EMA/585994/2013

European Medicines Agency decision

P/0270/2013

of 30 October 2013

on the agreement of a paediatric investigation plan and on the granting of a waiver for eptacog alfa (activated) (EMEA-001382-PIP01-12) 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 application submitted by Baxter Innovations GmbH on 6 December 2012 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 13 September 2013, in accordance with Article 18 of Regulation (EC) No 1901/2006, and Article 13 of said Regulation,

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 agreement of a paediatric investigation plan and on the granting of a waiver.

(2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.

(3) It is therefore appropriate to adopt a decision granting a waiver.

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

Has adopted this decision:

Article 1

A paediatric investigation plan for eptacog alfa (activated), powder and solvent for solution for injection, intravenous use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

Article 2

A waiver for eptacog alfa (activated), powder and solvent for solution for injection, intravenous use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

This decision is addressed to Baxter Innovations GmbH, Industriestrasse 67, 1221 - Vienna, Austria.

Done at London, 30 October 2013

For the European Medicines Agency
Guido Rasi
Executive Director
(Signature on file)

EMA/PDCO/387447/2013

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a waiver

EMEA-001382-PIP01-12

Scope of the application

Active substance:

Eptacog alfa (activated)

Condition:

Treatment of congenital coagulation disorders

Pharmaceutical form:

Powder and solvent for solution for injection

Route of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Baxter Innovations GmbH

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Baxter Innovations GmbH submitted for agreement to the European Medicines Agency on 6 December 2012 an application for a paediatric investigation plan for the above mentioned medicinal product, a deferral under Article 20 of said Regulation, and a waiver under Article 13 of said Regulation.

The procedure started on 16 January 2013.

Supplementary information was provided by the applicant on 21 June 2013. The applicant proposed modifications to the paediatric investigation plan and withdrew its request for a deferral.

A meeting with the Paediatric Committee took place on 11 September 2013.

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Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree the paediatric investigation plan in accordance with Article 18 of said Regulation;
   - to grant a waiver for one subset of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(b) of said Regulation, on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified subset(s) of the paediatric population.

   The Icelandic and the Norwegian Paediatric Committee members agree with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed paediatric investigation plan and the subset of the paediatric population and condition covered by the waiver 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 September 2013

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

1. Waiver

1.1. Condition: treatment of congenital coagulation disorders

The waiver applies to:

- The paediatric population from birth to less than 6 months of age;
- for powder and solvent for solution for injection, for intravenous use;
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

2. Paediatric Investigation Plan

2.1. Condition: treatment of congenital coagulation disorders

2.1.1. Indication(s) targeted by the PIP

Treatment of bleeding episodes in haemophilia A patients with inhibitors to Factor VIII.

Treatment of bleeding episodes in haemophilia B patients with inhibitors to Factor IX.

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

From 6 months to less than 18 years of age.

2.1.3. Pharmaceutical form

Powder and solvent for solution for injection.

2.1.4. Measures

Area	Number of measures	Description
Quality	1	**Measure 1:** Development of a pharmaceutical form, for intravenous use, with a concentration of Polysorbate 80 not exceeding 0.014%.
Non-clinical	0	Not applicable.
Clinical	3	**Measure 2:** Open-label, randomised, multi-centre, non-comparative, multiple dose study to evaluate safety, and activity of eptacog alfa (activated), in the treatment of bleeding episodes per an on-demand regimen in children from 12 to less than 18 years of age and adults, with haemophilia A and inhibitors to Factor VIII or haemophilia B and inhibitors to Factor IX.

Area	Number of measures	Description
		Measure 3: Open-label, multi-centre, non-comparative, single dose study to evaluate pharmacokinetics, safety, activity and immunogenicity of eptacog alfa (activated), in the treatment of bleeding episodes per an on-demand regimen in children from 6 months to less than 12 years of age, with haemophilia A and inhibitors to Factor VIII or haemophilia B and inhibitors to Factor IX. **Measure 4:** Open-label, multi-centre, non-comparative, single dose study to evaluate the pharmacokinetics, safety, activity and immunogenicity of eptacog alfa (activated), during surgical/invasive procedures in children from 6 years to less than 18 years of age and adults, with haemophilia A and inhibitors to Factor VIII or haemophilia B and inhibitors to Factor IX, in the peri and post-operative setting.

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 June 2017
Deferral for one or more measures contained in the paediatric investigation plan:	No