EMA/538894/2018

European Medicines Agency decision

P/0296/2018

of 12 September 2018

on the acceptance of a modification of an agreed paediatric investigation plan for eftrenonacog alfa (Alprolix), (EMEA-000914-PIP01-10-M04) 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.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Contact EMA www.ema.europa.eu/contact

European Medicines Agency decision

P/0296/2018

of 12 September 2018

on the acceptance of a modification of an agreed paediatric investigation plan for eftrenonacog alfa (Alprolix), (EMEA-000914-PIP01-10-M04) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

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 Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency[2],

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 and the decision P/0261/2016 issued on 5 October 2016,

Having regard to the application submitted by Swedish Orphan Biovitrum AB (publ) on 27 April 2018 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 27 July 2018, 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.
[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, 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.

EMA/PDCO/299331/2018 Corr
London, 27 July 2018

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

EMEA-000914-PIP01-10-M04

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

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

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 27 April 2018 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 and the decision P/0261/2016 issued on 5 October 2016.

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

The procedure started on 29 May 2018.

Scope of the modification

Some measures 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 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 June 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