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

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Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a waiver

MHRA-100958-PIP01-23

Scope of the Application

Active Substance(s)

bidridistrogene xeboparvovec

Condition(s)

Treatment of Limb-girdle muscular dystrophy

Pharmaceutical Form(s)

Solution for infusion

Route(s) of Administration

INTRAVENOUS USE

Name / Corporate name of the PIP applicant

Sarepta Therapeutics

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Sarepta Therapeutics submitted to the licensing authority on 30/05/2023 18:12 BST an application for a Paediatric Investigation Plan

The procedure started on 26/09/2023 07:56 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 waiver

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-100958-PIP01-23

Of 23/10/2024 09:17 BST

On the adopted decision for bidridistrogene xeboparvovec (MHRA-100958-PIP01-23) 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 bidridistrogene xeboparvovec, Solution for infusion , INTRAVENOUS USE .

This decision is addressed to Sarepta Therapeutics, 215 First St, Cambridge, UNITED STATES OF AMERICA, MA 02142

ANNEX I

1. Waiver

1.1 Condition:

Treatment of limb-girdle muscular dystrophy The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 4 years of age Pharmaceutical form(s): Solution for infusion Route(s) of administration: INTRAVENOUS USE Reason for granting waiver: on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of limb-girdle muscular dystrophy

2.2 Indication(s) targeted by the PIP:

Treatment of limb-girdle muscular dystrophy type 2E/R4

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

The paediatric population from 4 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Solution for infusion

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	2	Study 1 (2023-051) A long-term study to evaluate bidridistrogene xeboparvovec biodistribution in SGCB -/- mice. Study 2 (2023-024) Study to evaluate the reproductive transmission and maternal-foetal biodistribution of bidridistrogene xeboparvovec in mice.
Clinical Studies	4	Study 3 (SRP-9003-101) Open label trial to evaluate safety and activity of bidridistrogene xeboparvovec and to quantify expression of #-sarcoglycan in skeletal muscle in children from 4 years of age to less than 15 years with limb-girdle muscular dystrophy type 2E/R4 (LGMD2E/ R4). Study 4 (SRP-9003-102) Open-label, single-arm multicentre trial to evaluate the safety, tolerability, and activity of bidridistrogene xeboparvovec in ambulatory adults and non-ambulatory children from 4 years to less than 18 years of age (and adults) with LGMD2E/ R4. Study 5 (SRP-9003-301) Open label, single-dose, trial to evaluate the activity, safety, and tolerability of bidridistrogene xeboparvovec on ambulatory and non-ambulatory children from 4 years to less than 18 years of age (and adults) with LGMD2E/R4 Study

		6 (SRP-LGMD-501-NHS) Non-interventional, longitudinal natural history study in paediatric patients from 4 years of age to less than 18 years of age (and adults) with limb girdle muscular dystrophy (LGMD).
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:	30/11/2029
Deferral of one or more studies contained in the paediatric investigation plan:	No