MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

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Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan (MHRA-100654-PIP01-22)

MHRA-100654-PIP01-22 -M01

Scope of the Application

Active Substance(s)

triheptanoin

Condition(s)

Treatment of long-chain fatty acid oxidation disorders (LC-FAOD)

Pharmaceutical Form(s)

Oral liquid

Route(s) of Administration

ORAL USE ENTERAL USE

Name / Corporate name of the PIP applicant

Ultragenyx Germany GmbH

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Ultragenyx Germany GmbH submitted to the licensing authority on 19/09/2025 20:23 BST an application for a Modification

The procedure started on 04/11/2025 14:08 GMT

1. The licensing authority, having assessed the application in accordance with the Human Medicines Regulations 2012, decides, as set out in the appended summary report:

to accept change(s) to the agreed paediatric investigation plan

2. The measures and timelines of the paediatric investigation plan are set out in the Annex I.

This decision is forwarded to the applicant, together with its annex and appendix.

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Final Decision Letter

MHRA-100654-PIP01-22 -M01

Of 27/01/2026 14:28 GMT

On the adopted decision for triheptanoin (MHRA-100654-PIP01-22 -M01) in accordance with the Human Medicines Regulations 2012.

The licensing authority, in accordance with the Human Medicines Regulations 2012, has adopted this decision:

Agreement on modification of a paediatric investigation plan (including modification of a waiver or deferral included in that paediatric investigation plan)

This decision applies to a Modification for triheptanoin, Oral liquid , ORAL USE; ENTERAL USE .

This decision is addressed to Ultragenyx Germany GmbH, Rahel-Hirsch-Str. 10, Berlin, GERMANY, 10557

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of long-chain fatty acid oxidation disorders (LC-FAOD)

2.2 Indication(s) targeted by the PIP:

Treatment of long-chain fatty acid oxidation disorders (LC-FAOD)

2.3 Subset(s) of the paediatric population concerned by the paediatric development:

All subsets of the paediatric population from birth to less than 18 years of age

2.4 Pharmaceutical Form(s):

Oral liquid

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	2	Study 1 (P_0876_41) Stability of Dojolvi (UX007) emulation with feeding tubes) Study 2 (VV-10677) Dojolvi (UX007) In-Use Food Stability Report.
Non-Clinical Studies	2	Study 3 (UX007-PC002) 9-Month Oral (Dietary) Toxicity and Toxicokinetic Study of Triheptanoin in Juvenile Yucatan Minipigs. Study 4 (UX007-PC017) Pre- and Post-Natal Development (PPND; Segment III) Toxicity study of Triheptanoin in Rats.
Clinical Studies	6	Study 5 (UX007-CL201) Prospective, interventional, open-label Phase 2 study to evaluate the impact of triheptanoin on acute clinical pathophysiology associated with long-chain fatty acid oxidation disorders (LC-FAOD). Study 6 (UX007-CL202) Prospective, interventional, open-label, long-term, Phase 2, 3-cohort study to evaluate the long-term safety and efficacy of triheptanoin in subjects with long-chain fatty acid oxidation disorders (LC-FAOD). Study 7 (NCT01379625) Randomised, double-blind Investigator-initiated Phase 2 study to determine if triheptanoin therapy (an odd-chain fatty acid triglyceride) has a therapeutic advantage over conventional treatment for long-chain fatty acid oxidation disorders. Gillingham et al., 2017 Study 8

		(UX007-CL001) Retrospective medical record review study comparing clinical outcomes before and after triheptanoin initiation in LC-FAOD patients to evaluate the therapeutic benefit and safety of triheptanoin treatment. Study 9 (UX007-CL003) Retrospective chart review study to assess the clinical outcome of triheptanoin treatment in patients with long-chain fatty acid oxidation disorders (LC-FAOD) treated under expanded access program. Study 10 UX007-CL302) Double-blind, randomised, active-controlled trial to evaluate safety and efficacy of triheptanoin compared to even-chain MCT in children from birth to less than 18 years of age with long-chain fatty acid oxidation disorders.
Extrapolation, Modeling & Simulation Studies	1	Study 11 Population PK Modelling of Heptanoate in Healthy Subjects and Patients with Long-Chain Fatty Acid Oxidation Disorders (LC-FAOD) Administered with Triheptanoin
Other Studies	0	Not applicable.
Other Measures	0	Not applicable.

3. Follow-up, completion and deferral of a PIP:

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	30/06/2027
Deferral of one or more studies contained in the paediatric investigation plan:	Yes