EMA/534531/2021

European Medicines Agency decision

P/0447/2021

of 29 October 2021

on the acceptance of a modification of an agreed paediatric investigation plan for oxalobacter formigenes strain HC-1 (EMEA-000370-PIP02-18-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 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/0273/2019 issued on 14 August 2019,

Having regard to the application submitted by OxThera AB on 7 May 2021 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 10 September 2021, 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 and to the deferral.

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

Changes to the agreed paediatric investigation plan for oxalobacter formigenes strain HC-1, gastro-resistant capsule, hard, powder for oral suspension, gastro-resistant tablet, gastro-resistant granules for oral suspension, oral use, gastric use, including changes to the deferral, 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 OxThera AB, Regeringsgatan 111, 11139 – Stockholm, Sweden.

EMA/PDCO/325423/2021
Amsterdam, 10 September 2021

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

EMEA-000370-PIP02-18-M01

Scope of the application

Active substance(s):

Oxalobacter formigenes Strain HC-1

Condition(s):

Treatment of hyperoxaluria

Pharmaceutical form(s):

Gastro-resistant capsule, hard

Powder for oral suspension

Gastro-resistant tablet

Gastro-resistant granules for oral suspension

Route(s) of administration:

Oral use

Gastric use

Name/corporate name of the PIP applicant:

OxThera AB

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, OxThera AB submitted to the European Medicines Agency on 7 May 2021 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0273/2019 issued on 14 August 2019.

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

The procedure started on 12 July 2021.

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 and to the deferral 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 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 hyperoxaluria

2.1.1. Indication(s) targeted by the PIP

Treatment of primary hyperoxaluria

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)

Gastro-resistant capsule, hard

Powder for oral suspension

Gastro-resistant tablet

Gastro-resistant granules for oral suspension

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	1	**Study 1** Development of powder for oral suspension.
Non-clinical studies	0	Not applicable
Clinical studies	3	**Study 2** Double-blind, randomised, placebo-controlled trial to evaluate efficacy and safety of O. formigenes (OC5) with long-term treatment in children from 2 to less than 18 years of age (and adults) with primary hyperoxaluria (PH). (OC5-DB-02) **Study 3** Open-label trial to evaluate long-term efficacy and safety of O. formigenes (OC5) in patients with primary hyperoxaluria (PH) who have completed Study OC5-DB-02. (OC5-OL-02)

		Study 4 Open-label, non-comparative trial to evaluate efficacy and safety of O. formigenes (OC5) in children from birth to less than 12 years of age with primary hyperoxaluria (PH) with chronic kidney disease (CKD) with maintained renal function or being on dialysis. (OC5-OL-03)
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 March 2027
Deferral for one or more measures contained in the paediatric investigation plan:	Yes