MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

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Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan and to the deferral

MHRA-100406-PIP01-21-M03

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 Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Pfizer Limited submitted to the licensing authority on 08/12/2025 10:15 GMT an application for a Modification

The procedure started on 09/12/2025 12:35 GMT

1. The licensing authority, having assessed the application in accordance with the Human Medicines Regulations 2012, decides, as set out in the appended summary report:

to accept change(s) to the agreed paediatric investigation plan and to the deferral.

2. The measures and timelines of the paediatric investigation plan are set out in the Annex I.

This decision is forwarded to the applicant, together with its annex and appendix.

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Final Decision Letter

MHRA-100406-PIP01-21-M03

Of 13/01/2026 09:14 GMT

On the adopted decision for MARSTACIMAB (MHRA-100406-PIP01-21-M03) in accordance with the Human Medicines Regulations 2012.

The licensing authority, in accordance with the Human Medicines Regulations 2012, has adopted this decision:

Agreement on modification of a paediatric investigation plan (including modification of a waiver or deferral included in that paediatric investigation plan).

This decision applies to a Modification for MARSTACIMAB, Solution for injection , SUBCUTANEOUS USE .

This decision is addressed to Pfizer Limited, Ramsgate Road, Sandwich, Kent, UNITED KINGDOM, CT139NJ

ANNEX I

1. Waiver

1.1 Condition:

Treatment of congenital haemophilia A. The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than1 years of age. Pharmaceutical form(s): Solution for injection Route(s) of administration: SUBCUTANEOUS USE Reason for granting waiver: on the grounds that the specific medicinal product is likely to be unsafe. 1.2 Condition: Treatment of congenital haemophilia B. The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than1 years of age. Pharmaceutical form(s): Solution for injection Route(s) of administration: SUBCUTANEOUS USE Reason for granting waiver: on the grounds that the specific medicinal product is likely to be unsafe.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Condition 1: Treatment of congenital haemophilia A Condition 2: Treatment of congenital haemophilia B

2.2 Indication(s) targeted by the PIP:

Condition 1: Prophylaxis of bleeding episodes in patients with congenital haemophilia A (factor VIII deficiency), with and without inhibitors. Condition 2: Prophylaxis of bleeding episodes in patients with congenital haemophilia B (factor IX deficiency), with and without inhibitors.

2.3 Subset(s) of the paediatric population concerned by the paediatric development:

For both conditions: The paediatric population from 1 year to less than 18 years of age.

2.4 Pharmaceutical Form(s):

For both conditions: Solution for injection

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not Applicable
Non-Clinical Studies	0	Not Applicable
Clinical Studies	2	(Same studies for both Conditions) Study 1 (87841005) 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). Study 2 (87841008) 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, Modeling & Simulation Studies	3	(Same studies for both Conditions) Study 3 (Modelling study 1) Population PK Modelling and Simulation study to evaluate a suitable dose of marstacimab for adolescents from 12 years 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 year to less than 18 years of age. Study 5 (Modelling study 3) Population PK/ PD exposure-response modelling and simulation study to characterise the PK/PD relationship in paediatric haemophilia patients from 1 year to less than 18 years of age (and adults).
Other Studies	0	Not Applicable
Other Measures	0	Not Applicable

3. Follow-up, completion and deferral of a PIP:

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	30/11/2028
Deferral of one or more studies contained in the paediatric investigation plan:	Yes