Ultragenyx Announces U.S. FDA Acceptance of BLA Resubmission for UX111 AAV Gene Therapy to Treat Sanfilippo Syndrome Type A (MPS IIIA)

April 2, 2026

If approved, UX111 will be the first approved therapy for the treatment of Sanfilippo syndrome Type A, a rare disease affecting young children that leads to progressive, irreversible neurodegeneration and early death

PDUFA action date set for September 19, 2026

NOVATO, Calif., April 02, 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 resubmitted Biologics License Application (BLA) seeking accelerated approval for UX111 (rebisufligene etisparvovec) AAV9 gene therapy as a treatment for patients with Sanfilippo syndrome Type A (MPS IIIA). The FDA set a Prescription Drug User Fee Act (PDUFA) action date of September 19, 2026.

"The FDA's acceptance of the BLA for UX111 brings us closer to the possibility of a first-ever therapy for Sanfilippo syndrome Type A—a milestone that we recognize cannot come soon enough for families facing this devastating diagnosis," said Emil D. Kakkis, M.D., Ph.D., chief executive officer and president of Ultragenyx. "We appreciate the FDA's prompt acceptance of the resubmission and look forward to working with the Agency throughout its review in order to bring this treatment option to the Sanfilippo syndrome community as quickly as possible."

During its prior late-cycle review, the FDA acknowledged that the neurodevelopmental outcome data are robust and that the biomarker data provide additional supportive evidence. The updated long-term clinical data included in the BLA and presented at WORLD*Symposium* ™ 2026 represent up to 8 years of follow-up and show further clinical improvement relative to the decline observed in natural history, and a durable treatment effect across clinical evaluations and multiple biomarkers, while maintaining an acceptable safety profile.

The FDA granted the UX111 BLA Priority Review in February 2025. If approved, UX111 will be manufactured entirely within the U.S., at Andelyn Biosciences in Columbus, Ohio and the Ultragenyx gene therapy manufacturing facility in Bedford, Massachusetts.

About UX111 (rebisufligene etisparvovec)
UX111 (rebisufligene etisparvovec) is a novel in vivo AAV9 gene therapy in Phase 1/2/3 development for Sanfilippo syndrome Type A (MPS IIIA), a rare fatal lysosomal storage disease with no approved treatment that primarily affects the brain. The therapy is designed to address the underlying sulfamidase (SGSH) enzyme deficiency responsible for abnormal accumulation of heparan sulfate (HS), a glycosaminoglycan, in the brain that results in progressive cell damage and neurodegeneration. UX111 is dosed in a one-time intravenous infusion using a self-complementary AAV9 vector to deliver a functional copy of the SGSH gene to cells. These transduced cells then secrete the functional enzyme into the tissue fluid where it can be taken up by surrounding neurons and other cells. The enzyme is taken up efficiently into other cells and is then routed to the lysosome where it can reduce the accumulation of the HS and prevent the progression of lysosomal storage and consequential injury that occurs in untreated patients. The product was originally developed by Abeona Therapeutics and transferred to Ultragenyx to complete development. The UX111 program has received Regenerative Medicine Advanced Therapy, Fast Track, Rare Pediatric Disease, and Orphan Drug designations in the U.S., and PRIME and Orphan medicinal product designations in the EU.

About Sanfilippo Syndrome Type A (MPS IIIA)
Sanfilippo syndrome Type A (MPS IIIA) is a rare, fatal lysosomal storage disease with no approved treatment that primarily affects the brain and is characterized by rapid neurodegeneration, with onset in early childhood. Children with MPS IIIA present with global developmental delay, which eventually leads to progressive cognitive, language and motor decline, behavioral abnormalities and early death. MPS IIIA is estimated to affect approximately 3,000 to 5,000 patients in commercially accessible geographies, with a median life expectancy of 15 years. MPS IIIA is caused by biallelic pathogenic variants in the SGSH gene that lead to a deficiency in the sulfamidase (SGSH) enzyme responsible for breaking down heparan sulfate (HS), a glycosaminoglycan, which accumulates in cells throughout the body, resulting in the observed rapid neurodegeneration that is associated with the disorder.

About Ultragenyx
Ultragenyx is a biopharmaceutical company committed to bringing novel products to patients for the treatment of serious rare and ultra-rare genetic diseases. The company has built a diverse portfolio of approved therapies and product candidates aimed at addressing diseases with high unmet medical need and clear biology for treatment, 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 the development, regulatory status, review, timing and potential approval of UX111 (rebisufligene etisparvovec), including the anticipated PDUFA action date, the potential for accelerated approval, the outcome of the FDA's review of the resubmitted Biologics License Application (BLA), the timing and outcome of any regulatory inspections, the sufficiency of clinical, biomarker and long-term follow-up data to support regulatory approval, expectations regarding the safety, tolerability and durability of UX111, manufacturing readiness and capabilities, including at third-party and company-owned manufacturing facilities, and the potential availability and commercial launch of UX111, 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 actual results to differ materially from those expressed or implied by the forward-looking statements. These risks and uncertainties include, among others, the uncertainty and unpredictability inherent in clinical drug development and the regulatory review and approval process; the possibility that the FDA may not accept or agree that the submitted data are

sufficient to support accelerated or full approval of UX111; the risk that additional data, analyses or studies may be required; the timing, scope and outcome of regulatory inspections; risks related to the manufacture of UX111, including reliance on third-party manufacturers and the Company's limited experience operating its own manufacturing facilities; the ability of the Company and its manufacturing partners to comply with regulatory requirements; potential safety or tolerability issues; competition from other therapies or products; smaller than anticipated market opportunities; and other risks related to the Company's ability to fund operations, future operating results and financial performance.

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-/).

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