EMADOC-1700519818-1854312

European Medicines Agency decision

EMA/PE/0000224768

of 28 January 2025

on the acceptance of a modification of an agreed paediatric investigation plan for vosoritide (Voxzogo) 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 vosoritide (Voxzogo) 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 726/2004[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/0286/2024 issued on 16 August 2024,

Having regard to the application submitted by Biomarin International Limited on 6 September 2024 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 13 December 2024, 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 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, 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 for vosoritide (Voxzogo), 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 International Limited, Shanbally, P43 - R298 Ringaskiddy, Ireland.

EMADOC-1700519818-1710349 Corr[1]
Amsterdam, 13 December 2024

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

EMA/PE/0000224768

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 22 of Regulation (EC) No 1901/2006 as amended, Biomarin International Limited submitted to the European Medicines Agency on 6 September 2024 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0286/2024 issued on 16 August 2024.

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

The procedure started on 14 October 2024.

[1] 22 January 2025

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 and to the deferral in the scope set out in the Annex I of this opinion.

   The Paediatric Committee members of Liechtenstein and Norway agree 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 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 (BMN 111-308)** 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 and 4 (BMN111-303 and BMN 111-212).

	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) – removed during procedure EMA/PE/0000224768***
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 January 2042
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