EMA/193794/2022

European Medicines Agency decision

P/0132/2022

of 13 April 2022

on the acceptance of a modification of an agreed paediatric investigation plan for fosdenopterin (ORGN001), (EMEA-001491-PIP01-13-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 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/0071/2014 issued on 21 March 2014 and the decision P/0048/2021 issued on 27 January 2021,

Having regard to the application submitted by Comharsa Life Sciences Limited on 19 November 2021 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 25 February 2022, 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 fosdenopterin (ORGN001), powder for solution for injection, 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 Comharsa Life Sciences Limited, 10 Earlsfort Terrace, D02 T380 - Dublin 2, Ireland.

EMA/PDCO/714560/2021
Amsterdam, 25 February 2022

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

EMEA-001491-PIP01-13-M02

Scope of the application

Active substance(s):

Fosdenopterin (ORGN001)

Condition(s):

Treatment of molybdenum cofactor deficiency type A

Pharmaceutical form(s):

Powder for solution for injection

Route(s) of administrationt

Intravenous use

Name/corporate name of the PIP applicant:

Comharsa Life Sciences Limited

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Comharsa Life Sciences Limited submitted to the European Medicines Agency on 19 November 2021 an application for modification of the agreed paediatric investigation plan as set out in the European Medicines Agency's decision P/0071/2014 issued on 21 March 2014 and the decision P/0048/2021 issued on 27 January 2021.

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

The procedure started on 4 January 2022.

Scope of the modification

Pharmaceutical form was amended.

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 investigational 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 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 molybdenum cofactor deficiency type A

2.1.1. Indication(s) targeted by the PIP

Treatment of molybdenum cofactor deficiency type A

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)

Powder for solution for injection

2.1.4. Measures

Area	**Description**
Quality-related studies	Not applicable.
Non-clinical studies	**Study 1** Juvenile toxicity study in rats, for 26-week treatment period and 28-day recovery period. **Study 2** Juvenile toxicity study in dogs, for 9-month treatment period and 3-month recovery period.
Clinical studies	**Study 3 (ALXN1101-MCD-201)** Open-label, multicentre, dose-escalation study to evaluate the safety, activity and pharmacokinetics of fosdenopterin in children with a genetically confirmed diagnosis of Molybdenum cofactor deficiency type A, treated with recombinant Escherichia Coli-derived cyclic pyranopterin monophosphate. **Study 4 (ALXN1101-MCD-202)** Open-label, multicentre study to evaluate the safety and activity of fosdenopterin in children with a genetic diagnosis of Molybdenum cofactor deficiency type A, or who present with clinical signs and symptoms consistent with Molybdenum cofactor deficiency type A.

	Study 5 (ALXN1101-MCD-501) Retrospective, observational, noninterventional data collection study to assess safety and efficacy of prior administration of intravenous (IV) recombinant cPMP (rcPMP; predecessor of fosdenopterin) in patients with a genetically confirmed diagnosis of molybdenum cofactor deficiency (MoCD) type A or who were suspected to have a diagnosis of MoCD type A based on signs and symptoms at the time of rcPMP treatment initiation. **Study 6 (ALX-MCD-502)** Retrospective and prospective, multinational, multicenter natural history study of molybdenum cofactor and isolated sulfite oxidase deficiencies to characterize the natural history of molybdenum cofactor deficiency (MoCD) type A.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By August 2021
Deferral for one or more measures contained in the paediatric investigation plan:	No