

EMA/494657/2012

European Medicines Agency decision

P/0216/2012

of 28 September 2012

on the agreement of a paediatric investigation plan and on the granting of a deferral for pegylated B-domain-deleted sequence-modified recombinant human factor VIII, (EMA-001229-PIP01-11) 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 Bayer Pharma AG on 4 November 2011 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 17 August 2012, 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 B-domain-deleted sequence-modified recombinant human factor VIII, 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 B-domain-deleted sequence-modified recombinant human factor VIII, 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 Bayer Pharma AG, Muellerstrasse 178, 13353 – Berlin, Germany.

Done at London, 28 September 2012

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

EMA/PDCO/383556/2012

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

EMA-001229-PIP01-11

Scope of the application

Active substance(s):

Pegylated B-domain-deleted sequence-modified recombinant human factor VIII

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 16(1) of Regulation (EC) No 1901/2006 as amended, Bayer Pharma AG submitted for agreement to the European Medicines Agency on 4 November 2011 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 14 December 2011.

Supplementary information was provided by the applicant on 16 May 2012. The applicant proposed modifications to the paediatric investigation plan and withdrew its request for a waiver.

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(es) and appendix.

London, 17 August 2012

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 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. Studies

Area	Number of studies	Description
Quality	1	Study 1: Development of a 250 IU vial size.
Non-clinical	0	Not applicable.
Clinical	3	Study 2: Open-label, uncontrolled trial to evaluate pharmacokinetics, safety and efficacy of Pegylated B-domain-deleted sequence-modified recombinant human factor VIII (BAY 94-9027) 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 BAY 94-9027 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 BAY 94-9027 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 and 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.