



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMADOC-1700519818-2115791

European Medicines Agency decision

EMA/PE/0000241327

of 16 May 2025

on the acceptance of a modification of an agreed paediatric investigation plan for palopegteriparatide (Yorvipath) 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 palopegteriparatide (Yorvipath) 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 726/2004¹,

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 Agency²,

Having regard to the European Medicines Agency's decision P/0021/2022 issued on 31 January 2022,

Having regard to the application submitted by Ascendis Pharma Bone Diseases A/S on 19 December 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 28 March 2025, 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 palopegteriparatide (Yorvipath), 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 Ascendis Pharma Bone Diseases A/S, Tuborg Boulevard 12, 2900 - Hellerup Hovedstaden, Denmark.



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMADOC-1700519818-1847129

Amsterdam, 28 March 2025

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA/PE/0000241327

Scope of the application

Active substance(s):

Palopegteriparatide

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of hypoparathyroidism

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Subcutaneous use

Name/corporate name of the PIP applicant:

Ascendis Pharma Bone Diseases A/S

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Ascendis Pharma Bone Diseases A/S submitted to the European Medicines Agency on 19 December 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/0021/2022 issued on 31 January 2022

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

The procedure started on 27 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 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 hypoparathyroidism

2.1.1. Indication(s) targeted by the PIP

Treatment of hypoparathyroidism

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)

Solution for injection

2.1.4. Measures

Area	Description
Quality-related studies	Study 1 Development of a junior pen presentation Study 2 Modification of existing adult pens to make them suitable for paediatric use
Non-clinical studies	Not applicable
Clinical studies	Study 3 Multicentre, open-label, single arm study to assess the efficacy, safety, pharmacokinetics (PK), and tolerability of palopegteriparatide in children from birth to less than 12 years of age with hypoparathyroidism Study 4 <i>Study added during procedure EMA/PE/0000241327</i> Multicentre, open-label, single arm study to assess the efficacy, safety, pharmacokinetics (PK), and tolerability of palopegteriparatide in adolescents from 12 years to less than 18 years of age with hypoparathyroidism
Extrapolation, modelling and simulation studies	Not applicable

Other studies	Not applicable
Other measures	Not applicable

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By October 2029
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 chronic hypoparathyroidism

- Authorised indication(s): Yorvipath is a parathyroid hormone (PTH) replacement therapy indicated for the treatment of adults with chronic hypoparathyroidism.
 - Invented name(s): Yorvipath
 - Authorised pharmaceutical form(s): solution for injection in pre-filled pen
 - Authorised route(s) of administration: Solution for injection (injection)
 - Authorised via centralised procedure