EMA/591163/2014

European Medicines Agency decision

P/0269/2014

of 27 October 2014

on the acceptance of a modification of an agreed paediatric investigation plan for recombinant fusion protein linking human coagulation factor IX with human albumin (EMEA-001107-PIP01-10-M02) 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.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Contact EMA www.ema.europa.eu/contact

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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/251/2011 issued on 25 October 2011 and the decision P/0138/2014 issued on 11 June 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,

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.

(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 fusion protein linking human coagulation factor IX with human albumin, powder and solvent for solution for injection/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 GmbH, Emil-von-Behring Str. 76, 35041 – Marburg, Germany.

Done at London, 27 October 2014

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

EMA/PDCO/417054/2014 Corr

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

EMEA-001107-PIP01-10-M02

Scope of the application

Active substance(s):

Recombinant fusion protein linking human coagulation factor IX with human albumin

Condition(s):

Treatment of hereditary factor IX deficiency

Pharmaceutical form(s):

Powder and solvent for solution for injection/infusion

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 23 June 2014 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/251/2011 issued on 25 October 2011 and the decision P/0138/2014 issued on 11 June 2014.

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

The procedure started on 16 July 2014.

Scope of the modification

Some measures of the Paediatric Investigation Plan have been modified.

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Send a question via our website www.ema.europa.eu/contact

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 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 annex 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

Not applicable.

2. Paediatric Investigation Plan

2.1. Condition

Treatment of hereditary factor IX deficiency

2.1.1. Indication(s) targeted by the PIP

Treatment and prophylaxis of bleeding in patients with Haemophilia B (hereditary factor IX 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/infusion.

2.1.4. Studies

Area	Number of studies	Description
Quality	1	**Study 1** Development of a 250 IU/vial formulation (powder and solvent for solution for injection/infusion)
Non-clinical	0	Not applicable.
Clinical	5	**Study 2** Open-label, multicentre, uncontrolled, dose-escalation study to evaluate the safety and pharmacokinetics of recombinant fusion protein linking human coagulation factor IX with human albumin (rIX-FP) in previously treated patients, 12 years of age or above, with Haemophilia B **Study 3** Open-label, multicentre, uncontrolled study to evaluate the safety, pharmacokinetics and clinical response of rIX-FP with regard to the prevention of bleeding in previously treated patients, 12 years of age or above, with Haemophilia B **Study 4** Open-label, multicentre, uncontrolled study to evaluate the clinical response, pharmacokinetics and safety of rIX-FP with regard to the prevention and

		treatment of bleeding in previously treated patients, 12 years of age or above, with Haemophilia B **Study 5** Open-label, multicentre, uncontrolled study to evaluate the safety, pharmacokinetics and clinical response of rIX-FP with regard to the prevention and treatment of bleeding in previously treated patients less than 12 years of age with Haemophilia B **Study 6** Open-label, multicentre, uncontrolled study to evaluate the safety, pharmacokinetics and clinical response of rIX-FP with regard to the prevention and treatment of bleeding in previously untreated patients (PUPs) with Haemophilia B

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 June 2017
Deferral for one or more studies contained in the paediatric investigation plan:	Yes