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-101602-PIP01-24

Scope of the Application

Active Substance(s)

navepegritide

Condition(s)

Treatment of Achondroplasia

Pharmaceutical Form(s)

Powder and solvent for solution for injection

Route(s) of Administration

SUBCUTANEOUS USE

Name / Corporate name of the PIP applicant

Ascendis Pharma Growth Disorders A/S

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Ascendis Pharma Growth Disorders A/S submitted to the licensing authority on 25/09/2024 14:33 BST an application for a Paediatric Investigation Plan

The procedure started on 05/11/2024 09:14 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-101602-PIP01-24

Of 21/01/2025 15:43 GMT

On the adopted decision for navepegritide (MHRA-101602-PIP01-24) 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 navepegritide, Powder and solvent for solution for injection , SUBCUTANEOUS USE .

This decision is addressed to Ascendis Pharma Growth Disorders 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 achondroplasia

2.2 Indication(s) targeted by the PIP:

Treatment of achondroplasia

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):

Powder and solvent for solution for injection

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	7	Study 1 (TCC-NHS-01) Multicentre, observational study to collect specific growth measurements in children with achondroplasia from birth to less than 9 years of age. Study 2 (TCC-201) Double-blind, randomised, placebo-controlled, dose-escalation trial to evaluate pharmacokinetics, safety and efficacy of navepegritide in children with achondroplasia from 2 years to less than 11 years of age. Study 3 (ASND0036) Double-blind, randomised, placebo-controlled, multicentre trial to evaluate safety and efficacy of navepegritide in children with achondroplasia from 2 to less than 12 years of age. Study 4 (ASND0039) Multicentre, open-label, long-term extension trial to evaluate tolerability, safety and efficacy of navepegritide in children with achondroplasia from 2 years to less than 18 years of age. Study 5 (ASND0030) Double-blind, randomised, placebo-controlled, multicentre, trial to evaluate safety, tolerability and efficacy of navepegritide in children with achondroplasia from birth to less than 2 years of age. Study 6 (ASND0045) Double-blind, randomised, placebo-controlled, multicentre trial to evaluate

		safety, tolerability and efficacy of navepegritide in children with achondroplasia and open growth plates from 12 years to less than 18 years of age. Study 7 (ASND0041) Open-label, randomised, 3-treatment, 6-sequence, 3-period crossover trial in healthy adult participants to compare the relative bioavailability of the navepegritide formulation used in Phase 2 trials versus the to-be-marketed drug product (DP) presentations.
Extrapolation, Modeling & Simulation Studies	3	Study 8 Population pharmacokinetic study to confirm or modify the paediatric posology compared to the regimen used in clinical trials in children from 2 years to less than 18 years of age. Study 9 Population pharmacokinetic study to confirm or modify the paediatric posology compared to the regimen used in clinical trials in children from birth to less than 2 years of age. Study 10 Population pharmacokinetic study to confirm or modify the paediatric posology compared to the regimen used in clinical trials in children from 12 years to less than 18 years of age.
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:	Yes
Date of completion of the paediatric investigation plan:	31/08/2034
Deferral of one or more studies contained in the paediatric investigation plan:	Yes