EMA/344245/2024

European Medicines Agency decision

P/0286/2024

of 16 August 2024

on the agreement of a paediatric investigation plan and on the granting of a deferral for vosoritide (Voxzogo), (EMEA-002033-PIP02-23) 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 application submitted by BioMarin International Limited on 13 October 2023 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 28 June 2024, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 of said Regulation,

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.

(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.

[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

A paediatric investigation plan for vosoritide (Voxzogo), powder and solvent for solution for injection, subcutaneous 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 vosoritide (Voxzogo), powder and solvent for solution for injection, subcutaneous 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

This decision covers all conditions, indications, pharmaceutical forms, routes of administration, measures, timelines, waivers and deferrals, as agreed in the decision P/0379/2017 issued on 19 December 2017, including subsequent modifications thereof.

Article 4

This decision is addressed to BioMarin International Limited, Shanbally, Ringaskiddy, P43 R298 – Cork, Ireland.

EMA/PDCO/149893/2024 Corr[1]
Amsterdam, 28 June 2024

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

EMEA-002033-PIP02-23

Scope of the application

Active substance(s):

Vosoritide

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of hypochondroplasia

Pharmaceutical form(s):

Powder and solvent 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 16(1) of Regulation (EC) No 1901/2006 as amended, BioMarin International Limited submitted for agreement to the European Medicines Agency on 13 October 2023 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 20 November 2023.

Supplementary information was provided by the applicant on 22 March 2024. The applicant proposed modifications to the paediatric investigation plan and withdrew its request for a waiver.

[1] 7 August 2024.

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 17(1) of said Regulation;
   - to grant a deferral in accordance with Article 21 of said Regulation.

   The Paediatric Committee member of Norway agrees 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 hypochondroplasia

2.1.1. Indication(s) targeted by the PIP

Treatment of hypochondroplasia

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 and solvent for solution for injection

2.1.4. Measures

Area	Description
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	**Study 1 (BMN 111-902)** Multi-centre, multinational, prospective/retrospective observational study to evaluate natural history of the disease in children and adolescents from birth to less than 15 years of age with a genetically confirmed FGFR3 pathogenic variant associated with hypochondroplasia. **Study 2 (BMN 111-303)** Randomized, double-blind, placebo-controlled, multi-centre study to evaluate the safety and efficacy of vosoritide in children from 3 years to less than 18 years of age with genetically confirmed FGFR3 pathogenic variant associated with hypochondroplasia. **Study 3 (Extension Study 1)** Open label extension study to evaluate the long-term efficacy and safety profile of vosoritide treatment in eligible participants with hypochondroplasia who have completed 1 year of vosoritide or placebo treatment in PIP study 2 (BMN111-303).

	Study 4 (BMN 111-212) Randomized, double-blind, placebo-controlled, multi-centre study to evaluate the safety and efficacy of vosoritide in infants and young children from birth to less than 36 months of age with genetically confirmed FGFR3 pathogenic variant associated with hypochondroplasia **Study 5 (Extension Study 2)** Open label extension study to evaluate the long-term efficacy and safety profile of vosoritide treatment in eligible participants with genetically confirmed FGFR3 pathogenic variant associated with hypochondroplasia who have completed 1 year of vosoritide or placebo treatment in PIP study 4 (BMN111-212)
Modelling and simulation analyses	Not applicable
Other studies	Not applicable
Extrapolation plan	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 November 2044
Deferral for one or more measures 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 achondroplasia

Authorised indication(s): Voxzogo is indicated for the treatment of achondroplasia in patients 4 months of age and older whose epiphyses are not closed. The diagnosis of achondroplasia should be confirmed by appropriate genetic testing

- Treatment of achondroplasia
  - Invented name(s): Voxzogo
  - Authorised pharmaceutical form(s): Powder and solvent for solution for injection
  - Authorised route(s) of administration: Subcutaneous use
  - Authorised via centralised procedure