EMA/658963/2019

European Medicines Agency decision

P/0004/2020

of 6 January 2020

on the acceptance of a modification of an agreed paediatric investigation plan for lumasiran (ALN-GO1) (EMEA-002079-PIP01-16-M01) 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 European Medicines Agency's decision P/0373/2017 issued on 1 December 2017,

Having regard to the application submitted by Alnylam UK Limited on 9 August 2019 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 15 November 2019, 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 and to the deferral.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan, including changes to the 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

Changes to the agreed paediatric investigation plan for lumasiran (ALN-GO1), solution for injection, subcutaneous use, including changes to the deferral, 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/479983/2019 Corr
Amsterdam, 15 November 2019

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

EMEA-002079-PIP01-16-M01

Scope of the application

Active substance(s):

Lumasiran (ALN-GO1)

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

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 9 August 2019 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 application for modification proposed changes to the agreed paediatric investigation plan and to the deferral.

The procedure started on 17 September 2019.

Scope of the modification

Some measures and timelines 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 and to the deferral 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 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	Number of measures	Description
Quality-related studies	1	**Study 1** Development of a solution with a concentration of less than 200 mg/mL
Non-clinical studies	1	**Study 2** 4-week dose range-finding toxicity study in neonate and juvenile rats (8335749/GO1-GLP15-043)
Clinical studies	5	**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).

Area	Number of measures	Description
		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	2	**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	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 October 2025
Deferral for one or more measures contained in the paediatric investigation plan:	Yes