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-100614-PIP01-22-M03

Scope of the Application

Active Substance(s)

VONICOG ALFA

Condition(s)

Treatment of von Willebrand disease

Pharmaceutical Form(s)

Powder and solvent for solution for injection

Route(s) of Administration

INTRAVENOUS USE

Name / Corporate name of the PIP applicant

Baxalta Innovations GmBH

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Baxalta Innovations GmBH submitted to the licensing authority on 12/04/2024 16:29 BST an application for a Modification

The procedure started on 27/08/2024 10:27 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 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-100614-PIP01-22-M03

Of 18/09/2024 09:52 BST

On the adopted decision for VONICOG ALFA (MHRA-100614-PIP01-22-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 VONICOG ALFA, Powder and solvent for solution for injection , INTRAVENOUS USE .

This decision is addressed to Baxalta Innovations GmbH, Industriestrasse 67, Vienna, AUSTRIA, 1221

ANNEX I

1. Waiver

1.1 Condition:

Not applicable.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of von Willebrand Disease.

2.2 Indication(s) targeted by the PIP:

Prevention and treatment of bleeding episodes and for surgical and invasive procedures in paediatric patients (less than 18 years of age) with von Willebrand disease.

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	0	Not applicable.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	3	Study 1 (071102) Surgery Arm Open-label study to assess the safety and efficacy of vonicog alfa (rVWF) in elective and emergency surgeries in children diagnosed with severe hereditary VWD and to determine the pharmacokinetics (PK) of rVWF. Study 2 This study was deleted during procedure EMEA-001164-PIP01-11-M01. Study 3 (TAK-577-3001) This study was added during procedure EMEA-001164-PIP01-11-M04. Open-label, uncontrolled study to assess the efficacy and safety vonicog alfa for prophylaxis to prevent or reduce the frequency and/ or severity of bleeding episodes in children with von Willebrand disease. Study 5 (071102) On-Demand arm This study was added during procedure MHRA-100614-PIP01-22-M03. Open-label study to assess the safety and efficacy of vonicog alfa (rVWF), with or without rhFVIII product in the treatment of bleeding episodes in children diagnosed with severe hereditary VWD, and to determine the PK of rVWF.
Extrapolation, Modeling & Simulation Studies	1	Study 4 This study was added during procedure MHRA-100614-PIP01-22-M02. Population PK and PK/PD

		model to assess the similarity of exposure and PK/PD between adults and children, and to support the use of vonicog alfa across all paediatric age cohorts.
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:	30/11/2027
Deferral of one or more studies contained in the paediatric investigation plan:	Yes