



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/683312/2010

European Medicines Agency decision

P/292/2010

of 22 December 2010

on the agreement of a paediatric investigation plan and on the granting of a deferral for 40K pegylated recombinant blood coagulation factor IX, (EMEA-000731-PIP01-09) 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.



European Medicines Agency decision

P/292/2010

of 22 December 2010

on the agreement of a paediatric investigation plan and on the granting of a deferral for 40K pegylated recombinant blood coagulation factor IX, (EMA-000731-PIP01-09) 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 726/2004¹,

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 Novo Nordisk A/S on 14 December 2009 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 12 November 2010, 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 40K pegylated recombinant blood coagulation factor IX, powder 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 40K pegylated recombinant blood coagulation factor IX, powder 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, 22 December 2010

For the European Medicines Agency
Thomas Lönngren
Executive Director

(Signature on file)

EMA/PDCO/608591/2010

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

EMA-000731-PIP01-09

Scope of the application

Active substance(s):

40K pegylated recombinant blood coagulation factor IX

Condition(s):

Treatment of hereditary factor IX deficiency

Pharmaceutical form(s):

Powder 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 14 December 2009 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 21 January 2010.

Supplementary information was provided by the applicant on 27 August 2010.

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, 12 November 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

Not applicable

2. Paediatric Investigation Plan

2.1. Condition

Treatment of Hereditary Factor IX deficiency

2.1.1. Indication(s) targeted by the PIP

Treatment and prophylaxis of bleeding in haemophilia B

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 for solution for injection, intravenous use

2.1.4. Studies

Area	Number of studies	Description
Quality	1	Development of a 250 UI vial
Non-clinical	0	Not applicable.
Clinical	5	Multi-centre, open-label study to assess PK, tolerability/safety and efficacy of on-demand and prophylactic treatment with N9-GP in adults and adolescents with haemophilia B Non-randomised, open-label, multicentre, multinational, single-arm, non-controlled, efficacy and safety trial to assess the efficacy and safety of N9-GP during surgical procedures in patients with haemophilia B Open-label, multicentre, uncontrolled, PK, safety and efficacy trial to assess the effect of N9-GP in children less than 12 years of age with haemophilia B Open-label, multicentre, uncontrolled, safety and efficacy trial in previously untreated patients with haemophilia B Open-label, multicentre, one-arm, non-controlled safety trial

3. Follow-up, completion and deferral of PIP

Measures to address long term follow-up of potential safety and efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By June 2019
Deferral for one or more studies contained in the paediatric investigation plan:	Yes