EMA/766294/2011

European Medicines Agency decision

P/236/2011

of 30 September 2011

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for vatreptacog alfa (activated) (EMEA-000328-PIP02-09) 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 application submitted by Novo Nordisk A/S on 11 February 2010 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 12 August 2011, in accordance with Article 18 of Regulation (EC) No 1901/2006, and Article 21 of said Regulation and Article 13 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 and on the granting of a waiver.

(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.

(4) It is therefore appropriate to adopt a decision granting a waiver.

[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 vatreptacog alfa (activated), 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 vatreptacog alfa (activated), 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

A waiver for vatreptacog alfa (activated), 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 4

This decision is addressed to Novo Nordisk A/S, Novo Allé, 2880 - Bagsværd, Denmark.

Done at London, 30 September 2011

For the European Medicines Agency
Andreas Pott
Acting Executive Director
(Signature on file)

EMA/PDCO/498927/2011

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

EMEA-000328-PIP02-09

Scope of the application

Active substance(s):

Vatreptacog alfa (activated)

Condition(s):

Treatment of hereditary factor VIII and 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:

Novo Nordisk A/S

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Novo Nordisk A/S submitted for agreement to the European Medicines Agency on 11 February 2010 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 23 March 2010.

Supplementary information was provided by the applicant on 27 May 2011. The applicant proposed modifications to the paediatric investigation plan.

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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,
   - to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

   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 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, 12 August 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

1.1. Condition: Treatment of hereditary factor VIII and IX deficiency

The waiver applies to:

- all subsets of the paediatric population from birth to less than 6 months of age;
- for powder and solvent for solution for injection; intravenous route;
- 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: treatment of hereditary factor VIII and IX deficiency

2.2. Indication(s) targeted by the PIP

- Treatment of bleeding episodes in patients with congenital haemophilia with inhibitors to coagulation factors VIII or IX and patients with congenital haemophilia who are expected to have a high anamnestic response to factor VIII or factor IX administration.
- Prevention and treatment of bleeding during surgical interventions or invasive procedures in patients with congenital haemophilia with inhibitors to coagulation factors VIII or IX and patients with congenital haemophilia who are expected to have a high anamnestic response to factor VIII or factor IX administration.

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

From 6 months to less than 18 years of age.

2.2.2. Pharmaceutical form(s)

Powder and solvent for solution for injection.

2.2.3. Studies

Area	Number of studies	Description
Quality	0	Not applicable.
Non-clinical	0	Not applicable.

Clinical	4	Study 1 Multi-centre, double-blinded, randomised dose escalation trial with an active control (Recombinant activated factor VII; rFVIIa), evaluating the safety and efficacy of vatreptacog alfa in 5 dose levels for treatment of acute mild-to-moderate joint bleeds in congenital haemophilia patients aged from 12 to less than 18 years (and adults) with inhibitors to human FVIII or IX conducted in the hospital setting. Study 2 Multi-centre, randomised, double-blind, active controlled (Recombinant activated factor VII; rFVIIa) trial to evaluate the efficacy and safety of vatreptacog alfa for treatment of acute bleeds of all types and severity in patients with congenital haemophilia A or B with inhibitors to human FVIII or IX aged from 12 to less than 18 years (and adults) in home and hospital setting. Study 3 Multi-centre, open-label, non-randomised, single-arm trial investigating safety and efficacy of vatreptacog alfa in treatment of acute bleeds in children with haemophilia and FVIII/IX inhibitors from 6 months to less than 12 years of age; the safety and pharmacokinetic profile of a single dose will be assessed in a non-bleeding state prior to first on-demand treatment of acute bleeds (home and hospital setting). Study 4 Multi-centre, open-label, non-randomised, single-arm trial on safety and efficacy of vatreptacog alfa administered to provide haemostatic cover for surgical interventions or invasive procedures in haemophilia A or B patients aged from 12 years to less than 18 years (and adults) with human FVIII or FIX inhibitors.

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 September 2014
Deferral for one or more studies contained in the paediatric investigation plan:	Yes