EMA/67795/2020

European Medicines Agency decision

P/0060/2020

of 10 February 2020

on the acceptance of a modification of an agreed paediatric investigation plan for vosoritide (BMN 111), (EMEA-002033-PIP01-16-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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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/0379/2017 issued on 19 December 2017,

Having regard to the application submitted by BioMarin International Limited on 9 September 2019 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 11 December 2019, 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) of Regulation (EC) No 1901/2006, 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 vosoritide (BMN 111), powder for solution for injection, subcutaneous 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, Cork, Ireland.

EMA/PDCO/8855/2020
Amsterdam, 31 January 2020

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

EMEA-002033-PIP01-16-M01

Scope of the application

Active substance(s):

Vosoritide (BMN 111)

Condition(s):

Treatment of achondroplasia

Pharmaceutical form(s):

Powder for solution for injection

Route(s) of administration:

Subcutaneous 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 9 September 2019 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0379/2017 issued on 19 December 2017.

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

An Opinion was adopted by the Paediatric Committee on 11 December 2019 for the above mentioned product. BioMarin International Limited received the Paediatric Committee Opinion on 6 January 2020.

On 8 January 2020 BioMarin International 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 9 January 2020.

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 revise its opinion and

    - to agree to the changes regarding the measures and the timelines of the paediatric investigation plan in the scope set out in the Annex I of this opinion.

    1.2. following re-examination, to amend the scope of the modifications of the paediatric investigation plan.

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 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)

1. Waiver

Not applicable

2. Paediatric investigation plan

2.1. Condition

Treatment of achondroplasia

2.1.1. Indication(s) targeted by the PIP

Treatment of achondroplasia

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)

Powder for solution for injection

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable
Non-clinical studies	1	**Study 1** Bio-distribution study in rat (BMN111-17-027)
Clinical studies	7	**Study 2** Open-label, sequential cohort dose-escalation study to evaluate the safety, efficacy and pharmacokinetics of BMN 111 in children with achondroplasia (111-202) **Study 3** Open-label extension study to evaluate the long-term safety, tolerability, and efficacy of BMN 111 in children with achondroplasia (111-205) **Study 4** Randomised, placebo-controlled, double-blind multicentre study to evaluate the efficacy, safety and tolerability of BMN 111 versus placebo in children from 5 to less than 18 years of age with achondroplasia (111-301)

		Study 5 Multicentre, multinational study to collect specific growth measurements on pediatric patients with achondroplasia (111-901) **Study 6** Randomised, placebo-controlled, double-blind multicentre study to assess the efficacy, safety, tolerability and pharmacokinetics of BMN 111 vs placebo in children from birth to less than 5 years of age with achondroplasia (111-206) **Study 7** Multicentre open-label extension of Study 4 (111-301) to evaluate the long-term safety and efficacy of BMN 111 in children from 5 to less than 18 years of age with achondroplasia (Extension Study 1) **Study 8** Multicentre open-label extension of Study 6 (111-206) to evaluate the long-term safety and efficacy of BMN 111 in children from birth to less than 5 years of age with achondroplasia (Extension Study 2)
Extrapolation, modelling and simulation studies	1	**Study 9** Extrapolation of efficacy to paediatric subjects from birth to less than 5 years of age with achondroplasia, based on data from paediatric subjects from 5 to less than 18 years of age with achondroplasia (Extrapolation Study 1)
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:	Yes
Date of completion of the paediatric investigation plan:	By December 2027
Deferral for one or more measures contained in the paediatric investigation plan:	Yes