MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral

MHRA-100574-PIP01-22

Scope of the Application

Active Substance(s)

lixmabegagene relduparvovec

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 the Decision

Pursuant to the Human Medicines Regulations 2012, LYSOGENE submitted to the licensing authority on 17/06/2022 18:01 BST an application for a Paediatric Investigation Plan

The procedure started on 10/10/2022 12:36 BST

1. The licensing authority, having assessed the application in accordance with the Human Medicines Regulations 2012, decides, as set out in the appended summary report:

to agree a paediatric investigation plan and grant a deferral

2. The measures and timelines of the paediatric investigation plan are set out in the Annex I.

This decision is forwarded to the applicant, together with its annex and appendix.

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Final Decision Letter

MHRA-100574-PIP01-22

Of 08/11/2022 16:39 GMT

On the adopted decision for lixmabegagene relduparvovec (MHRA-100574-PIP01-22) in accordance with the Human Medicines Regulations 2012.

The licensing authority, in accordance with the Human Medicines Regulations 2012, has adopted this decision:

Agreement on a paediatric investigation plan

This decision applies to a Paediatric Investigation Plan for lixmabegagene relduparvovec, Suspension for injection , INTRACISTERNAL USE .

This decision is addressed to LYSOGENE, 18-20 rue Jacques Dulud, Neuilly-sur Seine, FRANCE, 92200

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of GM1 gangliosidosis

2.2 Indication(s) targeted by the PIP:

Treatment of GM1 gangliosidosis

2.3 Subset(s) of the paediatric population concerned by the paediatric development:

All subsets of the paediatric population from birth to less than 18 years of age

2.4 Pharmaceutical Form(s):

Suspension for injection

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable
Non-Clinical Studies	0	Not applicable
Clinical Studies	1	Study 1 (P1-GM-101) Open-label, single arm single dose, historically controlled trial to evaluate safety and efficacy of adeno-viral vector serotype rh.10 expressing beta-galactosidase (LYS-GM101) in children from birth to less than 18 years of age with GM1 gangliosidosis.
Extrapolation, Modeling & Simulation Studies	0	Not applicable
Other Studies	0	Not applicable
Other Measures	0	Not applicable

3. Follow-up, completion and deferral of a PIP:

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	30/06/2031
Deferral of one or more studies contained in the paediatric investigation plan:	Yes