

EMA/485958/2020

European Medicines Agency decision

P/0419/2020

of 22 October 2020

on the agreement of a paediatric investigation plan and on the granting of a deferral for rAAVrh74.MHCK7.microdystrophin (SRP-9001) (EMA-002677-PIP01-19) 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 726/2004¹,

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency²,

Having regard to the application submitted by Sarepta Therapeutics Ireland on 20 December 2019 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 4 September 2020, 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.

² OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

A paediatric investigation plan for rAAVrh74.MHCK7.microdystrophin (SRP-9001), solution for injection, intravenous 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 rAAVrh74.MHCK7.microdystrophin (SRP-9001), solution for injection, intravenous 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 Roche Registration GmbH Emil-Barell-Strasse 1 - 79639 - Grenzach-Whylen - Germany.

EMA/PDCO/340691/2020
Amsterdam, 4 September 2020

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

EMA-002677-PIP01-19

Scope of the application

Active substance(s):

rAAVrh74.MHCK7.microdystrophin (SRP-9001)

Condition(s):

Treatment of Duchenne muscular dystrophy

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Sarepta Therapeutics Ireland

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Sarepta Therapeutics Ireland submitted for agreement to the European Medicines Agency on 20 December 2019 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 28 January 2020.

Supplementary information was provided by the applicant on 5 June 2020. 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 Norwegian Paediatric Committee member agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed paediatric investigation plan and the subset(s) of the paediatric population and condition(s) 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 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 injection

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable.
Non-clinical studies	0	Not applicable.
Clinical studies	5	Study 1 Open-label, single dose study to assess the safety of intravenous administration of SRP-9001 via peripheral limb vein in patients between 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 between 4 to less than 8 years of age with DMD (SRP-9001-102). Study 3 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-9001-301).

		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). Study 5 Randomised, double-blind, placebo-controlled study to evaluate efficacy and safety of SRP-9001 in non-ambulatory paediatric patients of less than 18 years of age with DMD (SRP-9001-303).
Extrapolation, modelling and simulation studies	0	Not applicable.
Other studies	0	Not applicable.
Other measures	0	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 October 2026
Deferral for one or more measures contained in the paediatric investigation plan:	Yes