EMA/296250/2015

European Medicines Agency decision

P/0109/2015

of 1 June 2015

on the acceptance of a modification of an agreed paediatric investigation plan for recombinant single chain coagulation factor VIII (EMEA-001215-PIP01-11-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.

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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/0215/2012 issued on 28 September 2012, the decision P/0189/2014 issued on 6 August 2014 and the decision P/0335/2014 issued on 22 December 2014,

Having regard to the application submitted by CSL Behring GmbH on 22 January 2015 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 22 May 2015, 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, following a re-examination procedure of the Paediatric Committee's opinion according to Article 25(3) Regulation (EC) No 1901/2006, 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 recombinant single chain coagulation factor VIII, 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 CSL Behring GmbH, Emil-von-Behring Str. 76, 35041 – Marburg, Germany.

Done at London, 1 June 2015

For the European Medicines Agency
Jordi Llinares Garcia
Head of Division (ad interim)
Human Medicines Research and Development Support
(Signature on file)

EMA/PDCO/315221/2015
London, 22 May 2015

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

EMEA-001215-PIP01-11-M03

Scope of the application

Active substance(s):

Recombinant single chain coagulation factor VIII

Condition(s):

Treatment of congenital factor VIII deficiency

Pharmaceutical form(s):

Powder and solvent for solution for injection

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

CSL Behring GmbH

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 22 January 2015 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0215/2012 issued on 28 September 2012, the decision P/0189/2014 issued on 6 August 2014, and the decision P/0335/2014 issued on 22 December 2014.

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

An Opinion was adopted by the Paediatric Committee on 17 April 2015 for the above mentioned product. CSL Behring GmbH received the Paediatric Committee Opinion on 28 April 2015.

On 8 May 2015 CSL Behring GmbH submitted to the European Medicines Agency a written request including detailed grounds for a re-examination of the Opinion.

The re-examination procedure started on 9 May 2015.

Scope of the modification

The identifier of study 3 has been modified.

Final opinion

1. The Paediatric Committee, having assessed the detailed grounds for re-examination, in accordance with Article 25(3) of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

1.1. to revise its opinion and

- to agree to the changes regarding the measures of the paediatric investigation plan in the scope set out in the Annex I of this opinion.

The Norwegian Paediatric Committee member agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed 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.

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

Not applicable.

2. Paediatric Investigation Plan

2.1. Condition

Treatment of congenital factor VIII Deficiency

2.1.1. Indication(s) targeted by the PIP

Treatment and prophylaxis of bleeding in patients with congenital factor VIII deficiency

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 and solvent for solution for injection

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable.
Non-clinical studies	0	Not applicable.
Clinical studies	3	**Study 1** Open-label, pharmacokinetic, safety, efficacy study for on-demand and prophylaxis treatment of haemophilia including a surgical sub-study in previously treated patients with severe haemophilia A from 12 to less than 18 years of age and adults (CSL627_1001). **Study 2** Open-label, pharmacokinetic, safety and efficacy study for on-demand and prophylaxis treatment of haemophilia in previously treated patients with severe haemophilia A below 12 years of age (CSL627_3002). **Study 3** Open-label, safety and efficacy study for on-demand and prophylaxis treatment of haemophilia in previously untreated patients (PUPs) from birth to less than 18 years of age and adults with haemophilia A (CSL627_3004).

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 and efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By May 2018
Deferral for one or more studies contained in the paediatric investigation plan:	Yes