



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/147275/2015

European Medicines Agency decision

P/0070/2015

of 1 April 2015

on the acceptance of a modification of an agreed paediatric investigation plan for damoctocog alfa pegol (EMEA-001229-PIP01-11-M01) 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/0216/2012 issued on 28 September 2012,

Having regard to the application submitted by Bayer Pharma AG on 19 November 2014 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 13 February 2015, 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 a refusal of changes to the deferral.
- (2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan and a refusal of 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 damoctocog alfa pegol, powder and solvent for solution for injection, intravenous use, refusing 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 Bayer Pharma AG, Muellerstrasse 178, 13353 – Berlin, Germany.

Done at London, 1 April 2015

For the European Medicines Agency
Jordi Llinares Garcia
Head of Division (ad interim)
Human Medicines Research and Development Support
(Signature on file)

EMA/PDCO/757289/2014 **Corr**

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

EMA-001229-PIP01-11-M01

Scope of the application

Active substance(s):

Damoctocog alfa pegol

Condition(s):

Treatment of hereditary factor VIII deficiency

Pharmaceutical form(s):

Powder and solvent for solution for injection

Route(s) of administration:

Intravenous use

Name / corporate name of the PIP applicant:

Bayer Pharma AG

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Bayer Pharma AG submitted to the European Medicines Agency on 19 November 2014 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0216/2012 issued on 28 September 2012.

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

The procedure started on 16 December 2014.

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 and to refuse the change 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 annex and appendix.

London, 13 February 2015.

On behalf of the Paediatric Committee
Dr Dirk Mentzer, Chairman
(Signature on file)

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 VIII deficiency

2.1.1. Indication(s) targeted by the PIP

Treatment and prophylaxis of bleeding in patients with Haemophilia A (hereditary 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 studies	Description
Quality-related studies	1	Study 1: Development of a 250 IU vial size.
Non-clinical studies	0	Not applicable.
Clinical studies	3	Study 2: Open-label, uncontrolled trial to evaluate pharmacokinetics, safety and efficacy of damoctocog alfa pegol for prophylaxis and treatment of bleeding in previously treated patients (PTP) from 12 to less than 18 years (and adults), with severe hereditary factor VIII deficiency. Study 3: Open-label, uncontrolled trial to evaluate pharmacokinetics, safety and efficacy of damoctocog alfa pegol for prophylaxis and treatment of bleeding in previously treated patients (PTP) less than 12 years of age, with severe hereditary factor VIII deficiency. Study 4: Open-label, uncontrolled trial to evaluate safety and efficacy of damoctocog alfa pegol for prophylaxis and treatment of bleeding in previously untreated children (PUP) with severe hereditary factor VIII deficiency.

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 December 2020
Deferral for one or more studies contained in the paediatric investigation plan:	Yes