Ultragenyx Resubmits Biologics License Application for UX111 AAV Gene Therapy to Treat Sanfilippo Syndrome Type A (MPS IIIA) to U.S. FDA

January 30, 2026

Company expects up to six-month review period per FDA guidelines

New longer-term clinical data demonstrating durable positive brain biochemical and clinical effect for as long as 8.5 years to be presented at WORLDSymposium™ 2026

NOVATO, Calif., Jan. 30, 2026 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE) today announced that it has resubmitted its 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) to the U.S. Food and Drug Administration (FDA or the Agency). The submission contains substantial longer-term data on multiple measures of neurologic benefit to support an intermediate clinical endpoint for accelerated approval supported further by CSF heparan sulfate and other biomarker data, as agreed with the FDA during the last clinical review.

“Today, with no approved treatment options to address the relentless progression of Sanfilippo syndrome type A, families must watch helplessly as their children lose the ability to communicate, play, move, and even eat before ultimately succumbing to this devastating and fatal disease,” said Emil D. Kakkis, M.D., Ph.D., chief executive officer and president of Ultragenyx. “We recognize the extraordinary stakes facing the Sanfilippo community as they await a first-ever treatment option and look forward to working with the Agency as it completes its review of this urgently needed therapy. There is no time to waste, and we believe we have addressed all of the Agency’s concerns to avoid further delays. Patients, and their families, need access now.”

The resubmitted BLA includes comprehensive responses to chemistry, manufacturing, and controls (CMC)-related observations outlined in a Complete Response Letter (CRL) issued in July 2025, as well as additional long-term clinical data from current patients as requested by the Agency in the CRL.

During its prior review, the FDA acknowledged that the neurodevelopmental outcome data are robust and that the biomarker data provide additional supportive evidence; updated clinical data included in the BLA representing an additional year of follow-up continue to show a durable treatment effect across multiple biomarkers and further clinical separation from natural history, while maintaining an acceptable safety profile. Detailed updates will be presented next week at the WORLD*Symposium* ™ 2026 in San Diego.

In February 2025, the FDA granted the UX111 BLA Priority Review. A Prescription Drug User Fee Act (PDUFA) action date is expected to be assigned within a month of resubmission. The company anticipates up to a 6-month review period from the date of resubmission per FDA regulations, with a PDUFA date expected in the third quarter of 2026. If approved, UX111 will be the first approved therapy for Sanfilippo syndrome type A.

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, 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 heparan sulfate 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, a glycosaminoglycans, which accumulate 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 Ultragenyx's ability to provide the requested documentation and address the comments in the CRL to the satisfaction of the FDA, the development, timing and progress of UX111, including the timing of FDA acceptance of the BLA resubmission and the timing of FDA review of any such resubmission, the timing and

outcome of any FDA inspections related to UX111, the timing of future regulatory interactions related to UX111, including the outcome of the BLA resubmission, business plans and objectives for UX111, expectations regarding the tolerability and safety of UX111, and future clinical and regulatory developments for 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 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 UX111, 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, smaller than anticipated market opportunities for the company's products and product candidates, manufacturing risks, our limited experience in operating our own manufacturing facility, the ability of the company and its third party manufacturers to comply with regulatory requirements, 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 Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) on November 4, 2025, 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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