EMA/66794/2023

European Medicines Agency decision

P/0095/2023

of 10 March 2023

on the acceptance of a modification of an agreed paediatric investigation plan for hydroxypropyl-β-cyclodextrin (EMEA-002839-PIP01-20-M01) 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 European Medicines Agency's decision P/0162/2021 issued on 14 April 2021,

Having regard to the application submitted by Cyclo Therapeutics Inc on 14 November 2022 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 20 January 2023, 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, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for hydroxypropyl-β-cyclodextrin, solution for infusion, intravenous 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 Cyclo Therapeutics Inc, 6714 NW 16th Street, 32616 - Gainesville, Florida, United States.

EMA/PDCO/906789/2022
Amsterdam, 20 January 2023

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

EMEA-002839-PIP01-20-M01

Scope of the application

Active substance(s):

Hydroxypropyl-β-cyclodextrin

Invented name and authorisation status:

See Annex II

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 22 of Regulation (EC) No 1901/2006 as amended, Cyclo Therapeutics Inc submitted to the European Medicines Agency on 14 November 2022 an application for modification of the agreed paediatric investigation plan as set out in the European Medicines Agency's decision P/0162/2021 issued on 14 April 2021.

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

The procedure started on 3 January 2023.

Scope of the modification

Some 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 Paediatric Committee member of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the 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 annexes 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	**Description**
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	**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	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 2025
Deferral for one or more measures contained in the paediatric investigation plan:	No

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

The product is not authorised anywhere in the European Community.