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

MHRA-100823-PIP01-22-M01

Scope of the Application

Active Substance(s)

Recombinant Human A Disintegrin and Metalloprotease with Thrombospondin Type-1 Motifs 13 (rADAMTS13)

Condition(s)

Treatment of thrombotic thrombocytopenic purpura

Pharmaceutical Form(s)

Powder and solvent for solution for injection

Route(s) of Administration

INTRAVENOUS USE

Name / Corporate name of the PIP applicant

Takeda UK Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Takeda UK Limited submitted to the licensing authority on 19/12/2022 15:35 GMT an application for a Modification

The procedure started on 25/04/2023 14:37 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 accept change(s) to the agreed paediatric investigation plan

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-100823-PIP01-22-M01

Of 13/06/2023 19:41 BST

On the adopted decision for Recombinant Human A Disintegrin and Metalloprotease with Thrombospondin Type-1 Motifs 13 (rADAMTS13) (MHRA-100823-PIP01-22-M01) 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 Recombinant Human A Disintegrin and Metalloprotease with Thrombospondin Type-1 Motifs 13 (rADAMTS13), Powder and solvent for solution for injection , INTRAVENOUS USE .

This decision is addressed to Takeda UK Limited, One Kingdom Street, London, UNITED KINGDOM, W2 6BD

ANNEX I

1. Waiver

1.1 Condition:

Not applicable.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of thrombotic thrombocytopenic purpura (TTP).

2.2 Indication(s) targeted by the PIP:

Treatment of hereditary thrombotic thrombocytopenic purpura (TTP).

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

The paediatric population from birth to less than 18 years of age.

2.4 Pharmaceutical Form(s):

Powder and solvent for solution for injection.

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Development of a paediatric vial.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	2	Study 2 A Phase 1, prospective, uncontrolled, open-label, multicentre, dose-escalation study evaluating the safety, including immunogenicity, and pharmacokinetics of BAX 930 (rADAMTS13) in congenital thrombotic thrombocytopenic purpura (cTTP) in children from 12 years of age to less than 18 years of age (and in adults). Study 3 Prospective, randomised, active-controlled, open-label, multicentre, 2-period crossover study (rADAMTS13 versus standard of care [SoC], 1:1 randomisation, Periods 1 and 2), followed by a single arm continuation period (rADAMTS13, Period 3) to evaluate the safety and efficacy of rADAMTS13 in the prophylactic and on-demand treatment of patients with severe congenital thrombotic thrombocytopenic purpura cTTP in children from birth to less than 18 years of age (and in adults).
Extrapolation, Modeling & Simulation Studies	0	Not applicable.
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:	30/04/2024
Deferral of one or more studies contained in the paediatric investigation plan:	Yes