EMA/480337/2019

European Medicines Agency decision

P/0329/2019

of 10 September 2019

on the acceptance of a modification of an agreed paediatric investigation plan for lonoctocog alfa (Afstyla) (EMEA-001215-PIP01-11-M07) 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 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/0215/2012 issued on 28 September 2012, the decision P/0189/2014 issued on 6 August 2014, the decision P/0335/2014 issued on 22 December 2014, the decision P/0109/2015 issued on 1 June 2015, the decision P/0227/2015 issued on 2 October 2015, the decision P/0183/2017 issued on 3 July 2017 and the decision P/0058/2018 issued on 16 March 2018,

Having regard to the application submitted by CSL Behring GmbH on 18 April 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 26 July 2019, 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 lonoctocog alfa (Afstyla), 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 CSL Behring GmbH, Emil-von-Behring-Str. 76, 35041- Marburg, Germany.

EMA/PDCO/261942/2019
Amsterdam, 26 July 2019

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

EMEA-001215-PIP01-11-M07

Scope of the application

Active substance(s):

Lonoctocog alfa

Invented name:

Afstyla

Condition(s):

Treatment of congenital factor VIII 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:

CSL Behring GmbH

Information about the authorised medicinal product:

See Annex II

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, CSL Behring GmbH submitted to the European Medicines Agency on 18 April 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/0215/2012 issued on 28 September 2012, the decision P/0189/2014 issued on 6 August 2014, the decision P/0335/2014 issued on 22 December 2014, the decision P/0109/2015 issued on 1 June 2015, the decision P/0227/2015 issued on 2 October 2015, the decision P/0183/2017 issued on 3 July 2017 and the decision P/0058/2018 issued on 16 March 2018.

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

The procedure started on 28 May 2019.

Scope of the modification

Some measures and 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 congenital factor VIII Deficiency

2.1.1. Indication(s) targeted by the PIP

Treatment and prophylaxis of bleeding in patients with congenital factor VIII 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 measures	Description
Quality-related studies	0	Not applicable
Non-clinical studies	0	Not applicable
Clinical studies	3	**Study 1** Open-label, pharmacokinetic, safety, efficacy study for on-demand and prophylaxis treatment of haemophilia including a surgical sub-study in previously treated patients with severe haemophilia A from 12 to less than 18 years of age and adults (CSL627_1001). **Study 2** Open-label, pharmacokinetic, safety and efficacy study for on-demand and prophylaxis treatment of haemophilia in previously treated patients with severe haemophilia A below 12 years of age (CSL627_3002). **Study 3** Open-label, safety and efficacy study for on-demand and prophylaxis treatment of haemophilia in previously untreated patients (PUPs) from birth to less than 18 years of age and adults with haemophilia A (CSL627_3004).

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 and efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By July 2025
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 congenital factor VIII deficiency

Authorised indication(s):

- Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency).

  AFSTYLA can be used for all age groups.

Authorised pharmaceutical form(s):

Powder and solvent for solution for injection / infusion

Authorised route(s) of administration:

Intravenous use