EMA/268980/2014

European Medicines Agency decision

P/0139/2014

of 11 June 2014

on the acceptance of a modification of an agreed paediatric investigation plan for PEGylated recombinant factor VIII (EMEA-001296-PIP01-12-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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on the acceptance of a modification of an agreed paediatric investigation plan for PEGylated recombinant factor VIII (EMEA-001296-PIP01-12-M01) 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/0072/2013 issued on 26 March 2013,

Having regard to the application submitted by Baxter Innovations GmbH on 3 February 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 21 March 2014, in accordance with Article 22 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 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 PEGylated recombinant factor VIII, 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 Baxter Innovations GmbH, Industriestrasse 67, 1221 – Vienna, Austria.

Done at London, 11 June 2014

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

EMA/PDCO/78604/2014

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

EMEA-001296-PIP01-12-M01

Scope of the application

Active substance(s):

PEGylated recombinant factor VIII

Condition(s):

Treatment and prophylaxis of bleeding in patients with haemophilia A (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 22 of Regulation (EC) No 1901/2006 as amended, Baxter Innovations GmbH submitted to the European Medicines Agency on 03 February 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/0072/2013 issued on 26 March 2013, .

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

The procedure started on 26 February 2014.

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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 and the subset(s) of the paediatric population and condition(s) 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, 21 March 2014

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

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. Measures

Area	Number of Measures	Description
Quality	0	Not applicable.
Non-clinical	0	Not applicable.
Clinical	5	Study 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. Study 2: Open-label, single-arm study to evaluate the efficacy and safety of BAX 855 in adults and adolescents male previously treated patients (PTPs) with severe haemophilia A undergoing elective major or minor, or minor emergency surgical, dental or other invasive procedures. Study 3: Open label, single-arm study in paediatric male PTPs less than 12 years of age with severe haemophilia A to evaluate safety,

		immunogenicity, efficacy and PK parameters of BAX 855 for prophylaxis of bleeding. Study 4: Open label, randomised, single-arm study to evaluate safety including immunogenicity and efficacy of BAX 855 in previously untreated patients (PUPs) below 6 years of age with severe haemophilia A. Study 5: Prospective, open label, study to further evaluate safety, including long-term safety and efficacy of BAX 855 for prophylactic use including paediatric and adult PTPs with severe hemophilia A from other BAX 855 studies and BAX 855-naïve patients.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By June 2021
Deferral for one or more measures contained in the paediatric investigation plan:	Yes