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-100198-PIP01-21-M01

Scope of the Application

Active Substance(s)

RURIOCTOCOG ALFA PEGOL

Condition(s)

Treatment and prophylaxis of bleeding in patients with haemophilia A

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 26/07/2021 11:58 BST an application for a

The procedure started on 24/08/2022 11:44 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-100198-PIP01-21-M01

Of 16/09/2022 16:59 BST

On the adopted decision for HUMAN COAGULATION FACTOR VIII; RURIOCTOCOG ALFA PEGOL (MHRA-100198-PIP01-21-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 for HUMAN COAGULATION FACTOR VIII; RURIOCTOCOG ALFA PEGOL, Powder and solvent for solution for injection , Intravenous use .

This decision is addressed to Baxalta Innovations GmbH, Industriestrasse 67, Vienna, Austria, A-1221

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of congenital factor VIII deficiency

2.2 Indication(s) targeted by the PIP:

Treatment and prophylaxis of bleeding with haemophilia A (congenital factor VIII deficiency)

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	5	Study 1 Randomised, open-label, 2-arm study in adults and adolescents previously treated male patients (PTPs) with severe haemophilia A to evaluate efficacy, safety, and pharmacokinetic (PK) parameters of pegylated recombinant FVIII (BAX 855) for prophylaxis and treatment of bleeding. Study 2 Open-label, single-arm study to evaluate the efficacy and safety of BAX 855 in adults and paediatric male previously treated patients (PTPs) with severe haemophilia A undergoing elective major or minor emergency surgical, dental or other invasive procedures. Study 3 Open label, single-arm study in paediatric PTPs less than 12 years of age with severe haemophilia A to evaluate safety, immunogenicity, efficacy and PK parameters of BAX 855 for prophylaxis of bleeding. Study 4 Open label, randomised, single-arm study to evaluate safety including immunogenicity and efficacy of BAX 855 in previously untreated patients (PUPs) below 6 years of age with severe haemophilia A. Study 5 Prospective, open label, study to further evaluate safety, including long-term safety and efficacy of BAX 855 for prophylactic use including paediatric and adult PTPs with severe haemophilia A from other BAX 855 studies and BAX 855-naive patients.
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:	Yes
Date of completion of the paediatric investigation plan:	31/10/2024
Deferral of one or more studies contained in the paediatric investigation plan:	Yes