EMA/192340/2021

European Medicines Agency decision

P/0162/2021

of 14 April 2021

on the agreement of a paediatric investigation plan for hydroxypropyl-β-cyclodextrin (EMEA-002839-PIP01-20) 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 Cyclo Therapeutics Inc on 5 June 2020 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 February 2021, in accordance with Article 17 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 agreement of a paediatric investigation plan.

(2) It is therefore appropriate to adopt a decision agreeing a 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

A paediatric investigation plan for hydroxypropyl-β-cyclodextrin, solution for infusion, intravenous 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

This decision is addressed to Cyclo Therapeutics Inc, 6714 NW 16th Street, 32616 - Gainesville, Florida, United States.

EMA/PDCO/1176/2021
Amsterdam, 26 February 2021

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

EMEA-002839-PIP01-20

Scope of the application

Active substance(s):

Hydroxypropyl-β-cyclodextrin

Condition(s):

Treatment of Niemann Pick disease type C

Pharmaceutical form(s):

Solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Cyclo Therapeutics Inc

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Cyclo Therapeutics Inc submitted for agreement to the European Medicines Agency on 5 June 2020 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 6 July 2020.

Supplementary information was provided by the applicant on 27 October 2020. 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 17(1) of said Regulation.

   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 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 Niemann Pick disease type C

2.1.1. Indication(s) targeted by the PIP

Systemic and neurological treatment of Niemann-Pick disease Type C1

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)

Solution for infusion

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable
Non-clinical studies	0	Not applicable
Clinical studies	2	**Study 1** Open-label, uncontrolled, multiple dose study to evaluate the safety and pharmacokinetics of intravenous hydroxypropyl-β-cyclodextrin in patients from 2 years to less than 18 years of age (and adults) with Niemann-Pick disease type C and the pharmacodynamic effects of treatment upon markers of cholesterol metabolism and clinical outcomes (CTD-TCNPC-201). **Study 2** Double blind, randomized, placebo controlled, parallel group study to evaluate the safety, tolerability, and efficacy of hydroxypropyl β cyclodextrin to placebo in paediatric patients from 3 years to less than 18 years of age (and adults) with Niemann Pick Disease Type C1, who are receiving current standard of care treatment

		(CTD-TCNPC-301). This study includes a single-arm substudy with patients from birth to less than 3 years of age with Niemann Pick Disease Type C1 to evaluate safety and to obtain descriptive data on global severity and improvement in response to hydroxypropyl β cyclodextrin as add-on to current standard of care treatment (CTD-TCNPC-301).
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:	Yes
Date of completion of the paediatric investigation plan:	By January 2023
Deferral for one or more measures contained in the paediatric investigation plan:	No