

EMA/406248/2018

European Medicines Agency decision

P/0185/2018

of 17 July 2018

on the acceptance of a modification of an agreed paediatric investigation plan for bempedoic acid (EMEA-001872-PIP01-15-M01) 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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on the acceptance of a modification of an agreed paediatric investigation plan for bempedoic acid (EMA-001872-PIP01-15-M01) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

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 P/0094/2017 issued on 11 April 2017,

Having regard to the application submitted by Esperion Therapeutics, Inc. on 7 March 2018 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 1 June 2018, 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.
- (2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

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 bempedoic acid, film-coated tablet, age-appropriate oral liquid dosage form, oral use, 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 Esperion Therapeutics, Inc., 3891 Ranchero Drive, Suite 150, 48108 - Ann Arbor, MI, United States.

EMA/PDCO/164095/2018

London, 1 June 2018

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

EMA-001872-PIP01-15-M01

Scope of the application

Active substance(s):

Bempedoic acid

Condition(s):

Treatment of elevated cholesterol

Pharmaceutical form(s):

Film-coated tablet

Age-appropriate oral liquid dosage form

Route(s) of administration:

Oral use

Name / corporate name of the PIP applicant:

Esperion Therapeutics, Inc.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Esperion Therapeutics, Inc. submitted to the European Medicines Agency on 7 March 2018 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/0094/2017 issued on 11 April 2017.

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

The procedure started on 3 April 2018.

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 in the scope set out in the Annex I of this opinion.

The Norwegian Paediatric Committee member 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 annex 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)

Waiver

1.1. Condition:

Treatment of elevated cholesterol

The waiver applies to:

- the paediatric population from birth to less than 4 years;
- film-coated tablet, age-appropriate formulation, 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 elevated cholesterol

2.1.1. Indication(s) targeted by the PIP

Treatment of heterozygous and homozygous familial hypercholesterolemia.

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

From 4 to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Film-coated tablet

Age-appropriate formulation

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	1	Study 1: Development of an age-appropriate formulation.
Non-clinical	2	Study 2: Dose range-finding juvenile rat toxicity study to evaluate potential adverse effects of bempedoic acid on developing organ systems in juvenile rats beginning on PND 15 until adulthood, and to determine the relevant dose(s) for the definitive juvenile rat study.

Area	Number of measures	Description
		Study 3: Definitive juvenile rat toxicity study to evaluate potential toxicity of bempedoic acid on developing organ systems in juvenile rats from PND 15 (prepubertal) through sexual maturity to support administration of bempedoic acid to children of at least 4 years of age.
Clinical	3	Study 4: Open-label, un-controlled, dose escalating PK/PD study to evaluate the dose and exposure/response relationship, safety and tolerability of bempedoic acid when added to stable background lipid modifying therapy in patients from 6 to less than 18 years of age with heterozygous familial hypercholesterolaemia.(1002-041) Study 5: Double-blind, placebo-controlled, parallel-group, efficacy and safety study to evaluate the effect of bempedoic acid versus placebo when added to baseline lipid-modifying therapy on percent change from baseline to Week 12 in LDL-C in patients from 6 to less than 18 years of age with heterozygous familial hypercholesterolaemia. (1002-042) Study 6: Open-label, un-controlled, PK/PD, safety and activity study to evaluate the dose and exposure/response relationship, tolerability, safety and activity of bempedoic acid when added to stable background lipid modifying therapy in patients from 4 to less than 18 years of age with homozygous and compound heterozygous familial hypercholesterolaemia (HoFH and ChEhFH). (1002-051)
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/efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By November 2024
Deferral for one or more measures contained in the paediatric investigation plan:	Yes