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

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 Biovitrium AB (publ)

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Swedish Orphan Biovitrium AB (publ) submitted to the licensing authority on 07/03/2022 14:42 GMT an application for a Modification

The procedure started on 20/09/2022 17:08 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-100465-PIP01-22-M01

Of 11/10/2022 16:36 BST

On the adopted decision for efanesoctocog alfa (MHRA-100465-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

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 Biovitrium AB (publ), 225 Second Avenue, Waltham, MA, UNITED STATES OF AMERICA, 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 (242HA301) 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 (242HA302) 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:	31/12/2022
Deferral of one or more studies contained in the paediatric investigation plan:	No