EMA/480700/2024

European Medicines Agency decision

P/0369/2024

of 25 October 2024

on the agreement of a paediatric investigation plan and on the granting of a deferral for idroxioleic acid (EMEA-003565-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 Laminar Pharmaceuticals SA on 18 December 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 6 September 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 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, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

A paediatric investigation plan for idroxioleic acid, powder 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 idroxioleic acid, powder 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 Laminar Pharmaceuticals SA, Ctra. de Valldemossa Km. 7,4; ParcBIT. Edificio Naorte, 07121 – Palma, Spain.

EMA/PDCO/277464/2024
Amsterdam, 6 September 2024

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

EMEA-003565-PIP01-23

Scope of the application

Active substance(s):

Idroxioleic acid

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of glioma

Pharmaceutical form(s):

Powder for oral suspension

Route(s) of administration:

Oral use

Gastric use

Name/corporate name of the PIP applicant:

Laminar Pharmaceuticals SA

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Laminar Pharmaceuticals SA submitted for agreement to the European Medicines Agency on 18 December 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 22 January 2024.

Supplementary information was provided by the applicant on 3 June 2024. 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 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. Condition:

Treatment of glioma

2.1.1. Indication(s) targeted by the PIP

Treatment of newly diagnosed patients with high grade glioma

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 oral suspension

2.1.4. Measures

Area	Description
Quality-related studies	**Study 1** (MIN-001P-1501-AP/ MIN-001P-1501-AF/ MIN-001P-1501-DU) Development of an age-appropriate formulation
Non-clinical studies	**Study 2** (no identifier available) In vivo proof of concept study to confirm effect of idroxioleic acid in paediatric high-grade glioma cells-orthotopic derived tumours implanted in immunosuppressed mice.
Clinical studies	**Study 3** (MIN-001P-1501) Open-label, single arm trial to evaluate recommended phase 2 dose of idroxioleic acid, followed by an expansion cohort to evaluate safety and activity in children from birth to less than 18 years of age with malignant glioma and other advanced solid tumours. **Study 4** (LAM-002P-XXXX) Double-blind, randomised, placebo controlled, trial to evaluate pharmacokinetics, safety, efficacy, acceptability/palatability of idroxioleic acid as add-on to radiotherapy and surgery in children from birth to less than 18 years of age with newly diagnosed malignant brain tumours, including paediatric-type diffuse high grade glioma (pdHGG).

Modelling and simulation analyses	**Study 5** (LAM-002P-XXXX-Mod) Modelling and simulation analyses, to evaluate the use of the product in children from birth to less than 18 years of age with malignant glioma.
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:	No
Date of completion of the paediatric investigation plan:	By December 2029
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

The product is not authorised anywhere in the European Community.