EMA/170999/2023

European Medicines Agency decision

P/0170/2023

of 15 May 2023

on the acceptance of a modification of an agreed paediatric investigation plan for elosulfase alfa (Vimizim), (EMEA-000973-PIP01-10-M04) 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/90/2011 issued on 8 April 2011, decision P/0240/2012 issued on 22 October 2012, and decision P/0055/2015 issued on 30 March 2015,

Having regard to the application submitted by BioMarin International Limited on 16 December 2022 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 31 March 2023, 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, 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 or elosulfase alfa (Vimizim), concentrate for solution for infusion, intravenous 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 BioMarin International Limited, Shanbally, Ringaskiddy, P43 R298 - County Cork, Ireland.

EMA/PDCO/3872/2023
Amsterdam, 31 March 2023

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

EMEA-000973-PIP01-10-M04

Scope of the application

Active substance(s):

Elosulfase alfa

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of mucopolysaccharidosis, type IVA (Morquio A Syndrome)

Pharmaceutical form(s):

Concentrate for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

BioMarin International Limited

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, BioMarin International Limited submitted to the European Medicines Agency on 16 December 2022 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/90/2011 issued on 8 April 2011, the decision P/0240/2012 issued on 22 October 2012, and the decision P/0055/2015 issued on 30 March 2015.

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

The procedure started on 30 January 2023.

Scope of the modification

Some measures or 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 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 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

Not applicable.

2. Paediatric Investigation Plan

2.1. Condition

Treatment of mucopolysaccharidosis, type IVA (Morquio A Syndrome)

2.1.1. Indication(s) targeted by the PIP

Treatment of mucopolysaccharidosis, type IVA (Morquio A Syndrome).

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

From birth to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Concentrate for solution for infusion.

2.1.4. Measures

Area	Description
Quality-related studies	Not applicable.
Non-clinical studies	Study 1: Embryo-foetal development in rabbits (dose range finding). Study 2: Combined fertility and embryo-foetal developmental study in rats. Study 3: Embryo-foetal development in rabbits. Study 4: Peri- and post-natal developmental study in rats. Study 5: Repeat dose toxicity and toxicokinetics study in monkey.
Clinical studies	Study 6: Open-label, multicentre, dose-escalation safety study in children from 5 to less than 18 years. Study 7: Open-label, multicentre, extension study in adults and children from 5 years of age. Study 8: Double-blind, randomised, multicentre, 3-arm, placebo-controlled, efficacy and safety study in adults and children from 5 years of age. Study 9: Open-label, multicentre, safety and efficacy study in children under 5 years of age. Study 10: Multicentre, extension study in children from 5 years to less than 18 years of age (and adults).

Extrapolation, modelling and simulation studies	Not applicable.
Other studies	Not applicable.
Other measures	Not applicable.
Extrapolation, modelling and simulation studies	Not applicable.

3. Follow-up, completion and deferral of PIP

Measures to address long term follow-up of potential safety/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By April 2017
Deferral for one or more studies contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

Condition(s) and authorised indication(s):

1. Treatment of mucopolysaccharidosis, type IVA (Morquio A Syndrome)

Authorised indication(s):

- Vimizim is indicated for the treatment of mucopolysaccharidosis, type IVA (Morquio A Syndrome, MPS IVA) in patients of all ages.

Authorised pharmaceutical form(s):

Concentrate for solution for infusion

Authorised route(s) of administration:

Intravenous use