



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/878747/2011

European Medicines Agency decision P/293/2011

of 8 December 2011

on the agreement of a paediatric investigation plan and on the granting of a deferral for nonacog alfa (recombinant coagulation factor IX) (EMEA-001139-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 Baxter Innovations GmbH on 14 February 2011 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 11 November 2011, 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 nonacog alfa (recombinant coagulation factor IX), 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 nonacog alfa (recombinant coagulation factor IX), 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, A-1221- Vienna, Austria.

Done at London, 8 December 2011

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



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EMA/PDCO/719743/2011

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

EMA-001139-PIP01-11

Scope of the application

Active substance(s):

Nonacog alfa (recombinant coagulation factor IX)

Condition(s):

Treatment of haemophilia B (congenital factor IX 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 14 February 2011 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 23 March 2011.

Supplementary information was provided by the applicant on 26 August 2011.



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 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 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, 11 November 2011

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 Haemophilia B (congenital factor IX deficiency)

2.1.1. Indication(s) targeted by the PIP

Treatment and prophylaxis of bleeding in patients with haemophilia B (congenital factor IX 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	0	Not applicable.
Non-clinical	0	Not applicable.
Clinical	5	Study 1: Prospective study in previously treated patients (PTPs) with severe or moderately severe haemophilia B to assess PK, efficacy, immunogenicity, and safety of nonacog alfa for the prophylaxis and on-demand treatment Study 2: Prospective, open-label, extension study of nonacog alfa in PTPs with severe or moderately severe haemophilia B who have completed Baxter clinical study protocols 250901 or 251101. Study 3: Prospective, open-label, uncontrolled study of nonacog alfa in PTPs with severe or moderately severe haemophilia B undergoing minor and major emergency and elective surgical, dental, or other invasive procedures. Study 4: Prospective, uncontrolled study to assess PK, efficacy, safety, and immunogenicity of nonacog alfa in paediatric subjects with severe or moderately severe haemophilia B. Study 5: Prospective study of nonacog alfa in previously untreated patients (PUPs) with severe or moderately severe haemophilia B.

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 March 2019.
Deferral for one or more studies contained in the paediatric investigation plan:	Yes.