Ultragenyx Announces Early Access to triheptanoin for long-chain fatty acid oxidation disorders (LC-FAOD) in the United Kingdom.

Positive scientific opinion through Early Access to Medicines Scheme (EAMS) recognises significant unmet need for adults and children of all ages.

London, 14 November 2025 – Ultragenyx Pharmaceutical Inc., a biopharmaceutical company focused on the development and commercialisation of novel therapies for rare and ultra-rare genetic diseases, today announces that earlier this year (April 2025), the UK's Medicines and Healthcare products Regulatory Agency (MHRA) awarded a positive scientific opinion under the Early Access to Medicines Scheme (EAMS) for triheptanoin, a source of calories and fatty acids for the treatment of adult and paediatric patients, of all ages, with long-chain fatty acid oxidation disorders (LC-FAOD). This positive opinion means that individuals living with LC-FAOD, that meet the eligibility criteria, can gain early pre-license access to triheptanoin, while Ultragenyx pursues a Marketing Authorisation Application.[1]

The aim of the EAMS is to provide earlier availability of promising unlicensed medicines to UK patients that have a high unmet clinical need. The medicines included in the scheme are those that are intended to treat, diagnose or prevent seriously debilitating or life-threatening conditions where there are no adequate treatment options. It should be noted that positive scientific opinion is not a recommendation for use of the medicine and should not be interpreted as such.[1] Medicines under EAMS that receive marketing authorisation by the MHRA as well as a positive assessment by the National Institute of Health and Care Excellence (NICE) also benefit from accelerated NHS England commissioning.

"LC-FAOD are rare inherited disorders that prevent the body from breaking down long-chain fatty acids into energy, and this EAMS designation for triheptanoin represents a significant milestone in providing a potential new treatment option for eligible patients," commented Dr. Nin Dass, Medical Director, UK, IRE & Nordics at Ultragenyx.

"LC-FAOD present significant clinical challenges with limited treatment options in the UK. This positive EAMS scientific opinion for triheptanoin represents an important step in addressing the unmet medical need for these patients," said Dr. Stephanie Grünewald, Consultant in Inherited Metabolic Diseases at Great Ormond Street Hospital.

"Our LC-FAOD community may benefit from this positive step in accessing this promising treatment. Early access is a vital tool for our rare communities," stated Kirsty Hoyle, CEO, Metabolic Support UK.

About LC-FOAD

LC-FAOD are a group of rare, life-threatening and seriously debilitating genetic disorders with a high unmet need and no approved treatment in the UK. Patients with LC-FAOD may have difficulty producing enough energy because of their body's inability to use long-chain fats as an energy source. This can put patients at risk for medical emergencies, especially during times of illness, fasting, or prolonged exercise.[1] Current management options in the UK include avoidance of fasting and a reduced long-chain fat diet with even-chain medium-chain triglyceride supplementation.

Further information is available at metabolicsupportuk.org.

About Triheptanoin

Triheptanoin is a pharmaceutical-grade, highly purified medium-chain triglyceride that serves as a source of calories and fatty acids for the treatment of paediatric and adult patients with LC-FAOD. By providing odd-chain, seven-carbon fatty acids, triheptanoin helps bypass the long-chain FAOD enzyme deficiencies to support energy production and replacement. It is manufactured via a multi-step chemical process and supplied as a colourless to light-yellow clear oil.

Triheptanoin is currently approved in the United States, Canada, Mexico, Brazil and Kuwait.

About Ultragenyx

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 commercialisation of rare disease therapeutics. Ultragenyx's strategy is predicated upon time- and cost-efficient drug development, with the goal of delivering innovative therapies to patients with the utmost urgency.

For more information on Ultragenyx, please visit https://www.ultragenyx.eu/uk/.

References

1. MHRA. *Early Access to Medicines Scientific Opinion – Public Assessment Report: Triheptanoin (Dojolvi)*. 07 April 2025. EAMS 41104/0001. Available at: MHRA EAMS Triheptanoin Report

Forward-Looking Statements and Use of Digital Media

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 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, risks related to adverse side effects, risks related to reliance on third party partners to conduct certain activities on the company's behalf, the potential for any license or collaboration agreement, including the company's collaboration agreement with Regeneron to be terminated, smaller than anticipated market opportunities for the company's products and product candidates, manufacturing risks, competition from other therapies or products, market acceptance of the company's products, risks related to international expansion of the company's business, uncertainties related to insurance coverage and reimbursement status of newly approved 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 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 August 6, 2025, 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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Date of prep: October 2025
Job code: UK-MRCNP-UX007-00109