EMA/826252/2012

European Medicines Agency decision

P/0001/2013

of 11 January 2013

on the acceptance of a modification of an agreed paediatric investigation plan for catridecacog (NovoThirteen) (EMEA-000185-PIP01-08-M05) 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/90/2008 issued on 14 October 2008, the decision P/40/2010 issued on 9 April 2010, the decision P/179/2010 issued on 23 September 2010, the decision P/273/2010 issued on 3 December 2010, and the decision P/0178/2012 issued on 20 August 2012,

Having regard to the application submitted by Novo Nordisk A/S on 14 September 2012 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 7 December 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 and to the deferral.

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

Changes to the agreed paediatric investigation plan for catridecacog (NovoThirteen), powder and solvent for solution for injection, intravenous use, including changes to the deferral, 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 Allé, DK-2880 - Bagsværd, Denmark.

Done at London, 11 January 2013

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

EMA/PDCO/609578/2012

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

EMEA-000185-PIP01-08-M05

Scope of the application

Active substance(s):

Catridecacog

Invented name:

NovoThirteen

Condition(s):

Treatment of congenital factor XIII A-subunit deficiency

Authorised indication(s):

See Annex II

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

Information about the authorised medicinal product:

See Annex II

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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 14 September 2012 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/90/2008 issued on 14 October 2008, the decision P/40/2010 issued on 9 April 2010, the decision P/179/2010 issued on 23 September 2010, the decision P/273/2010 issued on 3 December 2010 and the decision P/0178/2012 issued on 20 August 2012.

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

The procedure started on 10 October 2012.

Scope of the modification

Some timelines 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 and to the deferral 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, 7 December 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

Treatment of congenital factor XIII A-subunit deficiency

1.1. Prevention of bleeding during surgical interventions in congenital factor XIII A-subunit deficiency

The waiver applies to:

- All subsets of the paediatric population from birth to less than 18 years of age,
- for powder for solution for injection, intravenous use,
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

1.2. Treatment of bleeding in congenital factor XIII A-subunit deficiency

The waiver applies to:

- All subsets of the paediatric population from birth to less than 18 years of age,
- for powder for solution for injection, intravenous use,
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

1.3. Prevention of bleeding in congenital factor XIII A-subunit deficiency

The waiver applies to:

- The paediatric population from birth to less than 1 year of age,
- for powder for solution for injection, intravenous 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 to be investigated

Treatment of congenital factor XIII A-subunit deficiency*.*

2.1.1. Indication targeted by the PIP

Prevention of bleeding in patients with congenital FXIII A-subunit deficiency.

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

From 1 year 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	Measure 1 Development of an age-appropriate diluted formulation.
Non-clinical	1	Measure 2 PK, safety study Single Dose Pharmacokinetic Study in Juvenile and Mature Cynomolgus Monkeys.
Clinical	3	Measure 3 Efficacy and safety study Multi-Centre, Open-Label, Single-Arm and Multiple Dosing, 12 month safety and efficacy study in factor XIII A-subunit deficient patients aged 6 to less than 18 years old (and adults). Measure 4 Safety study Open label safety follow-on protocol for all patients completing the Measure 3 protocol. Measure 5 PK, safety study Single dose, PK and safety study in patients from 1 to less than 6 years of age.

The date of completion of the paediatric investigation plan corresponds to the timeline for completion of the latest measure(s) reported below.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety issues in relation to paediatric use:	Yes.
Date of completion of the paediatric investigation plan:	By December 2015.
Deferral for one or more measures contained in the paediatric investigation plan:	Yes.

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

1. Treatment of congenital factor XIII A-subunit deficiency

   Authorised indications:

   - Long-term prophylactic treatment of bleeding in patients 6 years and above with congenital factor-XIII-A-subunit deficiency.

Authorised pharmaceutical formulation(s):

Powder and solvent for solution for injection

Authorised route(s) of administration:

Intravenous use