EMA/339828/2013

European Medicines Agency decision

P/0152/2013

of 5 July 2013

on the acceptance of a modification of an agreed paediatric investigation plan for human coagulation Factor VIII / von Willebrand Factor (EMEA-000312-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/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, and the decision P/0154/2012 issued on 25 July 2012,

Having regard to the application submitted by CSL Behring on 18 February 2013 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 17 May 2013, 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 Human coagulation Factor VIII / von Willebrand Factor, powder and solvent 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 CSL Behring, Emil-von-Behring-Str.76, 35041 – Marburg, Germany.

Done at London, 5 July 2013

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

EMA/PDCO/116329/2013

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

EMEA-000312-PIP01-08-M05

Scope of the application

Active substance(s):

Human coagulation Factor VIII / von Willebrand Factor

Condition(s):

Treatment of hereditary Factor VIII deficiency (Haemophilia A)

Treatment of von Willebrand disease

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

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, CSL Behring submitted to the European Medicines Agency on 18 February 2013 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, and the decision P/0154/2012 issued on 25 July 2012.

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

The procedure started on 20 March 2013.

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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 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 subsets of the paediatric population and condition 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, 17 May 2013

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

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 targeted by the PIP

- Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital Factor VIII deficiency).
- Immune tolerance induction in patients who have inhibitors to factor VIII.

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 studies	Description
Quality	0	Not applicable.
Non-clinical	0	Not applicable.
Clinical	3	Measure 1: Double blind, randomised, multicentre, 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.

Area	Number of studies	Description
		Measure 2: Open-label, multicentre, 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. Measure 3: Open-label, multicentre, trial to evaluate safety and efficacy of Human Factor VIII/von Willebrand Factor in children from 1 month to less than 12 years of age with haemophilia A who have developed high responding antibodies to FVIII.

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

2.2. Condition: treatment of von Willebrand disease

2.2.1. Indication 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. Studies

Area	Number of measures	Description
Quality	0	Not applicable.
Non-clinical	0	Not applicable.
Clinical	2	Measure 4: Open-label, multicentre 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.

Area	Number of measures	Description
		Measure 5: Open-label, multicentre 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.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By July 2017
Deferral for one or more measures contained in the paediatric investigation plan:	Yes