EMA/195738/2015

European Medicines Agency decision

P/0055/2015

of 30 March 2015

on the acceptance of a modification of an agreed paediatric investigation plan for recombinant human N-acetylgalactosamine-6-sulfatase (BMN110) Vimizim (elosulfase alfa) (EMEA-000973-PIP01-10-M03) 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 Community procedures for the authorisation and supervision of medicinal products for human and veterinary 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 and the decision P/0240/2012 issued on 22 October 2012,

Having regard to the application submitted by BioMarin Europe Limited on 25 September 2014 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 16 January 2015, 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, following a re-examination procedure of the Paediatric Committee's opinion according to Article 25(3) Regulation (EC) No 1901/2006, 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 recombinant human N-acetylgalactosamine-6-sulfatase (BMN110) Vimizim (elosulfase alfa), concentrate for solution for infusion, intravenous 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 BioMarin Europe Limited, 164 Shaftesbury Avenue, WC2H 8HL – London United Kingdom.

Done at London, 30 March 2015

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

EMA/PDCO/136580/2015
London, 20 March 2015

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

EMEA-000973-PIP01-10-M03

Scope of the application

Active substance(s):

Recombinant human N-acetylgalactosamine-6-sulfatase (BMN110)

Invented name:

Vimizim (elosulfase alfa)

Condition(s):

Treatment of mucopolysaccharidosis, type IVA (Morquio A Syndrome)

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Concentrate for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

BioMarin Europe Limited

Information about the authorised medicinal product:

See Annex II

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Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, BioMarin Europe Limited submitted to the European Medicines Agency on 25 September 2014 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 and the decision P/0240/2012 issued on 22 October 2012.

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

An Opinion was adopted by the Paediatric Committee on 16 January 2015 for the above mentioned product. BioMarin Europe Limited received the Paediatric Committee Opinion on 27 January 2015.

On 25 February 2015 BioMarin Europe Limited submitted to the European Medicines Agency a written request including detailed grounds for a re-examination of the Opinion.

The re-examination procedure started on 26 February 2015.

Scope of the modification

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

Final Opinion

1. The Paediatric Committee, having assessed the detailed grounds for re-examination, in accordance with Article 25(3) of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

1.1. to maintain its opinion and

- to agree to the changes regarding the measures and the timelines of the paediatric investigation plan and the timelines of 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 agreed 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	Number of studies	Description
Quality-related studies	None	Not applicable.
Non-clinical studies	5	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	5	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).

Area	Number of studies	Description
Extrapolation, modelling and simulation studies	0	Not applicable.
Other studies	0	Not applicable.
Other measures	0	Not applicable.
Extrapolation, modelling and simulation studies	0	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

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