GSDIa News Update

The U.S. FDA has granted Priority Review for the Biologics License Application (BLA) for our investigational gene therapy for Glycogen Storage Disease Type Ia (GSDIa).

ultragenyx

What Happens Next?

- The U.S. FDA granted the BLA Priority Review and assigned a Prescription Drug User Fee Act (PDUFA) action date of August 23, 2026.

- The PDUFA date indicates when the FDA would be expected to communicate a decision about whether or not to approve for use outside of a clinical trial.

- If approved by the FDA, access to treatment would go through Qualified Treatment Centers (QTCs) located in the U.S.

ultragenyx

Current dietary approaches to managing GSDIa place an extraordinary burden on individuals and families, while still leaving patients with significant medical needs. If approved, our gene therapy would be the first treatment to address the disease at its root cause.

Eric Crombez, M.D.
Chief Medical Officer

ultragenyx