EMA/764095/2010

European Medicines Agency decision

P/273/2010

of 3 December 2010

on the acceptance of a modification of an agreed paediatric investigation plan for catridecacog (EMEA-000185-PIP01-08-M03) 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.

7 Westferry Circus • Canary Wharf • London E14 4HB • United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7418 8409
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

European Medicines Agency decision

P/273/2010

of 3 December 2010

on the acceptance of a modification of an agreed paediatric investigation plan for catridecacog (EMEA-000185-PIP01-08-M03) 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 European Medicines Agency's decision P/90/2008 issued on 14 October 2008, the decision P/40/2010 issued on 9 April 2010, and the decision P/179/2010 issued on 23 September 2010,

Having regard to the application submitted by Novo Nordisk A/S on 27 August 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 12 November 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 catridecacog, powder and solvent for solution for injection, intravenous 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 Allé, DK-2880, Bagsværd, Denmark.

Done at London, 3 December 2010

For the European Medicines Agency
Thomas Lönngren
Executive Director

(Signature on file)

EMA/PDCO/633928/2010

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

EMEA-000185-PIP01-08-M03

Scope of the application

Active substance(s):

Catridecacog

Condition(s):

Congenital factor XIII A-subunit 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 22 of Regulation (EC) No 1901/2006 as amended, Novo Nordisk A/S submitted to the European Medicines Agency on 27 August 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/90/2008 issued on 14 October 2008, the decision P/40/2010 issued on 9 April 2010 and decision P/179/2010 issued on 23 September 2010.

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

The procedure started on 15 September 2010.

Scope of the modification

Some measures and timelines of the original Opinion have been modified.

7 Westferry Circus • Canary Wharf • London E14 4HB • United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

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 member(s) agree(s) with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the paediatric investigation plan and the subsets of the paediatric population and conditions 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 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. Condition(s)

Congenital factor XIII A-subunit deficiency

2. Waiver

2.1. Indication

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).

2.2. Indication

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).

2.3. Indication

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.

3. Paediatric Investigation Plan

3.1. Condition to be investigated

Congenital factor XIII A-subunit deficiency

3.2. Indication targeted by the pip

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

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

From 1 year to less than 18 years of age

3.4. Pharmaceutical form(s)

Powder and solvent for solution for injection

3.5. 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.

4. 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 31 October 2011
Deferral for one or more studies contained in the paediatric investigation plan:	Yes