

EMA/667883/2014

European Medicines Agency decision

P/0312/2014

of 26 November 2014

on the acceptance of a modification of an agreed paediatric investigation plan for lomitapide (Lojuxta), (EMA-001124-PIP01-10-M02) 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 726/2004¹,

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency²,

Having regard to the European Medicines Agency's decision P/0306/2011 issued on 20 December 2011, and the decision P/0018/2014 issued on 22 January 2014,

Having regard to the application submitted by Aegerion Pharmaceuticals SAS on 18 July 2014 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 10 October 2014, 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.

² OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for lomitapide (Lojuxta), capsule, hard, age-appropriate oral form, 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 Aegerion Pharmaceuticals SAS, 37-39, avenue Ledru Rollin, 75012 - Paris, France.

Done at London, 26 November 2014

For the European Medicines Agency
Zaide Frias
Head of Division (ad interim)
Human Medicines Research and Development Support
(Signature on file)



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/PDCO/463979/2014

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

EMA-001124-PIP01-10-M02

Scope of the application

Active substance(s):

Lomitapide

Invented name:

Lojuxta

Condition(s):

Treatment of (heterozygous or homozygous) familial hypercholesterolaemia

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Capsule, hard

Age-appropriate oral form

Route(s) of administration:

Oral use

Name / corporate name of the PIP applicant:

Aegerion Pharmaceuticals SAS

Information about the authorised medicinal product:

See Annex II



Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Aegerion Pharmaceuticals SAS submitted to the European Medicines Agency on 18 July 2014 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, and the decision P/0018/2014 issued on 22 January 2014.

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

The procedure started on 13 August 2014.

Scope of the modification

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

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 Icelandic and the Norwegian Paediatric Committee members agree 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.

London, 10 October 2014

On behalf of the Paediatric Committee
Dr Dirk Mentzer, Chairman
(Signature on file)

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

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;
- for capsule, hard and age-appropriate oral form, 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; age appropriate oral form

2.1.4. Measures

Area	Number of measures	Description
Quality	1	Study 1: Development of an age-appropriate oral form (different from the hard capsule).
Non-clinical	1	Study 2: Juvenile rat toxicity study to assess the potential effects of lomitapide on postnatal growth and development, reproductive development and neurobehavioral development.
Clinical	1	Study 3: Randomized, double-blind, placebo-controlled trial in patients with homozygous familial hypercholesterolaemia (HoFH) on stable lipid-lowering therapy, followed by an open-label, single arm extension phase.

Extrapolation, modelling and simulation studies	0	Not applicable.
Other studies	0	Not applicable.
Other measures	0	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 May 2019
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).

Authorised pharmaceutical form(s):

Capsule, hard

Authorised route(s) of administration:

Oral use