



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/443546/2012

European Medicines Agency decision

P/0159/2012

of 25 July 2012

on the acceptance of a modification of an agreed paediatric investigation plan for nonacog alfa, (EMA-001139-PIP01-11-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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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 European Medicines Agency's decision P/293/2011 issued on 8 December 2011,

Having regard to the application submitted by Baxter Innovations GmbH on 23 March 2012 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 8 June 2012, in accordance with Article 22 of Regulation (EC) No 1901/2006,

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.
- (2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

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 nonacog alfa, powder and solvent for solution for injection, intravenous use, 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, A-1221 – Vienna, Austria.

Done at London, 25 July 2012

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

EMA/443546/2012

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA-001139-PIP01-11-M01

Scope of the application

Active substance(s):

Nonacog alfa

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 22 of Regulation (EC) No 1901/2006 as amended, Baxter Innovations GmbH submitted to the European Medicines Agency on 23 March 2012 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/293/2011 issued on 8 December 2011.

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

The procedure started on 16 April 2012.

Scope of the modification

Some measures 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 in the scope set out in the Annex I of this opinion.

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 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, 8 June 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 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.

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 2015.
Deferral for one or more studies contained in the paediatric investigation plan:	Yes.