EMA/420429/2020

European Medicines Agency decision

P/0327/2020

of 14 August 2020

on the acceptance of a modification of an agreed paediatric investigation plan for lenadogene nolparvovec (GS010) (EMEA-001992-PIP02-16-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/0166/2020 issued on 24 April 2020,

Having regard to the application submitted by GenSight-Biologics on 17 April 2020 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 24 July 2020, 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 lenadogene nolparvovec (GS010), suspension for injection, intravitreal 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 GenSight-Biologics, 74 RUE DU FAUBOURG SAINT ANTOINE, 75012 - PARIS, France.

EMA/PDCO/250480/2020
Amsterdam, 24 July 2020

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

EMEA-001992-PIP02-16-M01

Scope of the application

Active substance(s):

Lenadogene nolparvovec (GS010)

Condition(s):

Treatment of Leber hereditary optic neuropathy

Pharmaceutical form:

Suspension for injection

Route(s) of administration:

Intravitreal use

Name/corporate name of the PIP applicant:

GenSight-Biologics

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, GenSight-Biologics submitted to the European Medicines Agency on 17 April 2020 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/0166/2020 issued on 24 April 2020.

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

The procedure started on 26 May 2020.

Scope of the modification

Some 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 and the subset(s) of the paediatric population and condition(s) covered by the waiver 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)

Waiver

1.1. Condition:

Treatment of Leber Hereditary Optic Neuropathy

The waiver applies to:

- the paediatric population from birth to less than 6 years of age;
- suspension for injection, intravitreal use;
- on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2. Paediatric investigation plan

2.1. Condition:

Treatment of Leber Hereditary Optic Neuropathy

2.1.1. Indication(s) targeted by the PIP

Treatment of Leber Hereditary Optic Neuropathy due to ND4 mutation

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

From 6 to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Suspension for injection

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable
Non-clinical studies	2	**Study 1 (GS010_NC_MECA_001)** Biodistribution study of GS010 in cynomolgus monkeys **Study 2 (GS010_NC_MECA_002)** Study of the transfer of mitochondria in mice visual system
Clinical studies	4	**Study 3 (RESCUE GS-LHON-CLIN03A)** Double-blind, randomised, sham-controlled trial to evaluate the efficacy of GS010 in adolescents from 15 to less than 18 years of age (and adults) affected for 6 months or less by Leber Hereditary Optic Neuropathy

		Study 4 (REVERSE GS-LHON-CLIN03B) Double-blind, randomised, sham-controlled trial to evaluate the efficacy of GS010 in adolescents from 15 to less than 18 years of age (and adults) affected for more than 6 months and up to 1 year by Leber Hereditary Optic Neuropathy **Study 5 (REFLECT GS-LHON-CLIN-05)** Double-blind, randomised, placebo-controlled trial to evaluate the efficacy of GS010 in adolescents from 15 to less than 18 years of age (and adults) affected for up to one year by Leber Hereditary Optic Neuropathy **Study 6 (GS-LHON-CLIN-08)** Open-label, single arm, single dose trial to evaluate safety and efficacy of unilateral intravitreal injection of GS010 in children from 6 to less than 15 years of age with G11778A ND4 Leber Hereditary Optic Neuropathy
Extrapolation, modelling and simulation studies	0	Not applicable
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 December 2025
Deferral for one or more measures contained in the paediatric investigation plan:	Yes