



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/28205/2024

European Medicines Agency decision P/0073/2024

of 8 March 2024

on the acceptance of a modification of an agreed paediatric investigation plan for fordadistrogene movaparvovec (EMEA-002741-PIP01-20-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 726/2004¹,

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 Agency²,

Having regard to the European Medicines Agency's decision P/0518/2020 issued on 21 December 2020, and the decision P/0062/2023 issued on 24 February 2023,

Having regard to the application submitted by Pfizer Europe MA EEIG on 16 October 2023 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 19 January 2024, 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 fordadistrogene movaparvovec, concentrate for 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 Pfizer Europe MA EEIG, Boulevard de la Plaine 17, 1050 – Bruxelles, Belgium.



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/PDCO/479715/2023 Corr¹
Amsterdam, 19 January 2024

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA-002741-PIP01-20-M02

Scope of the application

Active substance(s):

Fordadistrogene movaparvovec

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of Duchenne muscular dystrophy

Pharmaceutical form(s):

Concentrate for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Pfizer Europe MA EEIG

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Pfizer Europe MA EEIG submitted to the European Medicines Agency on 16 October 2023 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0518/2020 issued on 21 December 2020, and the decision P/0062/2023 issued on 24 February 2023.

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

The procedure started on 20 November 2023.

¹ 28 February 2024.



Scope of the modification

Some measures 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 the Annex I of this opinion.

The Paediatric Committee member of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the paediatric investigation plan 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 annexes and appendix.

Annex I

The measures and timelines of the agreed paediatric investigation plan (PIP)

1. Waiver

Not applicable

1.1. Condition:

Treatment of Duchenne muscular dystrophy

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)

Concentrate for solution for infusion.

2.1.4. Measures

Area	Description
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	Study 1 (C3391001) Open-label, single arm, single ascending dose study to evaluate safety and tolerability of fordadistrogene movaparvovec (PF-06939926) in ambulatory and non-ambulatory patients with DMD. Study 2 (C3391003) Randomized (2:1), double-blind, placebo-controlled study to evaluate safety and efficacy of fordadistrogene movaparvovec (PF-06939926) in ambulatory paediatric patients from 4 to less than 8 years of age with DMD. Study 3 (C3391002) Randomized (1:1), double-blind, placebo-controlled study to evaluate safety and efficacy of fordadistrogene movaparvovec (PF-06939926) in non-ambulatory paediatric patients with DMD.

	Study 4 (C3391008) Single arm, open-label safety study of fordadistrogene movaparvovec (PF-06939926) in paediatric patients from birth to less than 4 years of age with DMD.
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 June 2036
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

The product is not authorised anywhere in the European Community.