EMA/388710/2020

European Medicines Agency decision

P/0283/2020

of 12 August 2020

on the acceptance of a modification of an agreed paediatric investigation plan for olenasufligene relduparvovec (EMEA-002122-PIP02-17-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/0078/2018 issued on 16 March 2018,

Having regard to the application submitted by LYSOGENE on 19 March 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 26 June 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.

(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.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for olenasufligene relduparvovec, suspension for injection, intracerebral 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 LYSOGENE, 18-20 rue Jacques Dulud, 92200 - Neuilly-sur-Seine, France.

EMA/526040/2008
Amsterdam, 26 June 2020

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

EMEA-002122-PIP02-17-M01

Scope of the application

Active substance(s):

Olenasufligene relduparvovec

Condition(s):

Treatment of mucopolysaccharidosis type IIIA

Pharmaceutical form(s):

Suspension for injection

Route(s) of administration:

Intracerebral use

Name/corporate name of the PIP applicant:

LYSOGENE

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, LYSOGENE submitted to the European Medicines Agency on 19 March 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/0078/2018 issued on 16 March 2018.

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

The procedure started on 30 April 2020.

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 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)

1. Waiver

1.1. Condition:

Treatment of mucopolysaccharidosis type IIIA

The waiver applies to:

- adolescents from 12 to less than 18 years of age;
- suspension for injection, intracerebral use;
- on the grounds that the specific medicinal product is likely to be ineffective.

The waiver applies to:

- children from birth to less than 6 months of age;
- suspension for injection, intracerebral use;
- on the grounds that the specific medicinal product is likely to be unsafe.

2. Paediatric investigation plan

2.1. Condition:

Treatment of mucopolysaccharidosis type IIIA

2.1.1. Indication(s) targeted by the PIP

Treatment of children with Sanfilippo Type A Syndrome

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

From 6 months to less than 12 years of age

2.1.3. Pharmaceutical form(s)

Suspension for injection

2.1.4. Measures

Area	**Number of measures**	**Description**
Quality-related studies	1	**Study 1** Device compatibility study – suspension for injection

Non-clinical studies	4	**Study 2** Long-term (up to 6 months) juvenile MPS IIIA mice efficacy study **Study 3** Juvenile dog brain distribution study to evaluate the effect of various infusion volumes and rates on brain distribution (up to 4 weeks). **Study 4** Juvenile toxicity study in Cynomolgus monkeys to characterize the brain distribution of olenasufligene relduparvovec (LYS-SAF302) (up to 4 weeks). **Study 5** Safety study in Cynomolgus monkeys (up to 5 weeks).
Clinical studies	1	**Study 6** Open-label, single dose, single-time administration (via 6 different intraparenchymal injections), historically controlled trial to evaluate efficacy of LYS-SAF302 in children from 6 months to less than 12 years of age with severe MPS IIIA.
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 October 2024
Deferral for one or more measures contained in the paediatric investigation plan:	Yes