



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/83075/2020

European Medicines Agency decision P/0071/2020

of 18 March 2020

on the acceptance of a modification of an agreed paediatric investigation plan for Eftrenonacog alfa (Alprolix) (EMEA-000914-PIP01-10-M05) 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 726/2004¹,

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency²,

Having regard to the European Medicines Agency's decision P/123/2011 issued on 7 June 2011, the decision P/0198/2013 issued on 2 September 2013, the decision P/0303/2014 issued on 24 November 2014, the decision P/0261/2016 issued on 5 October 2016 and the decision P/0296/2018 issued on 12 September 2018,

Having regard to the application submitted by Swedish Orphan Biovitrum AB (publ) on 16 October 2019 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 31 January 2020, 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 and to the deferral.
- (2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan, including changes to the deferral.

¹ OJ L 378, 27.12.2006, p.1.

² OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for Eftrenonacog alfa (Alprolix), powder and solvent for solution for injection , intravenous use, including changes to the deferral, 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, 112 76 – Stockholm, Sweden.



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/PDCO/607544/2019
Amsterdam, 31 January 2020

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA-000914-PIP01-10-M05

Scope of the application

Active substance(s):

Eftrenonacog alfa

Invented name:

Alprolix

Condition(s):

Treatment of hereditary factor IX deficiency

Authorised indication(s):

See Annex II

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)

Information about the authorised medicinal product:

See Annex II



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 16 October 2019 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/123/2011 issued on 7 June 2011, the decision P/0198/2013 issued on 2 September 2013, the decision P/0303/2014 issued on 24 November 2014, the decision P/0261/2016 issued on 5 October 2016 and the decision P/0296/2018 issued on 12 September 2018.

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

The procedure started on 3 December 2019.

Scope of the modification

Some measures or 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 and to the deferral in the scope set out in the Annex I of this opinion.

The Norwegian Paediatric Committee member 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 annexes 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 hereditary factor IX deficiency

2.1.1. Indication(s) targeted by the PIP

Treatment and prophylaxis of bleeding in patients with Haemophilia B (hereditary factor IX 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	Number of studies	Description
Quality-related studies	1	Study 1 Development of a 250 IU/vial formulation (powder and solvent for solution for injection)
Non-clinical studies	0	Not applicable
Clinical studies	3	Study 2 Open-label, multicentre, uncontrolled study to evaluate the safety, pharmacokinetics, and efficacy of recombinant human coagulation factor IX attached to the Fc domain of human IgG1 (rFIXFc) in the prevention and treatment of bleeding in previously treated subjects, 12 years of age or above, with Haemophilia B Study 3 Open-label, multicentre, uncontrolled trial to evaluate the safety, pharmacokinetics, and efficacy of rFIXFc in the prevention and treatment of bleeding in previously treated patients less than 12 years of age with Haemophilia B

		Study 4 Open-label, multicentre, uncontrolled trial to evaluate the safety and efficacy of rFIXFc in the prevention and treatment of bleeding in previously untreated patients (PUPs) with Haemophilia B
Extrapolation, modelling and simulation studies	0	Not applicable
Other studies	0	Not applicable
Other measures	0	Not applicable

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By August 2019
Deferral for one or more studies contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

1. Treatment of hereditary factor IX deficiency

Authorised indication(s):

- Treatment and prophylaxis of bleeding in patients with Haemophilia B (congenital factor IX deficiency). Alprolix can be used in all age groups.

Authorised pharmaceutical form(s):

Powder and solvent for solution for injection

Authorised route(s) of administration:

Intravenous use