Ultragenyx Announces Orphan Drug Designation for UX701 for the Treatment of Wilson Disease

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NOVATO, Calif., Dec. 09, 2020 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development and commercialization of novel products for rare and ultra-rare genetic diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation to UX701 for the treatment of Wilson disease, a larger rare metabolic disease that affects more than 50,000 people in the developed world.

“FDA Orphan Drug Designation is an important milestone in the development of UX701, a gene therapy for the treatment of Wilson Disease, and highlights the significant unmet medical need for people with this genetic disorder,” stated Eric Crombez, M.D., Chief Medical Officer of the Ultragenyx Gene Therapy development unit. “Current treatment options involve the often complicated and lifetime use of medications that block the absorption of copper from the diet or remove copper by chelation. UX701 is designed to directly address the underlying cause of disease by restoring normal copper metabolism in the liver. By correcting copper trafficking and removal, this one-time treatment has the potential to better address the many serious effects of this disease and improve the lives of patients.”

UX701 is currently in late preclinical development, and an investigational new drug (IND) application is expected to be filed with the FDA by the end of the year.

About Orphan Drug Designation

The FDA Orphan Drug Designation program provides orphan status to drugs and biologics that are intended for the safe and effective treatment, diagnosis, or prevention of rare diseases that affect fewer than 200,000 people in the U.S. Among the benefits of orphan designation in the U.S. are seven years of market exclusivity following FDA approval, waiver or partial payment of application fees, and tax credits for clinical testing expenses conducted after orphan designation is received.

About Wilson Disease

Wilson disease is a rare inherited disorder caused by mutations in the ATP7B gene, which results in deficient production of ATP7B, a protein that transports copper. Loss of function of this copper-binding protein results in the accumulation of copper in the liver and other tissues, most notably the central nervous system. Patients with Wilson disease experience hepatic, neurologic and/or psychiatric problems. Those with liver disease can experience such symptoms as fatigue, lack of appetite, abdominal pain and jaundice, and can progress to fibrosis, cirrhosis, life-threatening liver failure and death. Wilson disease can be treated by reducing copper absorption or removing excess copper from the body using life-long chelation therapy, but unmet needs exist because some treated patients experience clinical deterioration and severe side effects. Wilson disease affects more than 50,000 individuals in the developed world.

About UX701

UX701 is an investigational AAV type 9 gene therapy designed to deliver stable expression of the ATP7B copper transporter following a single intravenous infusion. It has been shown in preclinical studies to normalize copper trafficking and excretion from the body. UX701 was granted Orphan Drug Designation in the United States.

About Ultragenyx Pharmaceutical Inc.

Ultragenyx is a biopharmaceutical company committed to bringing patients novel products 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.

Ultragenyx Forward-Looking Statements

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 business plans and objectives for UX701 and future clinical developments for UX701 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 effects from the COVID-19 pandemic on the company's clinical development programs, business and operating results, the timing of submissions to regulatory authorities and the timing and likelihood of regulatory approvals for our product candidates, the timing of clinical trial activities and reporting results from same, the availability or commercial potential of Ultragenyx's products and drug 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. 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 on October 27, 2020, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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