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-100465-PIP01-22-M01)

MHRA-100465-PIP01-22-M02

Scope of the Application

Active Substance(s)

efanesoctocog alfa

Condition(s)

Treatment of congenital 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

Swedish Orphan Biovitrum AB

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Swedish Orphan Biovitrum AB submitted to the licensing authority on 06/12/2022 20:39 GMT an application for a Modification

The procedure started on 09/03/2023 13:04 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

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-100465-PIP01-22-M02

Of 16/06/2023 19:20 BST

On the adopted decision for efanesoctocog alfa (MHRA-100465-PIP01-22-M02) 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 efanesoctocog alfa , Powder and solvent for solution for injection , INTRAVENOUS USE .

This decision is addressed to Swedish Orphan Biovitrum AB, Strandbergssgatan 49, Stockholm, SWEDEN, 112 76

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of congenital haemophilia A

2.2 Indication(s) targeted by the PIP:

Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital FVIII 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	2	Study 1 (EFC16293) Open-label study to evaluate pharmacokinetics, safety and efficacy of rFVIIIFc-VWF-XTEN in adolescents from 12 to less than 18 years of age (and adults) with severe haemophilia A. Study 2 (EFC16295) Open-label study to evaluate pharmacokinetics, safety and efficacy of rFVIIIFc-VWF-XTEN administered as prophylaxis in previously treated patients (PTPs) from birth to less than 12 years of age with severe haemophilia A.
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:	30/04/2023
Deferral of one or more studies contained in the paediatric investigation plan:	No