EMA/575567/2022

European Medicines Agency decision

P/0230/2022

of 8 July 2022

on the acceptance of a modification of an agreed paediatric investigation plan for delandistrogene moxeparvovec (EMEA-002677-PIP01-19-M02) 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 Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the European Medicines Agency's decision P/0419/2020 issued on 22 October 2020 and the decision P/0052/2022 issued on 11 March 2022,

Having regard to the application submitted by Roche Registration GmbH on 15 February 2022 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 20 May 2022, 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, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for delandistrogene moxeparvovec, solution for infusion, intravenous 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 Roche Registration GmbH, 1 Emil-Barell-Strasse, 79639 - Grenzach-Wyhlen, Germany.

EMA/PDCO/132351/2022
Amsterdam, 20 May 2022

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

EMEA-002677-PIP01-19-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 Registration GmbH

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Roche Registration GmbH submitted to the European Medicines Agency on 15 February 2022 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0419/2020 issued on 22 October 2020 and the decision P/0052/2022 issued on 11 March 2022.

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

The procedure started on 21 March 2022.

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 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 are set out in 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 Duchenne Muscular Dystrophy

2.1.1. Indication(s) targeted by the PIP

Treatment of Duchenne Muscular Dystrophy (DMD)

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

From birth to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Solution for infusion

2.1.4. Measures

Area	**Description**
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	**Study 1** Open-label, single dose study to assess the safety of intravenous administration of SRP-9001 via peripheral limb vein in patients from 4 to less than 8 years of age, inclusive with Duchenne Muscular Dystrophy (DMD) [microDys-IV-001 (aka SRP-9001-101)]. **Study 2** Double-blind, randomized, placebo-controlled study to evaluate the safety and efficacy of SRP-9001 in paediatric patients from 4 to less than 8 years of age with DMD (SRP-9001-102). **Study 3** Double-blind, randomized, placebo-controlled 2-part study to evaluate the safety and efficacy of SRP-9001 in paediatric patients from 4 to less than 8 years of age with DMD (SRP-9001-301).

	Study 4 Open-label, single arm study to evaluate the safety of SRP-9001 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) (SRP-9001-302/BN43881). **Study 5** Randomised, double-blind, placebo-controlled 2-part study to evaluate efficacy and safety of SRP-9001 in non-ambulatory paediatric patients of less than 18 years of age (and adult patients) and ambulatory paediatric patients from 8 to less than 18 years of age with DMD (SRP-9001-303).
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:	Yes
Date of completion of the paediatric investigation plan:	By September 2027
Deferral for one or more measures contained in the paediatric investigation plan:	Yes