EMA/605305/2014

European Medicines Agency decision

P/0274/2014

of 28 October 2014

on the acceptance of a modification of an agreed paediatric investigation plan for human coagulation Factor VIII / von Willebrand Factor (Voncento), (EMEA-000312-PIP01-08-M07) 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/164/2009 issued on 14 August 2009, the decision P/140/2010 issued on 30 July 2010, the decision P/107/2011 issued on 6 May 2011, the decision P/0081/2012 issued on 30 April 2012, the decision P/0154/2012 issued on 25 July 2012, the decision P/0152/2013 issued on 5 July 2013, and the decision P/0123/2014 issued on 8 May 2014,

Having regard to the application submitted by CSL Behring GmbH on 23 June 2014 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 September 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 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 human coagulation Factor VIII / von Willebrand Factor (Voncento), powder and solvent for solution for injection, powder and solvent for solution for infusion, 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 CSL Behring GmbH, Emil-von-Behring-Str.76, 35041 - Marburg, Germany.

Done at London, 28 October 2014

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

EMA/PDCO/410950/2014

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

EMEA-000312-PIP01-08-M07

Scope of the application

Active substance(s):

Human coagulation Factor VIII / von Willebrand Factor

Invented name:

Voncento

Condition(s):

Treatment of hereditary Factor VIII deficiency (Haemophilia A)

Treatment of von Willebrand disease

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Powder and solvent for solution for injection

Powder and solvent for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

CSL Behring GmbH

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, CSL Behring GmbH submitted to the European Medicines Agency on 23 June 2014 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/164/2009 issued on 14 August 2009, the decision P/140/2010 issued on 30 July 2010, the decision P/107/2011 issued on 6 May 2011, the decision P/0081/2012 issued on 30 April 2012, the decision P/0154/2012 issued on 25 July 2012, the decision P/0152/2013 issued on 5 July 2013, and the decision P/0123/2014 issued on 8 May 2014.

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

The procedure started on 16 July 2014.

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 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 annexes and appendix.

London, 12 September 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 (PIP)

1. Waiver

1.1. Condition

Treatment of hereditary Factor VIII deficiency (Haemophilia A)

The waiver applies to:

- infants from birth to less than 28 days;
- for powder and solvent for solution for injection and power and solvent for solution for infusion, intravenous use;
- on the grounds that clinical studies cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the paediatric population.

2. Paediatric Investigation Plan

2.1. Condition

Treatment of hereditary Factor VIII deficiency (Haemophilia A)

2.1.1. Indication(s) targeted by the PIP

Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital Factor VIII deficiency)

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

From 1 month to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Powder and solvent for solution for injection, intravenous use

Powder and solvent for solution for infusion, intravenous use

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable.
Non-clinical studies	0	Not applicable.
Clinical studies	2	**Study 1:** Double blind, randomised cross-over trial to evaluate pharmacokinetics, safety, efficacy, of Human Factor VIII/von Willebrand Factor in previously treated children from 12 to less than 18 years of age (and adults) with haemophilia A.

		Study 2: Open-label, trial to evaluate pharmacokinetics, safety and efficacy, of Human Factor VIII/von Willebrand Factor in previously treated children from birth to less than 12 years of age with haemophilia A. **Study 3:** (This study was deleted in procedure EMEA-000312-PIP01-08-M07)
Extrapolation modelling and simulation studies	0	Not applicable.
Other studies	0	Not applicable.
Other measures	0	Not applicable.

2.2. Condition

Treatment of von Willebrand disease

2.2.1. Indication(s) targeted by the PIP

Prophylaxis and treatment of haemorrhage or surgical bleeding in von Willebrand disease, when desmopressin (DDAVP) treatment alone is ineffective or contraindicated

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

From birth to less than 18 years of age

2.2.3. Pharmaceutical form(s)

Powder and solvent for solution for injection, intravenous use

Powder and solvent for solution for infusion, intravenous use

2.2.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable.
Non-clinical studies	0	Not applicable.
Clinical studies	2	**Study 4:** Open-label trial to evaluate pharmacokinetics, safety, efficacy, of Human Factor VIII/von Willebrand Factor in children from 12 to less than 18 years of age (and adults) with von Willebrand disease.

		Study 5: Open-label trial to evaluate pharmacokinetics, safety, efficacy, of Human Factor VIII/von Willebrand Factor in children from birth to less than 12 years of age with von Willebrand disease.
Extrapolation, modelling and simulation studies	0	Not applicable.
Other studies	0	Not applicable.
Other measures	0	Not applicable.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By July 2014
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 hereditary Factor VIII deficiency (Haemophilia A)

Authorised indication(s):

- prophylaxis and treatment of bleeding in patients with haemophilia A.

2. Treatment of von Willebrand disease

Authorised indication(s):

- treatment of haemorrhage or prevention and treatment of surgical bleeding in patients with VWD, when desmopressin (DDAVP) treatment alone is ineffective or contraindicated.

Authorised pharmaceutical form(s)

Powder and solvent for solution for injection

Powder and solvent for solution for infusion

Authorised route(s) of administration

Intravenous use