EMA/461645/2017

European Medicines Agency decision

P/0214/2017

of 9 August 2017

on the acceptance of a modification of an agreed paediatric investigation plan for coagulation Factor VIIa (Recombinant) (EMEA-001203-PIP02-14-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.

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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/0042/2015 issued on 6 March 2015 and the decision P/0103/2016 issued on 4 May 2016,

Having regard to the application submitted by LFB SA on 3 April 2017 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 23 June 2017, 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 coagulation Factor VIIa (Recombinant), 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 LFB SA, 3, Avenue des Tropiques - BP 40305, 91958 - Courtaboeuf Cedex, France.

EMA/PDCO/234337/2017
London, 23 June 2017

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

EMEA-001203-PIP02-14-M02

Scope of the application

Active substance(s):

Coagulation Factor VIIa (Recombinant)

Condition(s):

Treatment of congenital coagulation disorders

Treatment of acquired haemophilia

Pharmaceutical form(s):

Powder and solvent for solution for injection

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

LFB SA

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, LFB SA submitted to the European Medicines Agency on 3 April 2017 an application for modification of the agreed paediatric investigation plan with a waiver as set out in the European Medicines Agency's decision P/0042/2015 issued on 6 March 2015 and the decision P/0103/2016 issued on 4 May 2016.

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

The procedure started on 25 April 2017.

Scope of the modification

Some measures and 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 Norwegian Paediatric Committee member agrees 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.

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

1.1. Condition:

Treatment of congenital coagulation disorders

The waiver applies to:

- the paediatric population from birth to less than 6 months;
- powder and solvent for solution for injection, intravenous use;
- on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

1.2. Condition

Treatment of acquired haemophilia

The waiver applies to:

- all subsets of the paediatric population from birth to less than 18 years of age;
- powder and solvent for solution for injection, intravenous use;
- on the grounds that the disease or condition for which the specific medicinal product is intended occurs only in adult populations.

2. Paediatric investigation plan

2.1. Condition

Treatment of congenital coagulation disorders

2.1.1. Indication(s) targeted by the PIP

Treatment of bleeding and prevention of bleeding in those undergoing surgery or invasive procedures in patients with haemophilia A or B with inhibitors to Factors VIII or IX.

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From 6 months 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 studies	Description
Quality-related studies	0	Not applicable.

Non-clinical studies	0	Not applicable.
Clinical studies	3	**Study 1** Open-label, randomised, external controlled, cross-over trial to evaluate pharmacokinetics, efficacy and safety of coagulation factor VIIa (recombinant) in children from 12 years to less than 18 years of age with congenital haemophilia A or B with inhibitors to factor VIII or IX. (RB-FVIIa-006-13). **Study 2** Open-label, randomised, external controlled, cross-over trial to evaluate pharmacokinetics, efficacy and safety of coagulation factor VIIa (recombinant) in children from 6 months to less than 12 years of age with congenital haemophilia A or B with inhibitors to factor VIII or IX. (LFB-FVIIa-007-14). **Study 3** Open-label, non-comparative, uncontrolled trial to evaluate safety and efficacy of coagulation factor VIIa (recombinant) in children from 6 months to 18 years of age with congenital haemophilia A or B with inhibitors to factor VIII or IX undergoing surgery or an invasive procedure. (LFB-FVIIa-008-14).
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/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By December 2017.
Deferral for one or more measures contained in the paediatric investigation plan:	No