EMA/697719/2022

European Medicines Agency decision

P/0362/2022

of 19 September 2022

on the acceptance of a modification of an agreed paediatric investigation plan for lomitapide (Lojuxta), (EMEA-001124-PIP01-10-M05) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

Disclaimer

This decision does not constitute entitlement to the rewards and incentives referred to in Title V of Regulation (EC) No 1901/2006.

Only the English text is authentic.

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The European Medicines Agency,

Having regard to the Treaty on the Functioning of the European Union,

Having regard to Regulation (EC) No 1901/2006 of the European Parliament and of the Council of 12 December 2006 on medicinal products for paediatric use and amending Regulation (EEC) No. 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004[1],

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the European Medicines Agency's decision P/0306/2011 issued on 20 December 2011, decision P/0018/2014 issued on 22 January 2014, decision P/0312/2014 issued on 26 November 2014, decision P/0282/2015 issued on 27 November 2015, and decision P/0332/2019 issued on 10 September 2019,

Having regard to the application submitted by Amryt Pharmaceuticals DAC on 25 April 2022 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 22 July 2022, in accordance with Article 22 of Regulation (EC) No 1901/2006,

Having regard to Article 25 of Regulation (EC) No 1901/2006,

Whereas:

(1) The Paediatric Committee of the European Medicines Agency has given an opinion on the acceptance of changes to the agreed paediatric investigation plan and to the deferral.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan, including changes to the deferral.

[1] OJ L 378, 27.12.2006, p.1, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for lomitapide (Lojuxta), capsule, hard, oral use, including changes to the deferral, are hereby accepted in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

Article 2

This decision is addressed to Amryt Pharmaceuticals DAC, 45 Mespil Road, D04 W2F1 - Dublin 4, Ireland.

EMA/PDCO/245061/2022
Amsterdam, 22 July 2022

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMEA-001124-PIP01-10-M05

Scope of the application

Active substance(s):

Lomitapide

Invented name:

Lojuxta

Condition(s):

Treatment of (heterozygous and homozygous) familial hypercholesterolaemia

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Capsule, hard

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Amryt Pharmaceuticals DAC

Information about the authorised medicinal product:

See Annex II

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Amryt Pharmaceuticals DAC submitted to the European Medicines Agency on 25 April 2022 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/0306/2011 issued on 20 December 2011, decision P/0018/2014 issued on

22 January 2014, decision P/0312/2014 issued on 26 November 2014, decision P/0282/2015 issued on 27 November 2015, and decision P/0332/2019 issued on 10 September 2019.

The application for modification proposed changes to the agreed paediatric investigation plan and to the deferral.

The procedure started on 23 May 2022.

Scope of the modification

Some measures and timelines of the Paediatric Investigation Plan have been modified.

A pharmaceutical form was deleted.

Opinion

1. The Paediatric Committee, having assessed the application in accordance with Article 22 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree to changes to the paediatric investigation plan and to the deferral in the scope set out in the Annex I of this opinion.

   The Paediatric Committee member of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the paediatric investigation plan and the subset(s) of the paediatric population and condition(s) covered by the waiver are set out in the Annex I.

This opinion is forwarded to the applicant and the Executive Director of the European Medicines Agency, together with its annexes and appendix.

Annex I

The subset(s) of the paediatric population and condition(s) covered by the waiver and the measures and timelines of the agreed paediatric investigation plan (PIP)

1. Waiver

1.1. Condition:

Treatment of (heterozygous or homozygous) familial hypercholesterolaemia

The waiver applies to:

- all subsets of the paediatric population from birth to less than 5 years of age;
- capsule, hard, oral use;
- 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:

Treatment of (heterozygous or homozygous) familial hypercholesterolaemia

2.1.1. Indication(s) targeted by the PIP

Treatment of homozygous familial hypercholesterolaemia

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From 5 years to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Capsule, hard

2.1.4. Measures

Area	Description
Quality	**Study 1 (study deleted during procedure EMEA-001124-PIP01-10-M05)**
Non-clinical	**Study 2:** Juvenile rat toxicity study to assess the potential effects of lomitapide on postnatal growth and development, reproductive development and neurobehavioral development. (AEGR-733PC0031)
Clinical	**Study 3:** Single-arm, open-label, multi-centre trial to evaluate the efficacy and long-term safety of lomitapide in paediatric patients with homozygous familial hypercholesterolaemia on stable lipid-lowering therapy. (APH-19).
Extrapolation, modelling and simulation studies	Not applicable

Other studies	Not applicable
Other measures	Not applicable

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By August 2024
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

1. Treatment of homozygous familial hypercholesterolaemia.

Authorised indication(s):

- Lojuxta is indicated as an adjunct to a low-fat diet and other lipid-lowering medicinal products with or without low density lipoprotein (LDL) apheresis in adult patients with homozygous familial hypercholesterolaemia (HoFH). Genetic confirmation of HoFH should be obtained whenever possible. Other forms of primary hyperlipoproteinemia and secondary causes of hypercholesterolaemia (e.g., nephrotic syndrome, hypothyroidism) must be excluded.

Authorised pharmaceutical form(s):

Capsule, hard

Authorised route(s) of administration:

Oral use