Ultragenyx Announces U.S. FDA Acceptance and Priority Review of the Biologics License Application (BLA) for DTX401 AAV Gene Therapy for Glycogen Storage Disease Type Ia (GSDIa)

February 23, 2026

PDUFA action date set for August 23, 2026

If approved, DTX401 will be the first treatment to address the underlying cause of GSDIa

NOVATO, Calif., Feb. 23, 2026 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE) today announced the U.S. Food and Drug Administration (FDA or the Agency) has accepted for review the Biologics License Application (BLA) seeking approval of DTX401 AAV gene therapy (pariglasgene brecaparvovec) for the treatment of Glycogen Storage Disease Type Ia (GSDIa). The FDA granted the BLA Priority Review and assigned a Prescription Drug User Fee Act (PDUFA) action date of August 23, 2026.

“Current dietary approaches to managing GSDIa place an extraordinary burden on individuals and families while still leaving patients with significant medical needs, including the risk of potentially life-threatening episodes of acute hypoglycemia and accumulation of long-term complications over their lifetime,” said Eric Crombez, M.D., chief medical officer at Ultragenyx. “If approved, DTX401 would be the first treatment to address the disease at its root cause. We appreciate the FDA’s timely acceptance of the BLA and will continue to work with the Agency throughout its review process.”

The BLA is based on data from a rigorous clinical development program that includes 52 treated patients and up to six years of follow-up. Previously announced data from the randomized, double-blind, placebo-controlled Phase 3 *GlucoGene* study demonstrate that patients treated with DTX401 experienced significant and clinically meaningful reductions in both the quantity and frequency of daily cornstarch intake while maintaining low levels of hypoglycemia, improved levels of euglycemia, and improved fasting tolerance. These clinical benefits translated to meaningful improvements in patient-reported quality of life, as measured by the Patient Global Impression of Change (PGIC) scale. DTX401 was well tolerated, with an acceptable safety profile.

If approved, DTX401 will be manufactured entirely within the U.S. at the new Ultragenyx gene therapy manufacturing facility in Bedford, Massachusetts.

About DTX401 (pariglasgene brecaparvovec)
DTX401 is an investigational AAV8 gene therapy designed to deliver stable expression and activity of G6Pase under control of the native promoter to allow the treated liver cells to respond to normal hormonal signals intended to manage glucose, including insulin and cortisol. DTX401 is administered as a single intravenous infusion and has been shown in preclinical studies to improve G6Pase activity and reduce hepatic glycogen levels, a well-described biomarker of disease progression. DTX401 has been granted Rare Pediatric Disease designation, orphan drug designation, Fast Track designation, and regenerative medicine advanced therapy (RMAT) designation from the U.S. FDA, as well as orphan drug and PRIority MEdicines (PRIME) designations from the European Medicines Agency.

About Glycogen Storage Disease Type Ia (GSDIa)
GSDIa is a rare, serious, and life-threatening disease due to an inborn error of carbohydrate metabolism caused by pathogenic variants of the *G6PC* gene, which encodes G6Pase, an enzyme that is critical for the release of glucose from glycogen and other metabolic sources. Deficiency of G6Pase activity results in severe hypoglycemia during periods of fasting between meals and during the night, along with excess hepatic glycogen storage, metabolic derangements, and other disease related complications. Cornstarch is critical in the management of GSDIa throughout the day and night in providing an exogenous source of glucose to avoid sudden and severe drops in plasma glucose levels; however current management strategies carry a significant burden to patients and families. There are no approved pharmacologic therapies. GSDIa is estimated to affect approximately 6,000 people in commercially accessible geographies.

About Ultragenyx
Ultragenyx is a biopharmaceutical company committed to bringing novel therapies to patients for the treatment of serious rare and ultra-rare genetic diseases. The company has built a diverse portfolio of approved medicines and treatment candidates aimed at addressing diseases with high unmet medical need and clear biology, for which there are typically no approved therapies treating the underlying disease.

The company is led by a management team experienced in the development and commercialization of rare disease therapeutics. Ultragenyx’s strategy is predicated upon time- and cost-efficient drug development, with the goal of delivering safe and effective therapies to patients with the utmost urgency.

For more information on Ultragenyx, please visit the company's website at: www.ultragenyx.com.

Forward-Looking Statements and Use of Digital Media
Except for the historical information contained herein, the matters set forth in this press release, including statements related to Ultragenyx's expectations and projections regarding its future operating results and financial performance, business plans and objectives for DTX401, expectations regarding the tolerability and safety of DTX401, expectations regarding the adequacy of clinical data to support the marketing application and approval of DTX401, the potential timing and success of, the marketing application and other regulatory approvals for DTX401, expectations regarding timing of receiving potential approval of DTX401, expectations regarding the prevalence of patients of DTX401, future regulatory interactions, expectations regarding the company’s ability to resolve with the FDA the observations in the complete response letter, and the value to be generated by DTX401, and future clinical and regulatory developments for DTX401 are forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements involve substantial risks and uncertainties that could cause our clinical development programs, collaboration with third parties, future results, performance or achievements to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, the uncertainty of clinical drug development and unpredictability and lengthy process for obtaining regulatory approvals, the ability of the company to successfully develop DTX401, the company’s ability to achieve its projected development goals in its expected timeframes, risks related to adverse side effects, risks related to reliance on third

party partners to conduct certain activities on the company's behalf, the company's limited experience in operating its own manufacturing facility, the ability of the company and its third party manufacturers to comply with regulatory requirements, our ability to successfully manage the expansion of our company, delays or unexpected costs and other adverse effects related to the recently announced strategic restructuring plan, smaller than anticipated market opportunities for the company's products and product candidates, manufacturing risks, competition from other therapies or products, and other matters that could affect sufficiency of existing cash, cash equivalents and short-term investments to fund operations, the company's future operating results and financial performance, the timing of clinical trial activities and reporting results from same, and the availability or commercial potential of Ultragenyx's products and drug candidates. Ultragenyx undertakes no obligation to update or revise any forward-looking statements.

For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to the business of Ultragenyx in general, see Ultragenyx's Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) on February 18, 2026, and its subsequent periodic reports filed with the SEC.

In addition to its SEC filings, press releases and public conference calls, Ultragenyx uses its investor relations website and social media outlets to publish important information about the company, including information that may be deemed material to investors, and to comply with its disclosure obligations under Regulation FD. Financial and other information about Ultragenyx is routinely posted and is accessible on Ultragenyx's Investor Relations website (https://ir.ultragenyx.com/) and LinkedIn website (https://www.linkedin.com/company/ultragenyx-pharmaceutical-inc-/).

Ultragenyx Contacts

Investors
Joshua Higa
ir@ultragenyx.com

Media
Jess Rowlands
media@ultragenyx.com