EMA/369130/2020

European Medicines Agency decision

P/0275/2020

of 15 July 2020

on the agreement of a paediatric investigation plan and on the granting of a waiver for lonapegsomatropin (EMEA-002692-PIP01-19) 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 Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency[2],

Having regard to the application submitted by Ascendis Pharma Endocrinology Division A/S on 24 October 2019 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 26 June 2020, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 13 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 waiver.

(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 waiver.

[1] OJ L 378, 27.12.2006, p.1.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

A paediatric investigation plan for lonapegsomatropin, 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 waiver for lonapegsomatropin, 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 is addressed to Ascendis Pharma Endocrinology Division A/S, Tuborg Boulevard 12, 2900 – Hellerup, Denmark.

EMA/PDCO/198226/2020 Corr
Amsterdam, 26 June 2020

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

EMEA-002692-PIP01-19

Scope of the application

Active substance(s):

Lonapegsomatropin

Condition(s):

Treatment of growth hormone deficiency

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 Endocrinology Division A/S

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Ascendis Pharma Endocrinology Division A/S submitted for agreement to the European Medicines Agency on 24 October 2019 an application for a paediatric investigation plan for the above mentioned medicinal product and a waiver under Article 13 of said Regulation.

The procedure started on 3 December 2019.

Supplementary information was provided by the applicant on 24 March 2020.

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 waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

The Norwegian Paediatric Committee member agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed paediatric investigation plan and the subset(s) of the paediatric population and condition(s) covered by the waiver 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 annex 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

1.1. Condition:

Treatment of growth hormone deficiency

The waiver applies to:

- the paediatric population from birth to less than 6 months of age;
- powder and solvent for solution for injection, subcutaneous use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

2. Paediatric investigation plan

2.1. Condition:

Treatment of growth hormone deficiency

2.1.1. Indication(s) targeted by the PIP

Treatment of growth hormone deficiency

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From 6 months 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 Randomized, open-label, active-controlled, parallel-group trial investigating the safety, tolerability, and efficacy of lonapegsomatropin compared to standard daily growth hormone, in children from 3 years to less than 13 years for boys and to less than 12 years for girls with growth failure due to growth hormone deficiency (GHD), over 52 weeks (CT-301).

	Study 2 Open-label, non-controlled, single arm trial to investigate the safety, tolerability and efficacy of lonapegsomatropin administered once weekly in children with growth hormone deficiency (GHD) in children with GHD from 6 months to less than 18 years of age, over 26 weeks (CT-302). Study 3 Long-term, open-label extension study to investigate the long term safety and efficacy of lonapegsomatropin administered once-weekly in children from 1 to less than 18 years of age with growth hormone deficiency (GHD) who have completed a prior confirmatory lonapegsomatropin clinical trial (CT-301EXT).
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:	Yes
Date of completion of the paediatric investigation plan:	By September 2019
Deferral for one or more measures contained in the paediatric investigation plan:	No