EMA/57221/2014

European Medicines Agency decision

P/0038/2014

of 5 March 2014

on the acceptance of a modification of an agreed paediatric investigation plan for trenonacog alfa (EMEA-000661-PIP01-09-M06) 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 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

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on the acceptance of a modification of an agreed paediatric investigation plan for trenonacog alfa (EMEA-000661-PIP01-09-M06) 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/131/2010 issued on 28 July 2010, the decision P/337/2010 issued on 17 December 2010, the decision P/53/2011 issued on 4 March 2011, the decision P/172/2011 issued on 1 July 2011, the decision P/231/2011 issued on 20 September 2011, and the decision P/0073/2012 issued on 24 April 2012,

Having regard to the application submitted by Cangene Europe Ltd. on 25 October 2013 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 17 January 2014, 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 trenonacog alfa, powder for solution for injection, powder and solvent for solution for infusion, 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 Cangene Europe Ltd, Parkshot House, 5 Kew Road, TW9 2PR - Richmond, Surrey, United Kingdom.

Done at London, 5 March 2014

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

EMA/PDCO/662661/2013

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

EMEA-000661-PIP01-09-M06

Scope of the application

Active substance(s):

Trenonacog alfa

Condition(s):

Treatment of hereditary factor IX deficiency (Haemophilia B)

Pharmaceutical form(s):

Powder for solution for injection

Powder and solvent for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Cangene Europe Ltd.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Cangene Europe Ltd submitted to the European Medicines Agency on 25 October 2013 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/131/2010 issued on 28 July 2010, the decision P/337/2010 issued on 17 December 2010, the decision P/53/2011 issued on 4 March 2011, the decision P/172/2011 issued on 1 July 2011, the decision P/231/2011 issued on 20 September 2011, and the decision P/0073/2012 issued on 24 April 2012.

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

The procedure started on 19 November 2013.

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Scope of the modification

Some measures and 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 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 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, 17 January 2014

On behalf of the Paediatric Committee
Dr Dirk Mentzer, 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 (Haemophilia B)

2.1.1. Indication(s) targeted by the PIP

Replacement therapy in patients with hereditary Factor IX Deficiency (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.

Powder and solvent for solution for injection.

2.1.4. Measures

Area	Number of Measures	Description
Quality	1	**Measure 1:** Development of age-appropriate size vials.
Non-clinical		Not applicable.
Clinical	3	**Measure 2:** Randomized, double-blind, multicentre cross-over active control trial to evaluate pharmacokinetics, efficacy and safety of Trenonacog alfa in previously treated children from 12 years to less than 18 years of age (and in adults) with Hereditary Factor IX Deficiency. **Measure 3:** Open-label, multicentre trial to evaluate pharmacokinetics, safety, efficacy, and immunogenicity of Trenonacog alfa in previously treated children from birth to less than 12 years of age with Hereditary Factor IX Deficiency. **Measure 4:** Open-label, multicentre trial to evaluate pharmacokinetics, safety, efficacy, and immunogenicity of Trenonacog alfa in previously untreated children from birth to less than 6 years of age with Hereditary Factor IX Deficiency.

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 November 2019
Deferral for one or more measures contained in the paediatric investigation plan:	Yes