EMA/758347/2016

European Medicines Agency decision

P/0343/2016

of 2 December 2016

on the agreement of a paediatric investigation plan and on the granting of a deferral for recombinant Fusion Protein Linking Coagulation Factor VIIa with Albumin (rVIIa-FP; CSL689) (EMEA-001886-PIP02-15) 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 15 October 2015 under Article 16(1) of Regulation (EC) No 1901/2006,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 14 October 2016, in accordance with Article 18 of Regulation (EC) No 1901/2006 and of its own motion in accordance with 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 Coagulation Factor VIIa with Albumin (rVIIa-FP; CSL689), powder and solvent for solution for injection, 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 Coagulation Factor VIIa with Albumin (rVIIa-FP; CSL689), powder and solvent for solution for injection, 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.

EMA/PDCO/535196/2016
London, 14 October 2016

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

EMEA-001886-PIP02-15

Scope of the application

Active substance(s):

Recombinant Fusion Protein Linking Coagulation Factor VIIa with Albumin (rVIIa-FP; CSL689)

Condition(s):

Treatment of congenital Factor VII 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 16(1) of Regulation (EC) No 1901/2006 as amended, CSL Behring GmbH submitted for agreement to the European Medicines Agency on 15 October 2015 an application for a paediatric investigation plan for the above mentioned medicinal product.

The procedure started on 30 November 2015.

Supplementary information was provided by the applicant on 18 July 2016. 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 on its own motion in accordance with Article 21 of said Regulation.

   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 VII Deficiency

2.1.1. Indication(s) targeted by the PIP

Treatment of congenital Factor VII 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	1	**Study 1** Multicentre, open-label, parallel–arm, multiple-dose study to investigate pharmacokinetics, efficacy and safety of recombinant fusion protein linking coagulation factor VIIa with albumin (CSL689) in paediatric and adult patients with congenital Factor VII deficiency to evaluate the clinical efficacy of CSL689 with respect to on-demand and prophylactic treatment.
Extrapolation, modelling and simulation studies	4	**Study 2** Population PK model to describe the PK of CSL689 in healthy adults. **Study 3** Population PK model to describe the PK of CSL689 in FVII deficient adults.

Area	Number of measures	Description
		Study 4 Population PK model 1 to describe the PK of CSL689 in FVII deficient children less than 12 years of age. Population PK model 2 to describe the PK of CSL689 in FVII deficient adults and children. **Study 5** Extrapolation of exposure in children less than 12 years of age based on adult PopPK model, published data on Eptacog alfa (activated)(rFVIIa) and data reported for a fusion protein product from internal CSLB experience (rIX-FP/ CSL654).
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 December 2020
Deferral for one or more measures contained in the paediatric investigation plan:	Yes