EMA/49380/2015

# European Medicines Agency decision

P/0042/2015

of 6 March 2015

on the agreement of a paediatric investigation plan and on the granting of a waiver for coagulation factor VIIa (recombinant) (EMEA-001203-PIP02-14) 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 application submitted by LFB SA on 3 July 2014 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 16 January 2015, in accordance with Article 18 of Regulation (EC) No 1901/2006, and Article 13 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 waiver.

(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 waiver.

[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 coagulation factor VIIa (recombinant), 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 waiver for coagulation factor VIIa (recombinant), 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 LFB SA, 3, avenue des Tropiques - BP 40305, 91958 - Courtaboeuf Cedex, France.

Done at London, 6 March 2015

For the European Medicines Agency
Jordi Llinares Garcia
Head of Division (ad interim)
Human Medicines Research and Development Support
(Signature on file)

EMA/PDCO/672694/2014

# Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a waiver

EMEA-001203-PIP02-14

## 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 16(1) of Regulation (EC) No 1901/2006 as amended, LFB SA submitted for agreement to the European Medicines Agency on 3 July 2014 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 13 August 2014.

Supplementary information was provided by the applicant on 27 October 2014. The applicant proposed modifications to the paediatric investigation plan and withdrew its request for a deferral.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
**Telephone** +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5510
**Send a question via our website** www.ema.europa.eu/contact

## 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 waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(b) of said Regulation, on the grounds that the disease or condition for which the specific medicinal product is intended occurs only in adult populations, and with Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

   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 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, 16 January 2015

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

## *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**<br><br>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).<br><br>**Study 2**<br><br>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. (RB-FVIIa-007-13).<br><br>**Study 3**<br><br>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. (RB-FVIIa-008-13). |
| 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 2016 |
| Deferral for one or more measures contained in the paediatric investigation plan: | No |