

EMA/506548/2012

European Medicines Agency decision

P/0072/2013

of 26 March 2013

on the agreement of a paediatric investigation plan and on the granting of a deferral for pegylated human recombinant factor VIII (BAX 855) (EMEA-001296-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 726/2004¹,

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 application submitted by Baxter Innovations GmbH on 11 May 2012 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 8 February 2013, in accordance with Article 18 of Regulation (EC) No 1901/2006, and Article 21 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 deferral.
- (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 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

A paediatric investigation plan for pegylated human recombinant factor VIII (BAX 855), 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 deferral for pegylated human recombinant factor VIII (BAX 855), 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, 67 Industriestrasse, 1221 – Vienna, Austria.

Done at London, 26 March 2013

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

EMA/PDCO/494858/2012

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

EMA-001296-PIP01-12

Scope of the application

Active substance(s):

Pegylated human recombinant factor VIII (BAX 855)

Condition(s):

Treatment of congenital 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:

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 11 May 2012 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation.

The procedure started on 19 June 2012.

Supplementary information was provided by the applicant on 19 November 2012. The applicant proposed modifications to the paediatric investigation plan.

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 deferral in accordance with Article 21 of said Regulation.

The Norwegian Paediatric Committee member agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed 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, 8 February 2013

On behalf of the Paediatric Committee
Dr Daniel Brasseur, 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

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 (haemophilia A)

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. Studies

Area	Number of Measures	Description
Quality	0	Not applicable.
Non-clinical	0	Not applicable.
Clinical	5	Measure 1: Randomised, open-label, 2-arm study in adults and adolescents previously treated male patients (PTPs) with severe haemophilia A to evaluate efficacy, safety, and pharmacokinetic (PK) parameters of pegylated recombinant FVIII (BAX 855) for prophylaxis and treatment of bleeding. Measure 2: Open-label, single-arm study to evaluate the efficacy and safety of BAX 855 in adults and adolescents male previously treated patients (PTP) with severe haemophilia A undergoing elective major or minor, surgical, dental or other invasive procedures. Measure 3: Open label, 2-arm study in paediatric male PTPs less than 12 years of age with severe haemophilia A to evaluate efficacy, safety, and PK parameters of BAX 855 for prophylaxis and treatment of bleeding.

		Measure 4: Open label, randomised, 2-arm study in previously untreated patients (PUPs) with severe haemophilia A to evaluate safety and efficacy of BAX 855 for prophylaxis of bleeding. Measure 5: Two-part, 4-arm study in male PTPs or PUPs with severe haemophilia A to evaluate long-term immunogenicity, safety and efficacy of BAX 855.
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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 2022
Deferral for one or more measures contained in the paediatric investigation plan:	Yes