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

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Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a waiver

MHRA-102076-PIP01-25

Scope of the Application

Active Substance(s)

EMICIZUMAB

Condition(s)

Treatment of von Willebrand Disease.

Pharmaceutical Form(s)

Solution for injection

Route(s) of Administration

SUBCUTANEOUS USE

Name / Corporate name of the PIP applicant

Roche Products Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Roche Products Limited submitted to the licensing authority on 13/08/2025 14:55 BST an application for a Paediatric Investigation Plan

The procedure started on 14/10/2025 18:14 BST

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 agree a paediatric investigation plan and grant a waiver

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-102076-PIP01-25

Of 03/11/2025 14:55 GMT

On the adopted decision for EMICIZUMAB (MHRA-102076-PIP01-25) 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 a paediatric investigation plan

This decision applies to a Paediatric Investigation Plan for EMICIZUMAB, Solution for injection , SUBCUTANEOUS USE .

This decision is addressed to Roche Products Limited, 6 Falcon Way, Shire Park, Welwyn Garden City, UNITED KINGDOM, AL7 1TW

ANNEX I

1. Waiver

1.1 Condition:

Treatment of von Willebrand Disease The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 1 month of age Pharmaceutical form(s): Solution for injection Route(s) of administration: SUBCUTANEOUS USE Reason for granting waiver: on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of von Willebrand Disease

2.2 Indication(s) targeted by the PIP:

Routine prophylaxis to prevent or reduce the frequency of bleeding episodes in children and adolescents with Type 3 von Willebrand Disease (VWD).

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

The paediatric population from 1 month to less than 18 years of age

2.4 Pharmaceutical Form(s):

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	1	Study 1 (WP45338) Open label, randomised study to evaluate the efficacy, safety, pharmacokinetics, and pharmacodynamics of prophylactic emicizumab in participants with Type 3 Von Willebrand's Disease (VWD) in children and adolescents from 1 month to less than 18 years of age.
Extrapolation, Modeling & Simulation Studies	1	Extrapolation Plan Study 1 is part of an extrapolation plan covering the paediatric population from 1 month of age to less than 2 years of age.
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:	No
Date of completion of the paediatric investigation plan:	31/05/2028
Deferral of one or more studies contained in the paediatric investigation plan:	No