



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/941395/2011

European Medicines Agency decision P/306/2011

of 20 December 2011

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for lomitapide (EMA-001124-PIP01-10) 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 application submitted by Aegerion Pharmaceuticals on 10 May 2011 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 11 November 2011, in accordance with Article 18 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 agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver.
- (2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.
- (3) It is therefore appropriate to adopt a decision granting a deferral.
- (4) It is therefore appropriate to adopt a decision granting a waiver.

¹ OJ L 378, 27.12.2006, p.1.

² OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

A paediatric investigation plan for lomitapide, capsule, hard, age-appropriate oral form, oral use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

Article 2

A deferral for lomitapide, capsule, hard, age-appropriate oral form, oral use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

A waiver for lomitapide, capsule, hard, age-appropriate oral form, oral use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 4

This decision is addressed to Aegerion Pharmaceuticals, 101 Main Street, Suite 1850, 02142 - Cambridge, MA, USA.

Done at London, 20 December 2011

For the European Medicines Agency
Guido Rasi
Executive Director
(Signature on file)



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/PDCO/755669/2011

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a deferral and a waiver

EMA-001124-PIP01-10

Scope of the application

Active substance(s):

Lomitapide

Condition(s):

Treatment of heterozygous and homozygous familial hypercholesterolaemia

Pharmaceutical form(s):

Capsule, hard

Age-appropriate oral form

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Aegerion Pharmaceuticals

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Aegerion Pharmaceuticals submitted for agreement to the European Medicines Agency on 10 May 2011 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 15 June 2011.

Supplementary information was provided by the applicant on 26 August 2011. The applicant proposed modifications to the paediatric investigation plan.



Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

- to agree the paediatric investigation plan in accordance with Article 18 of said Regulation,
- to grant a deferral in accordance with Article 21 of said Regulation,
- to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(a) of said Regulation, on the grounds that the specific medicinal product is likely to be ineffective or unsafe in part or all of the paediatric population and with Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

The Norwegian and Icelandic Paediatric Committee members agree with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed 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, 11 November 2011

On behalf of the Paediatric Committee
Dr Daniel Brasseur, 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 and homozygous familial hypercholesterolaemia

The waiver applies to:

- All subsets of the paediatric population from birth to less than 8 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

The waiver applies to:

- Patients with heterozygous familial hypercholesterolaemia of all age subsets of the paediatric population from birth to less than 18 years of age;
- for capsule, hard and age-appropriate oral form, oral use;
- on the grounds that the specific medicinal product is likely to be unsafe.

2. Paediatric Investigation Plan

2.1. Condition: Treatment of heterozygous and 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 8 years to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Capsule, hard; age appropriate oral form

2.1.4. Studies

Area	Number of studies	Description
Quality	1	Study 1: Development of an age-appropriate oral form (different from the hard capsule).
Non-clinical	0	Not applicable.
Clinical	1	Study 2:

Area	Number of studies	Description
		Single-arm, open-label, multi-centre trial to evaluate the activity and long-term safety and tolerability of lomitapide in paediatric patients with homozygous familial hypercholesterolaemia on stable lipid-lowering therapy.

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 March 2017
Deferral for one or more studies contained in the paediatric investigation plan:	Yes