EMA/21390/2023

European Medicines Agency decision

P/0062/2023

of 24 February 2023

on the acceptance of a modification of an agreed paediatric investigation plan for fordadistrogene movaparvovec (EMEA-002741-PIP01-20-M01) 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/0518/2020 issued on 21 January 2021.

Having regard to the application submitted by Pfizer Europe MA EEIG on 9 September 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 16 December 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, following a re-examination procedure of the Paediatric Committee's opinion according to Article 25(3) of Regulation (EC) No 1901/2006, 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.

EMA/34026/2023 Corr[1]
Amsterdam, 17 February 2023

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

EMEA-002741-PIP01-20-M01

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 9 September 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/0518/2020 issued on 21 January 2021.

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

[1] 22 February 2023

An Opinion was adopted by the Paediatric Committee on 16 December 2022 for the above mentioned product. Pfizer Europe MA EEIG received the Paediatric Committee Opinion on 22 December 2022.

On 19 January 2023 Pfizer Europe MA EEIG submitted to the European Medicines Agency a written request including detailed grounds for a re-examination of the Opinion.

The re-examination procedure started on 20 January 2023.

Some measures of the Paediatric Investigation Plan have been modified.

Final Opinion

1. The Paediatric Committee, having assessed the detailed grounds for re-examination, in accordance with Article 25(3) of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

1.1. to maintain its opinion and

- to agree to the changes regarding the measures in the scope set out in the Annex I of this opinion.

The Paediatric Committee members of Liechtenstein and Norway 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)) covered by the waiver 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 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

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)

Area	Description
	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.