EMA/255060/2024

European Medicines Agency decision

P/0214/2024

of 13 June 2024

on the agreement of a paediatric investigation plan and on the refusal of a deferral for recombinant adeno-associated virus Olig001 containing human aspartoacylase cDNA (MYR-101), (EMEA-003459-PIP01-23) 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 application submitted by Myrtelle,Inc. on 30 May 2023 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 26 April 2024, 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 refusal 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 refusing a deferral.

[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

A paediatric investigation plan for recombinant adeno-associated virus Olig001 containing human aspartoacylase cDNA (MYR-101), solution for injection, intracerebral 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 recombinant adeno-associated virus Olig001 containing human aspartoacylase cDNA (MYR-101), solution for injection, intracerebral 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 refused.

Article 3

This decision is addressed to Myrtelle,Inc., 301 Edgewater Place, Suite 330, 01880 - Wakefield Massachusetts, USA.

EMA/PDCO/55020/2024 Corr[1]
Amsterdam, 26 April 2024

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

EMEA-003459-PIP01-23

Scope of the application

Active substance(s):

Recombinant adeno-associated virus Olig001 containing human aspartoacylase cDNA (MYR-101)

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of Canavan disease

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Intracerebral use

Name/corporate name of the PIP applicant:

Myrtelle,Inc.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Myrtelle,Inc. submitted for agreement to the European Medicines Agency on 30 May 2023 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 10 July 2023.

Supplementary information was provided by the applicant on 22 January 2024.

[1] 17 May 2024

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 refuse a deferral in accordance with Article 21 of said Regulation.

   The Paediatric Committee member of Norway 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 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

2.1.1. Condition:

Treatment of Canavan disease

2.1.2. Indication(s) targeted by the PIP

Treatment of Canavan disease

2.1.3. Subset(s) of the paediatric population concerned by the paediatric development

From birth to less than 18 years of age

2.1.4. Pharmaceutical form(s)

Solution for injection

2.1.5. Measures

Area	**Description**
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	***Study 1*** Natural history (NH) retrospective study based on a chart review of medical records, from patients with Canavan disease aged 1 month to 5 years. ***Study 2 (CD Olig001-101)*** Open-label study to evaluate safety, tolerability and activity of intracranial administration of the recombinant adeno-associated virus Olig001 containing human aspartoacylase cDNA (MYR-101) in children aged from 3 months to 60 months with the neonatal/infantile onset form of Canavan disease. ***Study 3*** Single-arm, open label and externally controlled study to confirm safety and efficacy of MYR-101 in patients from birth to less than 18 years of age with juvenile and neonatal/infantile onset Canavan disease.
Modelling and simulation analyses	Not applicable

Other studies	Not applicable
Extrapolation plan	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 April 2031
Deferral for one or more measures contained in the paediatric investigation plan:	No

3.1.1. Refusal of deferral

The PDCO denied a deferral on the grounds that:

- the product is being developed for a condition predominantly or exclusively affecting the paediatric population.

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

The product is not authorised anywhere in the European Community.