



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

EMADOC-1700519818-1859872

## European Medicines Agency decision

EMA/PE/0000226246

of 28 January 2025

on the acceptance of a modification of an agreed paediatric investigation plan for marstacimab 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.**



# European Medicines Agency decision

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of 28 January 2025

on the acceptance of a modification of an agreed paediatric investigation plan for marstacimab in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

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<sup>1</sup>,

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<sup>2</sup>,

Having regard to the European Medicines Agency's decision P/0292/2020 issued on 12 August 2020 and the decision P/0516/2021 issued on 3 December 2021 and the decision P/0443/2022 issued on 28 October 22,

Having regard to the application submitted by Pfizer Europe MA EEIG on 6 September 2024 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 13 December 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.

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<sup>1</sup> OJ L 378, 27.12.2006, p.1, as amended.

<sup>2</sup> OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

**Article 1**

Changes to the agreed paediatric investigation plan for marstacima, 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 Brussels, Brussels-Capital Region, Belgium.



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

EMADOC-1700519818-1704701

Amsterdam, 13 December 2024

## Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA/PE/0000226246

### 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 MAA EEIG

### Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Pfizer Europe MAA EEIG submitted to the European Medicines Agency on 6 September 2024 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/0292/2020 issued on 12 August 2020 and the decision P/0516/2021 issued on 3 December 2021 and the decision P/0443/2022 issued on 28 October 22.

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

The procedure started on 14 October 2024.

### Scope of the modification

Some measures and timelines 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 members of Liechtenstein and Norway agree with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the 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

## 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	Description
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	<p><b>Study 1 (B7841005)</b></p> <p>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 patients and moderately severe to severe congenital haemophilia B patients with and without inhibitors from 12 to less than 18 years of age (and adults)</p> <p><b>Study 2 (B7841008)</b></p> <p>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</p>
Extrapolation, modelling and simulation studies	<p><b>Study 3 (Modelling study 1)</b></p> <p>Population PK Modelling and Simulation study to evaluate a suitable dose of marstacimab for adolescents from 12 to less than 18 years of age</p> <p><b>Study 4 (Modelling study 2)</b></p> <p>Population PK Modelling and Simulation study to evaluate a suitable dose of marstacimab for children from 1 to less than 18 years of age</p> <p><b>Study 5 (Modelling study 3)</b></p> <p>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)</p>
Other studies	Not applicable
Other measures	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	Description
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	<b>Study 1</b> <i>Same as for condition Treatment of congenital haemophilia A, see above.</i> <b>Study 2</b> <i>Same as for condition Treatment of congenital haemophilia A, see above.</i>
Extrapolation, modelling and simulation studies	<b>Study 3</b> <i>Same as for condition Treatment of congenital haemophilia A, see above</i> <b>Study 4</b> <i>Same as for condition Treatment of congenital haemophilia A, see above.</i> <b>Study 5</b> <i>Same as for condition Treatment of congenital haemophilia A, see above</i>
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 November 2028
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.**