EMA/581052/2020

European Medicines Agency decision

P/0518/2020

of 21 December 2020

on the agreement of a paediatric investigation plan and on the granting of a deferral for adeno-associated viral vector serotype 9 containing the human mini-dystrophin gene (PF-06939926) (EMEA-002741-PIP01-20) 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 Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency[2],

Having regard to the application submitted by Pfizer Europe MA EEIG on 19 February 2020 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 16 October 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, following a re-examination procedure of the Paediatric Committee's opinion according to Article 25(3) of Regulation (EC) No 1901/2006, 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 adeno-associated viral vector serotype 9 containing the human mini-dystrophin gene (PF-06939926), solution for infusion, 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 adeno-associated viral vector serotype 9 containing the human mini-dystrophin gene (PF-06939926), solution for infusion, 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 Pfizer Europe MA EEIG, Boulevard de la Plaine 17, 1050 – Bruxelles, Belgium.

EMA/PDCO/620974/2020 Corr
Amsterdam, 11 December 2020

Final opinion of the Paediatric Committee on the agreement of a Paediatric Investigation plan and a deferral

EMEA-002741-PIP01-20

Scope of the application

Active substance(s):

Adeno-associated viral vector serotype 9 containing the human mini-dystrophin gene (PF-06939926)

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:

Pfizer Europe MA EEIG

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Pfizer Europe MA EEIG submitted for agreement to the European Medicines Agency on 19 February 2020 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. Supplementary information was provided by the applicant on 13 July 2020. The applicant proposed modifications to the paediatric investigation plan and withdrew its request for a waiver.

An Opinion was adopted by the Paediatric Committee on 16 October 2020. Pfizer Europe MA EEIG received the Paediatric Committee Opinion on 27 October 2020.

On 17 November 2020 Pfizer Europe MA EEIG submitted to the European Medicines Agency a written request, including detailed grounds for re-examination of the Opinion.

The re-examination procedure started on 18 November 2020.

A meeting with the Paediatric Committee took place on 9 December 2020.

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 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 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 (DMD)

2.1.1. Indication(s) targeted by the PIP

Treatment of Duchenne Muscular Dystrophy

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	Number of measures	Description
Quality-related studies	0	Not applicable.
Non-clinical studies	0	Not applicable.
Clinical studies	4	**Study 1** (C3391001) Open-label, single arm, single ascending dose study to evaluate safety and tolerability of PF-06939926 in ambulatory and non-ambulatory patients from birth to less than 18 years of age with DMD. **Study 2** (C3391003) Randomized (2:1), double-blind, placebo-controlled study to evaluate safety and efficacy of 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 PF06939926 in non-ambulatory paediatric patients from birth to less than 18 years of age (and young adults) with DMD. **Study 4** (C3391008) Single arm, open-label safety study of PF-06939926 in paediatric patients from birth to less than 4 years of age with DMD.

Area	Number of measures	Description
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 June 2036
Deferral for one or more measures contained in the paediatric investigation plan:	Yes