Medicines & Healthcare products Regulatory Agency

MHRA

# RENEWAL of Early Access to Medicines Scientific Opinion – ANNEX to Public Assessment Report. The renewal is effective from 7th April 2026 and is valid for 12 months.

**Triheptanoin**
**As treatment for adult and paediatric patients with long-chain fatty acid oxidation disorders (LC-FAOD).**

**Ultragenyx Netherlands B.V.**
**EAMS number 41104/0001**

## Introduction

The aim of the Early Access to Medicines Scheme (EAMS) is to provide earlier availability of promising new unlicensed medicines to UK patients that have a high unmet clinical need. The MHRA scientific opinion provides benefit and risk information to doctors who may wish to prescribe the unlicensed medicine under their own responsibility. More information about the scheme can be found here: http://www.mhra.gov.uk/Howweregulate/Innovation/EarlyaccesstomedicinesschemeEAMS/index.htm

The scientific opinion is based on the information supplied to the MHRA on the benefits and risks of a promising new medicine. As such this is a scientific opinion and should not be regarded as a medicine licensed by the MHRA or a future commitment by the MHRA to license such a medicine. The General Medical Council's guidance on prescribing unlicensed medicines can be found here: https://www.gmc-uk.org/guidance/28349.asp

## Background

An EAMS scientific opinion was granted by the MHRA on 7th April 2025 for triheptanoin as treatment of adults and children with long-chain fatty acid oxidation disorders (LC-FAOD), a group of rare inherited disorders. The basis of the decision is described in the Public Assessment Report:

https://www.gov.uk/government/publications/triheptanoin-in-the-treatment-of-long-chain-fatty-acid-oxidation-disorders-lc-faod

## EAMS Scientific Opinion Renewal

In April 2026, the MHRA considered the Company's request for a renewal of the EAMS Scientific Opinion for triheptanoin for a further twelve months from 7th April 2026. The basis of this request was to continue making triheptanoin available to LC-FAOD patients while a National marketing authorisation application to the MHRA is underway for the same indication.

The MHRA noted that no new safety issues have been identified in the EAMS reporting period.

The MHRA concluded that benefit-risk remains positive on the basis of the available data, and recommends to renew the EAMS whilst a marketing authorisation is being sought.

**The EAMS scientific opinion is renewed from 7th April 2026 for a further twelve months. The medicine will continue to be subject to the compulsory EAMS reporting requirements, with periodic reporting of adverse event data. The Company is also obliged to inform the MHRA of any alteration in benefit-risk.**