MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

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Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral

MHRA-101232-PIP01-23

Scope of the Application

Active Substance(s)

palopegteriparatide

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 the Decision

Pursuant to the Human Medicines Regulations 2012, Ascendis Pharma Bone Diseases A/S submitted to the licensing authority on 07/11/2023 16:51 GMT an application for a Paediatric Investigation Plan

The procedure started on 16/11/2023 08:24 GMT

1. The licensing authority, having assessed the application in accordance with the Human Medicines Regulations 2012, decides, as set out in the appended summary report:

to agree a paediatric investigation plan and grant a deferral

2. The measures and timelines of the paediatric investigation plan are set out in the Annex I.

This decision is forwarded to the applicant, together with its annex and appendix.

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Final Decision Letter

MHRA-101232-PIP01-23

Of 05/12/2023 12:14 GMT

On the adopted decision for palopegteriparatide (MHRA-101232-PIP01-23) in accordance with the Human Medicines Regulations 2012.

The licensing authority, in accordance with the Human Medicines Regulations 2012, has adopted this decision:

Agreement on a paediatric investigation plan

This decision applies to a Paediatric Investigation Plan for palopegteriparatide, Solution for injection , SUBCUTANEOUS USE .

This decision is addressed to Ascendis Pharma Bone Diseases A/S, Tuborg Boulevard 12, Hellerup 2900, Hellerup, DENMARK, 2900

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of hypoparathyroidism

2.2 Indication(s) targeted by the PIP:

Treatment of hypoparathyroidism

2.3 Subset(s) of the paediatric population concerned by the paediatric development:

The paediatric population from birth to less than 18 years of age

2.4 Pharmaceutical Form(s):

Solution for injection

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	2	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	0	Not applicable.
Clinical Studies	1	Study 3 A multicentre, open-label, single arm study to assess the efficacy, safety, pharmacokinetics (PK), and tolerability of palopegteriparatide in children from birth to less than 18 years of age with hypoparathyroidism.
Extrapolation, Modeling & Simulation Studies	0	Not applicable.
Other Studies	0	Not applicable.
Other Measures	0	Not applicable.

3. Follow-up, completion and deferral of a PIP:

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	31/10/2026
Deferral of one or more studies contained in the paediatric investigation plan:	Yes