



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/203384/2016

European Medicines Agency decision

P/0103/2016

of 4 May 2016

on the acceptance of a modification of an agreed paediatric investigation plan for coagulation Factor VIIa (Recombinant) (EMEA-001203-PIP02-14-M01) 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 726/2004¹,

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 Agency²,

Having regard to the European Medicines Agency's decision P/0042/2015 issued on 6 March 2015,

Having regard to the application submitted by LFB SA on 4 December 2015 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 26 February 2016, 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, following a re-examination procedure of the Paediatric Committee's opinion according to Article 25(3) Regulation (EC) No 1901/2006, 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.

Done at London, 4 May 2016

For the European Medicines Agency
Zaïde Frias
Head of Division
Human Medicines Research and Development Support
(Signature on file)



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/PDCO/254983/2016

London, 29 April 2016

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

EMA-001203-PIP02-14-M01

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 4 December 2015 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.

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

An Opinion was adopted by the Paediatric Committee on 26 February 2016 for the above mentioned product. LFB SA received the Paediatric Committee Opinion on 8 March 2016.



On 7 April 2016 LFB SA 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 8 April 2016.

Scope of the modification

Some measures and timelines of the Paediatric Investigation Plan have 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 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 May 2017
Deferral for one or more measures contained in the paediatric investigation plan:	No