Ultragenyx Announces Approval of Dojolvi® (triheptanoin) in Brazil for the Treatment of Long-chain Fatty Acid Oxidation Disorders in Adults and Children

August 23, 2021

Dojolvi is the only approved therapy for this rare, life-threatening metabolic disorder

NOVATO, Calif., Aug. 23, 2021 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development of novel products for serious rare and ultra-rare genetic diseases, today announced that Brazil's National Health Surveillance Agency (ANVISA) has approved Dojolvi® (triheptanoin) as a source of calories and fatty acids for the treatment of pediatric and adult patients with molecularly confirmed long-chain fatty acid oxidation disorders (LC-FAOD).

"Patients with LC-FAOD face frequent hospitalizations and major medical events with limited options to support management of their disease," said Eduardo Thompson, Senior Vice President and Regional Head, Latin America at Ultragenyx. "This approval of Dojolvi is critical to providing therapeutic options for patients in Brazil and we are now working with urgency to obtain reimbursement approval in order to support broad access to therapy. We are grateful to the community of patients, caregivers and physicians whose efforts have contributed to this important approval."

Dojolvi is also approved by the U.S. Food and Drug Administration (FDA) and by Health Canada for the treatment of adult and pediatric patients with LC-FAOD. Outside of the U.S., patients have had access to Dojolvi through named patient and early access programs.

About LC-FAOD
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 severe depletion of glucose in the body and serious complications, which can lead to hospitalizations or early death. 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 and are included in newborn screening panels in various geographies due to the risk for serious outcomes including death early in life. The Brazilian government recently sanctioned law 14.154/2021 that expands the list of diseases to be included in the National Newborn Screening Program carried out in the Unified Health System (SUS), including LC-FAOD. The implementation will take place in five stages within a period to be regulated by the Ministry of Health, with LC-FAOD included at the second stage.

About Dojolvi
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.

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

IMPORTANT SAFETY INFORMATION
WARNINGS AND PRECAUTIONS
Feeding Tube Dysfunction

- Feeding tube dysfunction was reported in patients receiving triheptanoin. The contribution of Dojolvi cannot be ruled out. Dojolvi should not be administered in feeding tubes manufactured of polyvinyl chloride (PVC). The feeding tube should be monitored regularly to ensure proper functioning and integrity.

Intestinal Malabsorption in Patients with Pancreatic Insufficiency

- Low or absent pancreatic enzymes may result in reduced absorption of heptanoate subsequently leading to insufficient supplementation of medium-chain fatty acids. Administration of Dojolvi should be avoided in patients with pancreatic insufficiency.

ADVERSE REACTIONS
Gastrointestinal (GI)

- The most common GI-related adverse reactions reported in the pooled safety population of Studies 1 and 2 were abdominal pain (abdominal discomfort, abdominal pain, abdominal distension, abdominal pain upper, GI pain) [60%], diarrhea [44%], vomiting [44%], and nausea [14%].

DRUG INTERACTIONS

Pancreatic Lipase Inhibitors

- Co-administration should be avoided due to potential for reduced clinical effect of Dojolvi.

USE IN SPECIFIC POPULATIONS
Pregnancy and Lactation

- There are no available data on triheptanoin use in pregnant women to evaluate for a drug-associated risk of major birth defects, miscarriage, or adverse maternal or fetal outcomes. Women are advised to report pregnancies to Ultragenyx Pharmaceutical Inc. at 1-888-756-8657 (U.S.) or 0800-770-4481 (Brazil).
- There are no data on the presence of triheptanoin or its metabolites in human or animal milk, the effects on the breastfed infant, or the effects on milk production.

PATIENT COUNSELING INFORMATION
The patient or caregiver should:

- Read the FDA/ANVISA-approved patient labeling, which includes information on the appropriate oral or feeding tube preparation, administration, and storage.
- Regularly inspect the feeding tube for proper functioning and integrity and report to the healthcare provider if any issues are identified.
- Be informed that pancreatic insufficiency may reduce the clinical effect of Dojolvi and to report any known pancreatic insufficiency to the healthcare provider.

In Brazil, you may report side effects to Ultragenyx Pharmaceutical Inc. using the local toll-free number at 0800-770-4481 or by email at ultragenyx@primevigilance.com, or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch

Please see Prescribing Information for physicians and the patient package insert (both in Portuguese) for a complete discussion of the risks associated with Dojolvi. The texts are based on the U.S. Prescribing Information and the indication is the same.

About Ultragenyx Pharmaceutical Inc.

Ultragenyx is a biopharmaceutical company committed to bringing novel therapies to patients for the treatment of serious rare and ultra-rare 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

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, its commercialization, marketing and manufacturing capabilities and strategy 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 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 commercialization activities, business and operating results, 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, uncertainties related to insurance coverage and reimbursement status of the company's newly approved products, the company's evolving integrated commercial organization 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 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 on August 3, 2021, and its subsequent periodic reports filed with the Securities and Exchange Commission.

Contacts
Ultragenyx Pharmaceutical Inc.
Investors
Joshua Higa
415-660-0951

Media
Carolyn Wang
415-225-5050

Ultragenyx Brazil Offices
0800-770-4481
Vitor Brandao, Regulatory Affairs Director Brazil

vbrandao@ultragenyx.com
Rua Josefina, 200, Guarulhos
Sao Paulo, 07093-080, Brazil