EMA/709081/2022

European Medicines Agency decision

P/0382/2022

of 10 October 2022

on the acceptance of a modification of an agreed paediatric investigation plan for efanesoctocog alfa (EMEA-002501-PIP01-18-M03) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

Disclaimer

This decision does not constitute entitlement to the rewards and incentives referred to in Title V of Regulation (EC) No 1901/2006.

Only the English text is authentic.

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The European Medicines Agency,

Having regard to the Treaty on the Functioning of the European Union,

Having regard to Regulation (EC) No 1901/2006 of the European Parliament and of the Council of 12 December 2006 on medicinal products for paediatric use and amending Regulation (EEC) No. 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004[1],

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the European Medicines Agency's decision P/0048/2020 issued on 29 January 2020, the decision P/0238/2020 issued on 16 June 2020 and the decision P/0095/2022 issued on 11 March 2022.

Having regard to the application submitted by Swedish Orphan Biovitrum AB (Publ) on 20 May 2022 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 22 July 2022, in accordance with Article 22 of Regulation (EC) No 1901/2006,

Having regard to Article 25 of Regulation (EC) No 1901/2006,

Whereas:

(1) The Paediatric Committee of the European Medicines Agency has given an opinion on the acceptance of changes to the agreed paediatric investigation plan.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

[1] OJ L 378, 27.12.2006, p.1, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for efanesoctocog alfa, powder and solvent for solution for injection, intravenous use, are hereby accepted in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

Article 2

This decision is addressed to Swedish Orphan Biovitrum AB (Publ), Tomtebodavägen 23A, Solna, 11276 – Stockholm, Sweden.

EMA/PDCO/575403/2022 Corr
Amsterdam, 22 July 2022

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMEA-002501-PIP01-18-M03

Scope of the application

Active substance(s):

Efanesoctocog alfa

Condition(s):

Treatment of congenital haemophilia

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 (Publ)

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Swedish Orphan Biovitrum AB (Publ) submitted to the European Medicines Agency on 20 May 2022 an application for modification of the agreed paediatric investigation plan as set out in the European Medicines Agency's decision P/0048/2020 issued on 29 January 2020, the decision P/0238/2020 issued on 16 June 2020 and the decision P/0095/2022 issued on 11 March 2022.

The application for modification proposed changes to the agreed paediatric investigation plan.

The procedure started on 11 July 2022.

Scope of the modification

Some timelines of the Paediatric Investigation Plan have been modified.

Opinion

1. The Paediatric Committee, having assessed the application in accordance with Article 22 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree to changes to the paediatric investigation plan in the scope set out in the Annex I of this opinion.

   The Paediatric Committee member of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the paediatric investigation plan are set out in the Annex I.

This opinion is forwarded to the applicant and the Executive Director of the European Medicines Agency, together with its annex and appendix.

Annex I

The subset(s) of the paediatric population and condition(s) covered by the waiver and the measures and timelines of the agreed paediatric investigation plan (PIP)

1. Waiver

Not applicable.

2. Paediatric investigation plan

2.1. Condition:

Treatment of congenital haemophilia A

2.1.1. Indication(s) targeted by the PIP

Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital FVIII deficiency)

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From birth to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Powder and solvent for solution for injection

2.1.4. Measures

Area	**Description**
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	**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, modelling and simulation studies	Not applicable
Other studies	Not applicable
Other measures	Not applicable

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By April 2023
Deferral for one or more measures contained in the paediatric investigation plan:	No