

# GSDIa Community Update



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

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## DTX401 Biologics License Application (BLA) Submitted to U.S. FDA

In December, Ultragenyx announced that it has completed the rolling submission of its BLA to the U.S. Food and Drug Administration (FDA) seeking approval for DTX401, our investigational gene therapy for the potential treatment of GSDIa. The FDA will now decide whether to accept the filing for review within 60 days for Priority Review or 74 days for Standard Review.

We shared a social graphic explaining what a BLA is 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.

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## Sharing Research Results

In November, Ultragenyx presented 3 posters on GSDIa at ISPOR Europe, a conference hosted by the International Society for Pharmacoeconomics and Outcomes Research (ISPOR) focused on health economics and outcomes research.

- “Unseen Burdens of GSDIa Experienced by Patients, Caregivers, and their Healthcare Team”
    - We partnered with patients, caregivers, dietitians, and physicians from the U.S., U.K., Italy and the Netherlands to understand the burdens that people impacted by GSDIa face daily and explore how these burdens impact healthcare delivery.
  - “The Clinical Burden and Complications Associated With GSDIa: Results From a Systematic Literature Review”
    - We partnered with physicians from the U.S. to conduct a review of existing publications on the epidemiology, clinical burden, treatment strategies, and the humanistic/societal impact of GSDIa to better understand the acute and long-term complications that place a substantial burden on patients and caregivers.
  - “Qualitative Trial Interviews to Explore the Experience of Adult and Pediatric Patients Participating in a Pivotal Phase 3 Trial of DTX401 for GSDIa”
    - Participants in the Phase 3 study for our investigational gene therapy for GSDIa shared personal perspectives about the impact of GSDIa on their life, their experiences with the gene therapy, and the impact of gene therapy on their quality of life and disease management.
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## Community Engagement

As part of our commitment to patient-focused drug development, we work closely with leaders from patient advocacy organizations and individuals impacted by GSDIa. These partnerships provide important opportunities for our team to listen to and learn from the community and helps 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 always welcome meeting community members - please reach out to [patientadvocacy@ultragenyx.com](mailto:patientadvocacy@ultragenyx.com) if you are interested in sharing your story.