

EMA/414997/2014

European Medicines Agency decision

P/0205/2014

of 8 August 2014

on the agreement of a paediatric investigation plan and on the granting of a deferral for recombinant parathyroid hormone (EMEA-001526-PIP01-13) 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 726/2004¹,

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 Agency²,

Having regard to the application submitted by NPS Pharma Holdings Limited on 2 September 2013 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 20 June 2014, in accordance with Article 18 of Regulation (EC) No 1901/2006, and Article 21 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 deferral.
- (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 deferral.

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 recombinant parathyroid hormone, 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 deferral for recombinant parathyroid hormone, 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 NPS Pharma Holdings Limited, Grand Canal House, 1 Grand Canal Street Upper, Dublin 4, Ireland.

Done at London, 8 August 2014

For the European Medicines Agency
Guido Rasi
Executive Director
(Signature on file)

EMA/PDCO/212982/2014

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

EMA-001526-PIP01-13

Scope of the application

Active substance(s):

Recombinant parathyroid hormone

Condition(s):

Treatment of hypoparathyroidism

Pharmaceutical form(s):

Powder and solvent for solution for injection

Route(s) of administration:

Subcutaneous use

Name / corporate name of the PIP applicant:

NPS Pharma Holdings Limited

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, NPS Pharma Holdings Limited submitted for agreement to the European Medicines Agency on 2 September 2013 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 21 January 2014.

Supplementary information was provided by the applicant on 31 March 2014. The applicant proposed modifications to the paediatric investigation plan and withdrew its request for a waiver.

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 18 of said Regulation;
- to grant a deferral in accordance with Article 21 of said Regulation.

The Icelandic and the Norwegian Paediatric Committee members agree with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed 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 annex and appendix.

London, 20 June 2014

On behalf of the Paediatric Committee
Dr Dirk Mentzer, Chairman
(Signature on file)

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

Long-term treatment of subjects with 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)

Powder and solvent for solution for injection

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	1	Study 1 Development of lower strength of existing dose form and age-appropriate delivery device for use in children from birth to less than 12 years of age.
Non-clinical studies	1	Study 2 Repeated toxicity study in juvenile rats, including bone densitometry, histomorphometry and histopathology, plus femur and tibia length
Clinical studies	4	Study 3 Open-label, non-controlled PK/PD trial to evaluate the mean baseline corrected change in serum total calcium concentrations and plasma PTH(1-84) concentrations after single dose subcutaneous injection of 50 mcg recombinant parathyroid hormone in children from 12 to less than 18 years of age with hypoparathyroidism. Study 4 Open-label, non-controlled PK/PD trial to evaluate the mean baseline corrected change in serum total calcium concentrations and plasma PTH (1-84) concentrations after single dose subcutaneous injection of recombinant parathyroid hormone in children from birth to less than 12 years of age with hypoparathyroidism.

Area	Number of measures	Description
		Study 5 Open-label, non-controlled trial to assess safety and activity of recombinant parathyroid hormone, administered with multiple-dose pen injection device as add-on to best standard of care in children from 12 to less than 18 years of age with hypoparathyroidism. Study 6 Open-label, non-controlled trial to assess safety and activity of recombinant parathyroid hormone, administered with age-appropriate delivery device as add-on to best standard of care in children from birth to less than 12 years of age with hypoparathyroidism.
Extrapolation, modelling and simulation studies	1	Study 7 Development of a population PK model to perform simulations and to support the choice of doses for the PK/PD study in children from birth to less than 18 years of age.
Other studies	0	Not applicable.
Other measures	0	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 June 2020
Deferral for one or more measures contained in the paediatric investigation plan:	Yes