EMA/629295/2022

European Medicines Agency decision

P/0285/2022

of 11 August 2022

on the agreement of a paediatric investigation plan and on the granting of a deferral for oxytocin (EMEA-003148-PIP01-21) 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 Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the application submitted by OT4B on 19 November 2021 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 24 June 2022, in accordance with Article 17 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, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

A paediatric investigation plan for oxytocin, nasal spray, solution, nasal 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 oxytocin, nasal spray, solution, nasal 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 OT4B, 81 Boulevard Lazare Carnot Résidence Cap Wilson - Boîte A6, 31000 – Toulouse, France.

EMA/PDCO/190708/2022 Corr
Amsterdam, 24 June 2022

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

EMEA-003148-PIP01-21

Scope of the application

Active substance(s):

Oxytocin

Condition(s):

Treatment of Prader-Willi syndrome

Pharmaceutical form(s):

Nasal spray, solution

Route(s) of administration:

Nasal use

Name/corporate name of the PIP applicant:

OT4B

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, OT4B submitted for agreement to the European Medicines Agency on 19 November 2021 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation.

The procedure started on 4 January 2022.

Supplementary information was provided by the applicant on 21 March 2022. The applicant proposed modifications to the paediatric investigation plan.

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 deferral in accordance with Article 21 of said Regulation.

   The Paediatric Committee member Norway agrees 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.

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 Prader-Willi syndrome

2.1.1. Indication(s) targeted by the PIP

Treatment of Prader-Willi syndrome in neonates and infants from birth to less than 2 years of age.

Treatment of dysphagia in children and adolescents from 2 years to less than 18 years of age with Prader-Willi Syndrome.

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)

Nasal spray, solution

2.1.4. Measures

Area	**Description**
Quality-related studies	Not applicable
Non-clinical studies	**Study 1** (OT4B-21-DRF-01) Dose range finding juvenile toxicity study of oxytocin by the intranasal route in the rat from PND7 to PND28 to support evaluation of safety of oxytocin when used in neonates. **Study 2** (OT4B-21-DRF-02) Dose range finding juvenile toxicity study of oxytocin by the intranasal route in the rat from PND7 to PND28 to support evaluation of safety of oxytocin when used in neonates. **Study 3** (OT4B-21-JAS-01) Definitive juvenile toxicity study of oxytocin by the intranasal route in the rat from PND7 up to PND70 (up to 10 weeks of age for dosing followed by a 3-week treatment-free period) to support evaluation of safety of oxytocin when used in neonates and children from birth to less than 18 years of age.

Clinical studies	**Study 4** (OTBB1) Open-label, single dose, uncontrolled, non-comparative trial to evaluate safety and activity of oxytocin in neonates and infants with Prader-Willi syndrome from 15 days to less than 5 months of age. **Study 5** (OTBB2) Open-label, dose escalation, uncontrolled, non-comparative trial to evaluate safety, tolerability and activity of oxytocin in neonates and infants with Prader-Willi syndrome from birth to less than 5 months of age. **Study 6** (OT2suite) Open-label, comparative trial to evaluate long term outcomes in children with Prader-Willi Syndrome from 3 years to less than 5 years of age who were or were not treated with oxytocin during their first 6 months of life. **Study 7** (OTBB3) Double-blind, placebo-controlled, randomised study to evaluate safety and efficacy of oxytocin in neonates and infants with Prader-Willi syndrome from birth to less than 5 months of age. **Study 8** (OTBB3 Follow-up) Open label, long-term safety follow-up study in children with Prader-Willi syndrome up to 4 years of age who were treated with oxytocin in OTBB3 and comparison with children who were not treated. **Study 9** (Dysmot) Double-blind, placebo-controlled, randomised study to evaluate safety and efficacy of oxytocin in children from 2 years to less than 18 years of age with Prader-Willi syndrome.
Extrapolation, modelling and simulation studies	**Study 10** Modelling and simulation study to evaluate the use of intranasal oxytocin in the treatment of Prader Willi Syndrome in children from 2 to less than 18 years of age.
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:	June 2027
Deferral for one or more measures contained in the paediatric investigation plan:	Yes