Ultragenyx Announces Approval of Dojolvi™ (UX007/triheptanoin) in Canada for the Treatment of Long-chain Fatty Acid Oxidation Disorders in Adults and Children

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First Approved Treatment for Adult and Pediatric Patients with Long-chain Fatty Acid Oxidation Disorders (LC-FAOD) in Canada

NOVATO, Calif., Feb. 17, 2021 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development and commercialization of novel products for serious rare and ultra-rare genetic diseases, today announced that Health Canada has approved Dojolvi™ (triheptanoin) as a source of calories and fatty acids for the treatment of adult and pediatric patients with long-chain fatty acid oxidation disorders (LC-FAOD). Long-chain fatty acid oxidation disorders (LC-FAOD) are a group of rare, genetic, life-threatening disorders caused by defects in the enzymes needed to produce energy from fatty acids. Dojolvi is a highly purified, synthetic, 7-carbon fatty acid triglyceride specifically designed to provide medium-chain, odd-carbon fatty acids as an energy source and metabolite replacement for people with LC-FAOD.

“Living with a serious disease like LC-FAOD comes with unique challenges such as management of caloric intake and around-the-clock monitoring of symptom deterioration and/or fatigue. We are pleased to bring Canadian patients with LC-FAOD an approved therapy to help them manage their disease,” said Camille L. Bedrosian, M.D., Chief Medical Officer of Ultragenyx. “This would not have been possible without the LC-FAOD community, including all the patients, families and physicians who participated in the clinical trials. Similar to the approach we took in the United States to ensure access following FDA approval of Dojolvi, our goal is to ensure that patients in Canada with LC-FAOD who might benefit from Dojolvi will have access to it.”

LC-FAOD are a group of autosomal recessive genetic disorders characterized by metabolic deficiencies in which the body is unable to convert long-chain fatty acids into energy. The inability to produce energy from fat can lead to marked depletion of glucose in the body and serious complications, which can lead to hospitalizations or early death. LC-FAOD are included in newborn screening panels across Canada due to the risk of serious outcomes, including death early in life. Other current treatment options for LC-FAOD include avoidance of fasting, low-fat/high-carbohydrate diets, carnitine and even-carbon medium-chain triglyceride (MCT) oil, a medical food product. LC-FAOD affect an estimated 8,000 to 14,000 children and adults in the developed world, including Canada.

“The number of options available to treat patients with rare long chain fatty oxidation defects have been quite limited for many years,” said Dr. Pranesh Chakraborty Associate Professor, Department of Pediatrics, University of Ottawa, Executive Director of Newborn Screening Ontario, and Co-Lead of the Canadian Inherited Metabolic Diseases Research Network. “Clinicians and patients, who have been working with researchers over many years to study the safety and efficacy of Dojolvi, are very excited to have this new therapy available to help patients in Canada.”

Ultragenyx expects Dojolvi to be available to patients in Canada in April and will be working closely with private insurers and through the public drug plan process to pursue coverage for Dojolvi as quickly as possible. To support access, Ultragenyx’s UltraCare® program helps patients and caregivers understand insurance coverage. The UltraCare program may also assist patients in finding support for access to Ultragenyx medicines, including Dojolvi. Dedicated UltraCare Nurse Case Managers are available Monday through Friday from 8 a.m. to 8 p.m. Eastern Time at 1 -833-388-5872 (U-LTRA).

About Dojolvi ™ (triheptanoin)
Dojolvi is a highly purified, pharmaceutical-grade, odd-carbon medium-chain triglyceride consisting of three 7-carbon fatty acids on a glycerol backbone created via a multi-step chemical process. It is designed to provide medium-chain, odd-carbon fatty acids as an energy source and metabolite replacement for people with LC-FAOD.

Dojolvi is indicated as a source of calories and fatty acids for the treatment of adult and pediatric patients with long-chain fatty acid oxidation disorders (LC-FAOD).

For important safety information, please consult the Dojolvi Product Monograph at www.ultragenyx.com/canada/medicines/dojolvi-product-monograph-CANADA/

About Ultragenyx Pharmaceutical Inc.

Ultragenyx is a biopharmaceutical company committed to bringing to 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.

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 future operating results and financial performance, anticipated cost or expense reductions, the timing, progress and plans for its clinical programs and clinical studies, future regulatory interactions, and the components and timing of regulatory submissions 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 activities, business and operating results, risks related to reliance on third party partners to conduct certain activities on the company's behalf, uncertainty and potential delays related to clinical drug development, 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 Annual Report on Form 10-K filed with the Securities and Exchange Commission on February 12, 2021, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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