EMA/206128/2022

European Medicines Agency decision

P/0145/2022

of 13 May 2022

on the agreement of a paediatric investigation plan and on the granting of a deferral for adeno-associated viral vector serotype rh.10 expressing beta-galactosidase (LYS-GM101) (EMEA-003020-PIP01-21) 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.

European Medicines Agency decision

P/0145/2022

of 13 May 2022

on the agreement of a paediatric investigation plan and on the granting of a deferral for adeno-associated viral vector serotype rh.10 expressing beta-galactosidase (LYS-GM101) (EMEA-003020-PIP01-21) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

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 Lysogene on 19 April 2021 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 25 March 2022, 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 adeno-associated viral vector serotype rh.10 expressing beta-galactosidase (LYS-GM101), suspension for injection, intracisternal 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 adeno-associated viral vector serotype rh.10 expressing beta-galactosidase (LYS-GM101), suspension for injection, intracisternal 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 Lysogene, 18-20 rue Jacques Dulud, 92200 - Neuilly-sur-Seine, France.

EMA/PDCO/20837/2022
Amsterdam, 25 March 2022

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

EMEA-003020-PIP01-21

Scope of the application

Active substance(s):

Adeno-associated viral vector serotype rh.10 expressing beta-galactosidase (LYS-GM101)

Condition(s):

Treatment of GM1 gangliosidosis

Pharmaceutical form(s):

Suspension for injection

Route(s) of administration:

Intracisternal use

Name/corporate name of the PIP applicant:

Lysogene

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Lysogene submitted for agreement to the European Medicines Agency on 19 April 2021 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 25 May 2021.

Supplementary information was provided by the applicant on 20 December 2021. The applicant proposed modifications to the paediatric investigation plan and withdrew its request for a waiver.

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 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 GM1 gangliosidosis

2.1.1. Indication(s) targeted by the PIP

Treatment of GM1 gangliosidosis

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

From birth to less than less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Suspension for injection

2.1.4. Measures

Area	Description
Quality-related studies	Not applicable.
Non-clinical studies	Not applicable.
Clinical studies	Study 1 Open-label, single-arm, single dose, historically controlled trial to evaluate safety and efficacy of adeno-associated viral vector serotype rh.10 expressing beta-galactosidase (LYS-GM101) in children from birth to less than 18 years of age with GM1 gangliosidosis. (P1-GM-101)
Extrapolation, modelling and simulation studies	Not applicable.
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 June 2031
Deferral for one or more measures contained in the paediatric investigation plan:	Yes