EMA/377926/2020

European Medicines Agency decision

P/0292/2020

of 12 August 2020

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for marstacimab (EMEA-002285-PIP02-19) 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 726/2004[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 25 October 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 26 June 2020, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 of said Regulation and Article 13 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 and on the granting of a waiver.

(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.

(4) It is therefore appropriate to adopt a decision granting a waiver.

[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 marstacimab, solution for injection, subcutaneous 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 marstacimab, solution for injection, subcutaneous 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

A waiver for marstacimab, solution for injection, subcutaneous 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 4

This decision is addressed to Pfizer Europe MA EEIG, Boulevard de la Plaine 17, 1050 - Brussels, Belgium.

EMA/PDCO/610009/2019
Amsterdam, 26 June 2020

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

EMEA-002285-PIP02-19

Scope of the application

Active substance(s):

Marstacimab

Condition(s):

Treatment of congenital haemophilia A

Treatment of congenital haemophilia B

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Subcutaneous 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 25 October 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 3 December 2019.

Supplementary information was provided by the applicant on 24 March 2020. The applicant proposed modifications to the paediatric investigation plan.

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;
   - to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(a) of said Regulation, on the grounds that the specific medicinal product is likely to be ineffective or unsafe in part or all of the paediatric population.

   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) 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 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

1.1. Condition:

Treatment of congenital haemophilia A

The waiver applies to:

- the paediatric population from birth to less than 1 year of age;
- solution for injection, subcutaneous use;
- on the grounds that the specific medicinal product is likely to be unsafe.

1.2. Condition:

Treatment of congenital haemophilia B

The waiver applies to:

- the paediatric population from birth to less than 1 year of age;
- solution for injection, subcutaneous use;
- on the grounds that the specific medicinal product is likely to be unsafe.

2. Paediatric investigation plan

2.1. Condition:

Treatment of congenital haemophilia A

2.1.1. Indication(s) targeted by the PIP

Prophylaxis of bleeding episodes in patients with congenital haemophilia A (factor VIII deficiency), with and without inhibitors

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

From 1 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.

Area	Number of measures	Description
Non-clinical studies	0	Not applicable.
Clinical studies	2	**Study 1 (B7841005)** Non-randomised, open-label, intra-patient controlled, one-way, cross-over, single-arm study to evaluate the safety and efficacy of marstacimab prophylaxis versus previous standard of care on-demand and prophylaxis treatment in severe congenital haemophilia A and B patients with and without inhibitors from 12 to less than 18 years of age (and adults). **Study 2 (B7841008)** Non-randomised, open-label, intra-patient controlled, one-way, cross-over, single-arm study with age staggered enrolment, to evaluate the safety and efficacy of marstacimab prophylaxis versus previous standard of care on-demand and prophylaxis treatment in severe congenital haemophilia A and B patients with and without inhibitors from 1 to less than 18 years of age.
Extrapolation, modelling and simulation studies	3	**Study 3 (Modelling study 1)** Population PK Modelling and Simulation study to evaluate a suitable dose of marstacimab for adolescents from 12 to less than 18 years of age. **Study 4 (Modelling study 2)** Population PK Modelling and Simulation study to evaluate a suitable dose of marstacimab for children from 1 to less than 18 years of age. **Study 5 (Modelling study 3)** Population PK/PD exposure-response modelling and simulation study to characterize the PK/PD relationship in paediatric haemophilia patients from 1 to less than 18 years of age (and adults).
Other studies	0	Not applicable.
Other measures	0	Not applicable.

2.2. Condition:

Treatment of congenital haemophilia B

2.2.1. Indication(s) targeted by the PIP

Prophylaxis of bleeding episodes in patients with congenital haemophilia B (factor IX deficiency), with and without inhibitors

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

From 1 to less than 18 years of age.

2.2.3. Pharmaceutical form(s)

Solution for injection

2.2.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable.
Non-clinical studies	0	Not applicable.
Clinical studies	2	**Study 1** *Same as for condition Treatment of congenital haemophilia A, see above* **Study 2** *Same as for condition Treatment of congenital haemophilia A, see above*
Extrapolation, modelling and simulation studies	3	**Study 3** *Same as for condition Treatment of congenital haemophilia A, see above* **Study 4** *Same as for condition Treatment of congenital haemophilia A, see above* **Study 5** *Same as for condition Treatment of congenital haemophilia A, see above*
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 November 2028
Deferral for one or more measures contained in the paediatric investigation plan:	Yes