EMA/693940/2011

European Medicines Agency decision

P/251/2011

of 25 October 2011

on the agreement of a paediatric investigation plan and on the granting of a deferral for recombinant fusion protein linking human coagulation factor IX with human albumin (EMEA-001107-PIP01-10) 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 application submitted by CSL Behring GmbH on 17 January 2011 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 9 September 2011, in accordance with Article 18 of Regulation (EC) No 1901/2006, and Article 21 of said Regulation,

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 agreement of a paediatric investigation plan and on the granting of a deferral.

(2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.

(3) It is therefore appropriate to adopt a decision granting a 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

A 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, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

Article 2

A deferral for recombinant fusion protein linking human coagulation factor IX with human albumin, powder and solvent for solution for injection/infusion, intravenous use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

This decision is addressed to CSL Behring GmbH, Emil-von-Behring Str. 76, 35041 Marburg, Germany.

Done at London, 25 October 2011

For the European Medicines Agency
Andreas Pott
Acting Executive Director
(Signature on file)

EMA/PDCO/498154/2011

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a deferral

EMEA-001107-PIP01-10

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 16(1) of Regulation (EC) No 1901/2006 as amended, CSL Behring GmbH submitted for agreement to the European Medicines Agency on 17 January 2011 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation.

The procedure started on 23 February 2011.

Supplementary information was provided by the applicant on 20 June 2011. The applicant proposed modifications to the paediatric investigation plan.

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Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree the paediatric investigation plan in accordance with Article 18 of said Regulation,
   - to grant a deferral in accordance with Article 21 of said Regulation.

   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 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(es) and appendix.

London, 09 September 2011

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

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,

Area	Number of studies	Description
		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 December 2015
Deferral for one or more studies contained in the paediatric investigation plan:	Yes