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

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Decision Cover Letter

Decision of the licensing authority to:

accept of change(s) to the agreed paediatric investigation plan (MHRA-100979-PIP01-M01) and to the deferral

MHRA-100949-PIP01-23-M02

Scope of the Application

Active Substance(s)

delandistrogene moxeparvovec

Condition(s)

Treatment of Duchenne Muscular Dystrophy

Pharmaceutical Form(s)

Solution for infusion

Route(s) of Administration

INTRAVENOUS USE

Name / Corporate name of the PIP applicant

Roche Products Ltd

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Roche Products Ltd submitted to the licensing authority on 28/02/2025 08:10 GMT an application for a Modification

The procedure started on 14/04/2025 10:07 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 accept change(s) to the agreed paediatric investigation plan and to the 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-100949-PIP01-23-M02

Of 17/04/2025 15:15 BST

On the adopted decision for delandistrogene moxeparvovec (MHRA-100949-PIP01-23-M02) 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 modification of a paediatric investigation plan (including modification of a waiver or deferral included in that paediatric investigation plan).

This decision applies to a Modification for delandistrogene moxeparvovec, Solution for infusion , INTRAVENOUS USE .

This decision is addressed to Roche Products Ltd, 6 Falcon Way, Welwyn Garden City, UNITED KINGDOM, AL7 1TW

ANNEX I

1. Waiver

1.1 Condition:

Not applicable.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of Duchenne Muscular Dystrophy.

2.2 Indication(s) targeted by the PIP:

Treatment of Duchenne Muscular Dystrophy.

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

The paediatric population from birth 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	0	Not applicable
Clinical Studies	6	Study 1 [microDys-IV-001 (aka SRP-9001-101)] Open-label, single dose study to assess the safety of intravenous administration of delandistrogene moxeparvovec via peripheral limb vein in patients from 4 years to less than 8 years of age, inclusive with Duchenne Muscular Dystrophy (DMD). Study 2 (SRP-9001-102) Double-blind, randomised, placebo-controlled study to evaluate the safety and efficacy of delandistrogene moxeparvovec in paediatric patients from 4 years to less than 8 years of age with DMD. Study 3 (SRP-9001-301) Double-blind, randomised, placebo-controlled 2-part study to evaluate the safety and efficacy of delandistrogene moxeparvovec in paediatric patients from 4 years to less than 8 years of age with DMD. Study 4 (SRP-9001-302/ BN43881) Open-label, single arm study to evaluate the safety of delandistrogene moxeparvovec in paediatric patients from birth to less than 4 years of age with DMD (part 1) followed by a safety follow-up extension phase (part 2). Study 5 (SRP-9001-303) Randomised, double-blind, placebo-controlled study to evaluate efficacy and safety

		of delandistrogene moxeparvovec in non-ambulatory paediatric patients of less than 18 years (and adult patients) and ambulatory paediatric patients from 8 to less than 18 years of age with DMD. Study 6 (Added during procedure MHRA-100949-PIP01-23-M01) Open label, single arm study to evaluate the safety of delandistrogene moxeparvovec in paediatric patients at least 4 years of age with DMD caused by mutations involving exons 1-17 of the dystrophin gene (Part 1) followed by a safety follow-up extension phase (Part 2).
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:	Yes
Date of completion of the paediatric investigation plan:	31/05/2033
Deferral of one or more studies contained in the paediatric investigation plan:	Yes