom (UK), Switzerland, and the Kingdom of Saudi Arabia (KSA).

If you live in one of these countries or regions and are interested in participating, please email GSDIa.Ultragenyx@envisionpharma.com.

Individuals interested in sharing their perspectives who reside outside the UK, EU, KSA, or Switzerland are encouraged to contact patientadvocacy@ultragenyx.com.

Raising Awareness of GSDIa

Ultragenyx continues to expand efforts to raise awareness about the burden of living with GSDIa by actively elevating patient and caregiver voices. This includes developing new social media content that highlights day-to-day challenges, amplifies community stories, and educates broader audiences, including policymakers and the media, about unmet needs. You can see a recent example of an animated infographic that we published in March here.

If you are interested in getting involved or sharing your story, please reach out to patientadvocacy@ultragenyx.com.

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