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-100681-PIP01-22)

MHRA-100681-PIP01-22-M02

Scope of the Application

Active Substance(s)

Lomitapide (as lomitapide mesylate)

Condition(s)

Treatment of heterozygous and homozygous familial hypercholesterolaemia

Pharmaceutical Form(s)

Capsule, hard

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

Chiesi Ltd

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Chiesi Ltd submitted to the licensing authority on 21/05/2025 13:10 BST an application for a Modification

The procedure started on 14/07/2025 10:49 BST

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-100681-PIP01-22-M02

Of 29/07/2025 09:34 BST

On the adopted decision for LOMITAPIDE MESYLATE (MHRA-100681-PIP01-22-M02) 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 LOMITAPIDE MESYLATE, Capsule, hard , ORAL USE .

This decision is addressed to Chiesi Ltd, 333 Styal Road, Manchester, UNITED KINGDOM, M22 5LG

ANNEX I

1. Waiver

1.1 Condition:

Treatment of (heterozygous or homozygous) familial hypercholesterolaemia The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 5 years of age Pharmaceutical form(s): Capsule, hard Route(s) of administration: ORAL USE Reason for granting waiver: on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of (heterozygous or homozygous) familial hypercholesterolaemia

2.2 Indication(s) targeted by the PIP:

Treatment of homozygous familial hypercholesterolaemia

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

The paediatric population from 5 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Capsule, hard

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Deleted during procedure MHRA-100681-PIP01-22
Non-Clinical Studies	1	Study 2 (AEGR-733PC0031) Juvenile rat toxicity study to assess the potential effects of lomitapide on postnatal growth and development, reproductive development and neurobehavioral development.
Clinical Studies	1	Study 3 (APH-19) Single-arm, open-label, international, multi-centre study to evaluate the efficacy and long-term safety of lomitapide in paediatric patients with homozygous familial hypercholesterolaemia (HoFH) on stable lipid-lowering therapy.
Extrapolation, Modeling & Simulation Studies	0	Not applicable.
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:	Yes
Date of completion of the paediatric investigation plan:	31/08/2024
Deferral of one or more studies contained in the paediatric investigation plan:	Yes