EMA/720293/2010

European Medicines Agency decision

P/235/2010

of 26 November 2010

on the acceptance of a modification of an agreed paediatric investigation plan for eptacog alfa pegol (activated) (EMEA-000189-PIP01-08-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 726/2004[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/28/2009 issued on 23 February 2009,

Having regard to the application submitted by Novo Nordisk A/S on 15 July 2010 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 8 October 2010, 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 eptacog alfa pegol (activated), powder for solution for injection, intravenous use, subcutaneous 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 Novo Nordisk A/S, Novo Alle, 2880 Bagsvaerd, Denmark.

Done at London, 26 November 2010

For the European Medicines Agency
Thomas Lönngren
Executive Director

(Signature on file)

EMA/PDCO/539326/2010

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

EMEA-000189-PIP01-08-M01

Scope of the application

Active substance(s):

Eptacog alfa pegol (activated)

Condition(s):

Hereditary factor VIII and factor IX deficiency

Pharmaceutical form(s):

Powder for solution for injection

Route(s) of administration:

Intravenous use

Subcutaneous use

Name/corporate name of the PIP applicant:

Novo Nordisk A/S

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Novo Nordisk A/S submitted to the European Medicines Agency on 15 July 2010 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/28/2009 issued on 23 February 2009.
The application for modification proposed changes to the agreed paediatric investigation.

The procedure started on 12 August 2010.

Scope of the modification

Some measures and timelines of the initial opinion have been modified.

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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 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(es) and appendix.

London, 8 October 2010

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

1.1. Condition: Hereditary factor VIII and factor IX deficiency

The waiver applies to:

- Children from birth to less than 6 months
- for powder for solution for injection, intravenous use and subcutaneous use
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2. Paediatric Investigation Plan

2.1. Condition: Hereditary factor VIII and factor IX deficiency

2.1.1. Indication(s) targeted by the PIP

Prevention of bleeding (secondary prophylaxis) in haemophilia A patients with inhibitors to Factor VIII.
Prevention of bleeding (secondary prophylaxis) in haemophilia B patients with inhibitors to Factor IX.

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From 6 months to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Powder for solution for injection

Intravenous use, subcutaneous use

2.1.4. Studies

Area	Number of studies	Description
Quality	1	Development of a subcutaneous formulation of pegylated recombinant FVIIa.
Non-clinical		Not applicable.
Clinical	6	Study 1: A randomised, placebo-controlled, double-blind single dose, dose-escalation trial investigating safety and pharmacokinetics of ascending intravenous doses of pegylated recombinant FVIIa in healthy male subjects Study 2: A randomised, placebo-controlled, double-blind, single dose, dose escalation trial to evaluate safety and pharmacokinetics of ascending subcutaneous doses of pegylated recombinant FVIIa in healthy male subjects. Study 3: An open label, non-randomised trial to evaluate safety and

		pharmacokinetics of intravenous administrations of pegylated recombinant FVIIa in haemophilia A and B patients (with or without inhibitors) above 18 years. Study 4: A randomised, double-blind, parallel-arm trial to evaluate safety, pharmacokinetics and efficacy of multiple intravenous administrations of pegylated recombinant FVIIa in inhibitor patients from 12 years to 65 years. Study 5: An open label, non-randomised trial to evaluate safety and pharmacokinetics of a single dose intravenous administration of pegylated recombinant FVIIa in haemophilia A and B patients (with or without inhibitors) from 6 months to less than 12 years. Study 6: An open label, randomised, parallel arm, controlled trial to evaluate safety, efficacy and immunogenicity of pegylated recombinant FVIIa prophylaxis in inhibitor patients from 6 months to 65 years.

3. Follow-up, completion and deferral of PIP

Measures to address long term follow-up of potential safety issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By April 2016
Deferral for one or more studies contained in the paediatric investigation plan:	Yes