EMA/616386/2012

European Medicines Agency decision

P/0227/2012

of 3 October 2012

on the agreement of a paediatric investigation plan and on the granting of a deferral for glycopegylated recombinant coagulation factor VIII (EMEA-001174-PIP02-12) 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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on the agreement of a paediatric investigation plan and on the granting of a deferral for glycopegylated recombinant coagulation factor VIII (EMEA-001174-PIP02-12) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council.

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 10 May 2012 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 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 glycopegylated recombinant coagulation 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 glycopegylated recombinant coagulation 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 Novo Nordisk A/S, Novo Allé, 2880 – Bagsværd, Denmark.

Done at London, 3 October 2012

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

EMA/PDCO/501348/2012

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

EMEA-001174-PIP02-12

Scope of the application

Active substance(s):

Glycopegylated recombinant coagulation 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:

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 10 May 2012 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 19 June 2012.

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,

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- 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		
Clinical	4	**Study 2:** Open-label trial to evaluate pharmacokinetics, safety, efficacy of Glycopegylated recombinant coagulation factor VIII (N8-GP) in previously treated children from 12 to less than 18 years of age (and adults) with severe hereditary Factor VIII deficiency **Study 3:** Open-label, non-randomised trial to evaluate safety and the haemostatic effect of N8-GP during surgical procedures in children with haemophilia A from 12 years to less than 18 years of age (and adults) **Study 4:** Open-label trial to evaluate pharmacokinetics, safety, efficacy, pharmacokinetics of N8-GP in previously treated children less than 12 years of age with Haemophilia A **Study 5:** Open-label trial to evaluate safety, efficacy, immunogenicity of N8-GP in previously untreated children from birth to less than 12 years of age with 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 2019
Deferral for one or more studies contained in the paediatric investigation plan:	Yes