EMA/12127/2020

European Medicines Agency decision

P/0030/2020

of 29 January 2020

on the acceptance of a modification of an agreed paediatric investigation plan for (RS)-baclofen / Naltrexone HCl / D-Sorbitol (PXT3003) (EMEA-002164-PIP01-17-M02) 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 European Medicines Agency's decision P/0097/2018 issued on 16 March 2018 and the decision P/0363/2018 issued on 7 December 2018,

Having regard to the application submitted by Pharnext S.A. on 9 September 2019 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 11 December 2019, in accordance with Article 22 of Regulation (EC) No 1901/2006,

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 acceptance of changes to the agreed paediatric investigation plan.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

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

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for (RS)-baclofen / Naltrexone HCl / D-Sorbitol (PXT3003), oral solution, oral use, are hereby accepted in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

Article 2

This decision is addressed to Pharnext S.A., Immeuble Vivaldi, 11 -13 rue René Jacques, 92130 - Issy-les-Moulineaux, France.

EMA/PDCO/558235/2019
Amsterdam, 11 December 2019

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMEA-002164-PIP01-17-M02

Scope of the application

Active substance(s):

(RS)-baclofen / Naltrexone HCl / D-Sorbitol (PXT3003)

Condition(s):

Treatment of Charcot-Marie-Tooth disease Type 1A

Pharmaceutical form(s):

Oral solution

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Pharnext S.A.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Pharnext S.A. submitted to the European Medicines Agency on 9 September 2019 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/0097/2018 issued on 16 March 2018 and the decision P/0363/2018 issued on 7 December 2018.

The application for modification proposed changes to the agreed paediatric investigation plan.

The procedure started on 15 October 2019.

Scope of the modification

Some measures and timelines of the Paediatric Investigation Plan have been modified.

Opinion

1. The Paediatric Committee, having assessed the application in accordance with Article 22 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree to changes to the paediatric investigation plan in the scope set out in the Annex I of this opinion.

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

2. The measures and timelines of the 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 Charcot-Marie-Tooth disease Type 1A

The waiver applies to:

- the paediatric population from birth to less than 3 years of age;
- oral solution, oral 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 Charcot-Marie-Tooth disease Type 1A (CMT1A)

2.1.1. Indication(s) targeted by the PIP

Treatment of Charcot-Marie-Tooth disease Type 1A

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

From 3 to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Oral solution

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable
Non-clinical studies	0	Not applicable
Clinical studies	4	**Study 1** International, multi-centre, randomised, double-blind, placebo-controlled study assessing in parallel groups the efficacy and safety of 2 doses of PXT3003 in patients with Charcot-Marie-Tooth disease type 1A treated for 15 months (CLN-PXT3003-02) **Study 2** Randomised, double-blind, parallel group, follow–up extension study with two PXT3003 dose groups, following Study 1 (CLN-PXT3003-03)

		Study 3 International, multicentre, 24-month, randomised, prospective, double-blind, placebo-controlled study, designed to assess the effect of PXT3003, administered orally twice per day, on disability of paediatric patients with CMT1A (CLN-PXT3003-04) **Study 4** Multicentre, 24-month, open-label extension study to assess the long term safety and tolerability of PXT3003 in CMT1A patients who completed Study 3 (CLN-PXT3003-05)
Extrapolation, modelling and simulation studies	0	Not applicable
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:	No
Date of completion of the paediatric investigation plan:	By December 2023
Deferral for one or more measures contained in the paediatric investigation plan:	Yes