Ultragenyx Announces Plans to File for Accelerated Approval of UX111 for the Treatment of Sanfilippo Syndrome Type A (MPS IIIA)

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Agreement reached with FDA that cerebral spinal fluid (CSF) heparan sulfate (HS) can be used as a reasonable surrogate endpoint for accelerated approval

Data from existing clinical studies appear sufficient for a biologics license application (BLA) submission

NOVATO, Calif., June 12, 2024 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE) today announced that the company held a successful meeting with the U.S. Food and Drug Administration (FDA or the Agency) during which the company reached agreement with the Agency that cerebral spinal fluid *(*CSF) heparan sulfate (HS) is a reasonable surrogate endpoint that could support submission of a biologics license application (BLA) seeking accelerated approval for UX111 (ABO-102) AAV gene therapy for the treatment of Sanfilippo syndrome (MPS IIIA). The company will need to finalize details of its BLA with the Agency in a pre-BLA meeting with the intent to file late this year or early next.

“Gaining alignment with the FDA that CSF HS is a relevant biomarker to enable accelerated approval in Sanfilippo syndrome is a pivotal moment for the community and paves the way for treatments for all fatal types of neuronopathic mucopolysaccharidoses,” said Emil D. Kakkis, M.D., Ph.D., chief executive officer and president of Ultragenyx. “We commend the FDA for appreciating the critical urgency to deliver potentially life-saving therapies to children with neurologically devastating diseases and we extend our gratitude to the patient and caregiver advocates, scientists and industry leaders that shared and collaborated to provide the comprehensive evidence needed to support this important decision.”

As discussed with the FDA, the BLA filing will be based on the available data including from the ongoing pivotal *Transpher A* study evaluating the safety and efficacy of UX111 in children with MPS IIIA. Results from the *Transpher A* and long-term follow-up studies were recently presented at the 20th Annual WORLDSymposium™.

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 central nervous system (CNS). 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 CNS 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 Pharmaceutical Inc.
Ultragenyx is a biopharmaceutical company committed to bringing novel products to patients for the treatment of serious rare and ultrarare 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.

Ultragenyx 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 May 3, 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-/).

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