EMA/84334/2023

European Medicines Agency decision

P/0070/2023

of 10 March 2023

on the acceptance of a modification of an agreed paediatric investigation plan for lumasiran (Oxlumo), (EMEA-002079-PIP01-16-M03) 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/0373/2017 issued on 1 December 2017, the decision P/0004/2020 issued on 6 January 2020 and the decision P/0505/2021 issued on 3 December 2021.

Having regard to the application submitted by Alnylam UK Limited on 13 October 2022 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 20 January 2023, 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 lumasiran (Oxlumo), solution for injection, subcutaneous 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 Alnylam UK Limited, Braywick Gate, Braywick Road, SL6 1DA – Maidenhead, United Kingdom.

EMA/PDCO/887591/2022
Amsterdam, 20 January 2023

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

EMEA-002079-PIP01-16-M03

Scope of the application

Active substance(s):

Lumasiran

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of hyperoxaluria

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Subcutaneous use

Name/corporate name of the PIP applicant:

Alnylam UK Limited

Information about the authorised medicinal product:

See Annex II

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Alnylam UK Limited submitted to the European Medicines Agency on 13 October 2022 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0373/2017 issued on 1 December 2017, the decision P/0004/2020 issued on 6 January 2020 and the decision P/0505/2021 issued on 3 December 2021.

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

The procedure started on 17 August 2021.

Scope of the modification

Some measures 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 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 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 hyperoxaluria

2.1.1. Indication(s) targeted by the PIP

Treatment of primary hyperoxaluria type 1

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)

Solution for injection

2.1.4. Measures

Area	Description
Quality-related studies	**Study 1** Study deleted in EMEA-002079-PIP01-16-M03
Non-clinical studies	**Study 2** 4-week dose range-finding toxicity study in neonate and juvenile rats (8335749/GO1-GLP15-043)
Clinical studies	**Study 3** Single (subject/patient) blind, randomised, placebo controlled trial to evaluate safety, tolerability pharmacokinetics and pharmacodynamics of ALN-GO1 in healthy adult subjects, and patients (children from 6 years of age (and adults) with primary hyperoxaluria type 1 (PH1)) (ALN-GO1-001 (2015-004407-23)) **Study 4** Open-label extension study for patients who previously participated in Study 3 (ALN-GO1-001) **Study 5** Double-blind, randomised, placebo controlled study with an extended dosing period to evaluate efficacy, safety, pharmacokinetics and pharmacodynamics of ALN-GO1 in children from 6 years of age (and adults) with primary hyperoxaluria type

	1 (PH1) and relatively intact renal function (ILLUMINATE-A; ALN-GO1-003) **Study 6** Open-label, uncontrolled trial to evaluate safety, pharmacokinetics and pharmacodynamics of ALN-GO1 in children from birth to less than 6 years of age with primary hyperoxaluria type 1 (PH1) and relatively intact renal function (ILLUMINATE B; ALN-GO1-004) **Study 7** Open-label, uncontrolled trial to evaluate safety, pharmacokinetics and pharmacodynamics of ALN-GO1 in children from birth to less than 18 years of age (and adults) with primary hyperoxaluria type 1 (PH1) and advanced renal disease including those on dialysis (ILLUMINATE C; ALN-GO1-005)
Extrapolation, modelling and simulation studies	**Study 8** Modelling and simulation study to evaluate the use of the product and support dose selection for the treatment of primary hyperoxaluria type 1 (PH1) in children from 6 years of age with relatively intact renal function (ALN-GO1-MS1) **Study 9** Modelling and simulation study to evaluate the use of the product and support dose selection for the treatment of primary hyperoxaluria type 1 (PH1) in children from birth to less than 6 years of age (ALN-GO1-MS2)
Other studies	Not applicable
Other measures	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 October 2025
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:

Condition(s) and authorised indication(s)

1. Treatment of hyperoxaluria

Authorised indication(s):

- Oxlumo is indicated for the treatment of primary hyperoxaluria type 1 (PH1) in all age groups.
  - Invented name(s): Oxlumo
  - Authorised pharmaceutical form(s): Solution for injection
  - Authorised route(s) of administration: Subcutaneous injection
  - Authorised via centralised procedure