Medicines and Healthcare products Regulatory Agency (MHRA): Early Access to Medicines Scheme (EAMS)

Summary of operational arrangements for Triheptanoin in NHS Scotland. EAMS number 41104/0001

The aim of the MHRA Early Access to Medicines Scheme (EAMS) is to provide earlier availability of promising new unlicensed and 'off label' medicines to UK patients that have a high unmet clinical need. The medicinal products 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.

The MHRA EAMS positive opinion should not be regarded as a medicine licensed by the MHRA or a future commitment by the MHRA to license such a medicine. The opinion and EAMS documentation published by the MHRA are intended only to inform clinicians' decision making and not to recommend use. Under EAMS the risk and legal responsibility for prescribing an EAMS medicine remains with the prescribing clinician.

More information about the scheme can be found on the MHRA website.

Availability of triheptanoin during EAMS period

Triheptanoin is made available free of charge for patients via EAMS during the EAMS period. This document summarises how the medicine can be used in Scotland.

Medicine

Triheptanoin

Indication and patient population

Triheptanoin is indicated for the treatment of paediatric and adult patients with long-chain fatty acid oxidation disorders (LC-FAOD).

Conditions for entry to EAMS

The EAMS makes free-of-charge triheptanoin for use in long-chain fatty acid oxidation disorders (LC-FAOD) available to Health Boards (subject to approval by Ultragenyx Netherlands B.V.) provided that the following conditions are met:

- The patient (who is the subject of each order for EAMS Triheptanoin) is a paediatric or adult patient with LC-FAOD.
- The Health Board does not have any policies, guidelines or procedures in place which prohibit the acceptance of EAMS medicines.
- While a valid EAMS positive opinion is in place (Marketing Authorisation expected Q1 2026 at the earliest).

Detailed patient eligibility information is included in the MHRA's Treatment Protocol – Information for Healthcare Professionals.

Supply arrangements

- Clinicians wishing to access treatment for their patients with Triheptanoin need to contact the Ultragenyx by email at UX007_EarlyAccess@ultragenyx.com .
- As for all unlicensed medicines and off-label uses of licensed medicines, individual health boards will also have local governance arrangements in place to authorise medicines supplied via EAMS. This review should be expedited as a rigorous risk/ benefit assessment has already been conducted by MHRA and can be accessed in the Public Assessment Report (PAR).
- You can find more information about supply arrangements on the MHRA website:

  Public Assessment Report

  Treatment Protocol – Information for Healthcare Professionals

  Treatment Protocol – Information for Patients

  Treatment Protocol – Information on the Pharmacovigilance System

  Information for NHS Medical Directors

Practical considerations

Triheptanoin is supplied as clear, colourless to light yellow oral liquid. The recommended target daily dosage for adults and paediatric patients is 25-35% of the patient's total prescribed Daily Caloric Intake (DCI) divided into at least four doses and administered by mixing thoroughly into semi-solid

food/liquid or medical food/formula at mealtimes or with snacks. See treatment protocol for further detail.

Prepare or administer triheptanoin using containers or oral syringes made of compatible materials such as stainless steel, glass, high density polyethylene (HDPE), polypropylene, low density polyethylene, polyurethane, and silicone.

Do not prepare or administer triheptanoin using containers or oral syringes made of polystyrene or polyvinyl chloride (PVC) plastics.

Regularly monitor the containers, dosing components, or utensils that are in contact with triheptanoin to ensure proper functioning and integrity.

No specific monitoring required.

This medicinal product does not require any special storage conditions. Store in the original container. Do not freeze.

The product will be delivered directly to hospitals. There is no homecare option available for this medicine during the EAMS program.

Pharmacovigilance and data collection

Clinicians are required to report to Ultragenyx using specific documentation and recording mechanisms.

Specific details relating to pharmacovigilance can be found in the Treatment Protocol for Pharmacovigilance.

EAMS termination arrangements/exit strategy

In the absence of an MHRA 'winding down' period, no new patients will be allowed to enroll following Marketing Authorisation of Triheptanoin for the treatment of patients with LC-FAOD. The EAMS scheme will close in line with the MHRA regulations, and no new patients will be allowed to enroll onto the scheme to access free of charge supply.

New patients can continue to be enrolled if the EAMS enters the MHRA managed 'winding down period.' However, no new patients may be enrolled once the MHRA 'winding down period' is terminated.

Access to treatment for new patients would be via local board processes from the point of licensing until Scottish Medicines Consortium (SMC) accepted advice is issued or, where relevant, until availability via the ultra-orphan pathway is confirmed by Scottish Government.

The provision of Triheptanoin, free of charge via EAMS for any one patient enrolled in the EAMS shall end, on the earliest of the following to occur:

A) In the event that SMC accepted advice is issued for this medicine and indication, 30 days after the publication of SMC accepted advice on their website.

or

B) In the event that SMC not recommended advice is issued:

I. until such time as Ultragenyx may receive SMC accepted advice for this medicine and indication (e.g. after a resubmission).

II. until such time that the patient:

a) Completes the defined course length of treatment

b) No longer derives clinical benefit (e.g. disease progression or unacceptable toxicity)

or

C) In the event that Marketing Authorisation is not granted, until such time as the patient no longer derives clinical benefit (disease progression or unacceptable toxicity).

or

D) In the event of availability through the ultra-orphan pathway, 30 days after Scottish Government confirmation.