Ultragenyx Submits Biologics License Application to the U.S. FDA for UX111 AAV Gene Therapy for the Treatment of Sanfilippo Syndrome Type A (MPS IIIA)

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If approved, UX111 would be the first approved therapy in the U.S. for Sanfilippo Syndrome Type A

NOVATO, Calif., Dec. 19, 2024 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE) today announced the submission of a Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA or the Agency) seeking accelerated approval for UX111 (ABO-102) AAV gene therapy as a treatment for patients with Sanfilippo syndrome type A (MPS IIIA).

“The path to get a treatment to the point of a BLA filing has been long and perilous for the Sanfilippo community. They have had to watch their children, once thriving, lose their ability to speak and walk, and eventually die, while research programs were shelved due to regulatory and funding hurdles,” said Emil D. Kakkis, M.D., Ph.D., chief executive officer and president of Ultragenyx. “We commend the FDA’s detailed evaluation and acceptance of cerebral spinal fluid (CSF) heparan sulfate (HS) as a well-characterized biomarker to support an accelerated approval pathway for mucopolysaccharidoses (MPS) disorders, including Sanfilippo syndrome. The FDA’s acceptance of CSF HS, which we define as a disease-cause biomarker since it measures the underlying disease, enabled us to file our BLA and may unlock the future accelerated approvals of a host of new therapies for these devastating MPS diseases that affect the brain.”

Earlier this year, Ultragenyx reached agreement with the Agency that CSF HS can be used as a surrogate endpoint for accelerated approval based on the body of data presented by the company, along with a consortium of academics and other industry sponsors at a workshop hosted by the Reagan-Udall Foundation for the FDA in February 2024.

The BLA submission for UX111 is supported by available data, including from the ongoing pivotal *Transpher A* study, demonstrating treatment with UX111 resulted in rapid and sustained decreased levels of HS in CSF in patients with Sanfilippo syndrome type A, and that sustained reduction in CSF HS exposure over time was correlated with improved long-term cognitive development compared to the decline observed during the same period of time in natural history data. The most frequently reported treatment-related adverse events to date were elevations in liver enzymes, and the majority of these events were mild (Grade 1) or moderate (Grade 2) in severity and all resolved.

About UX111
UX111 is a novel in vivo 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. UX111 is designed to be dosed in a one-time intravenous infusion using a self-complementary AAV9 vector to deliver a functional copy of the *SGSH* gene to cells. The therapy is designed to address the underlying SGSH enzyme deficiency responsible for abnormal accumulation of heparan sulfate, a glycosaminoglycan, in the brain that results in progressive cell damage and neurodegeneration. 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 language and cognitive 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 therapies to patients for the treatment of serious rare and ultrarare 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 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, 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 6, 2024, 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-/mycompany/).

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