# Ultragenyx Announces U.S. Commercial Launch of Dojolvi™ (triheptanoin), the First FDA-Approved Therapy for the Treatment of Long-chain Fatty Acid Oxidation Disorders

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**Established UltraCare® Program Provides Ongoing Patient Support Services**

NOVATO, Calif., July 22, 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 diseases, today announced that Dojolvi™ (triheptanoin) is now commercially available. The U.S. Food and Drug Administration (FDA) approved Dojolvi on June 30, 2020 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), a group of rare, lifelong, and life-threatening genetic disorders in which the body is unable to convert long-chain fatty acids into energy.

“Dojolvi, the only FDA-approved therapy for LC-FAOD, is now available for patients living with this serious, unpredictable, and often catastrophic disease,” said Erik Harris, Chief Commercial Officer of Ultragenyx. “We are now focused on ensuring that all patients in the U.S. with LC-FAOD who might benefit from Dojolvi will have access to it.”

To support access, Ultragenyx’s UltraCare® program helps patients and caregivers understand insurance coverage and assists them in finding financial support for Ultragenyx medicines, including Dojolvi, and for the administration of them. Dedicated in-house UltraCare Guides are available Monday through Friday from 9 a.m. to 8 p.m. Eastern Time at 888-756-8657.

**About Long-chain Fatty Acid Oxidation Disorders (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. LC-FAOD are included in newborn screening panels across the U.S. and in certain European countries due to the risk for 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 2,000 to 3,500 children and adults in the United States.

**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.
- 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-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.

You may report side effects to Ultragenyx Pharmaceutical Inc. at 1-888-756-8657 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

Please see full Prescribing Information, including the Patient Information leaflet, for a complete discussion of the risks associated with Dojolvi.

**About Ultragenyx**
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 commercialization activities, business and operating results, 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, 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 on May 7, 2020, and its subsequent periodic reports filed with the Securities and Exchange Commission.*

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