EMA/395099/2019

European Medicines Agency decision

P/0273/2019

of 14 August 2019

on the agreement of a paediatric investigation plan and on the granting of a deferral for oxalobacter formigenes Strain HC-1 (EMEA-000370-PIP02-18) 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 OxThera AB on 26 June 2018 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 28 June 2019, 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.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

A 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, 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 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, 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 OxThera AB, Regeringsgatan 111, 11139 – Stockholm, Sweden.

EMA/PDCO/210281/2019
Amsterdam, 28 June 2019

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

EMEA-000370-PIP02-18

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 16(1) of Regulation (EC) No 1901/2006 as amended, OxThera AB submitted for agreement to the European Medicines Agency on 26 June 2018 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 21 August 2018.

Supplementary information was provided by the applicant on 15 March 2019. 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 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 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 December 2024
Deferral for one or more measures contained in the paediatric investigation plan:	Yes