

EMA/10879/2014

European Medicines Agency decision

P/0017/2014

of 22 January 2014

on the acceptance of a modification of an agreed paediatric investigation plan for human coagulation factor X (EMEA-000971-PIP01-10-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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on the acceptance of a modification of an agreed paediatric investigation plan for human coagulation factor X (EMA-000971-PIP01-10-M01) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

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/57/2011 issued on 4 March 2011,

Having regard to the application submitted by Bio Products Laboratory on 16 September 2013 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 6 December 2013, 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 and to the deferral and on the refusal of a waiver.
- (2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan, including changes to the deferral.
- (3) It is therefore appropriate to adopt a decision on the refusal of 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

Changes to the agreed paediatric investigation plan for human coagulation factor X, powder for solution for injection, intravenous use, including changes to the deferral, 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

A waiver for human coagulation factor X, powder for solution for injection, intravenous use, the details of which are set out in the opinion of the Paediatric Committee the European Medicines Agency annexed hereto, together with its appendices, is hereby refused.

Article 3

This decision is addressed to Bio Products Laboratory Ltd, Dagger Lane, WD6 3BX - Elstree, United Kingdom.

Done at London, 22 January 2014

For the European Medicines Agency
Guido Rasi
Executive Director
(Signature on file)

EMA/PDCO/577540/2013

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA-000971-PIP01-10-M01

Scope of the application

Active substance(s):

Human coagulation factor X

Condition(s):

Treatment of hereditary factor X deficiency

Pharmaceutical form(s):

Powder for solution for injection

Route(s) of administration:

Intravenous use

Name / corporate name of the PIP applicant:

Bio Products Laboratory

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Bio Products Laboratory submitted to the European Medicines Agency on 16 September 2013 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/57/2011 issued on 4 March 2011.

The application for modification proposed changes to the agreed paediatric investigation plan and to the deferral and proposed a waiver.

The procedure started on 10 October 2013.

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 and to the deferral in the scope set out in the Annex I of this opinion;
 - to refuse the granting of a waiver for some of the subsets of the paediatric population and the above mentioned condition as it does not meet the grounds detailed in Article 11(1) 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 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.

London, 6 December 2013

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

1. Waiver

1.1. Condition: Treatment of hereditary factor X deficiency

The request for the waiver applied to:

- The paediatric population from birth to less than 12 years;
- for powder for solution for injection, intravenous use.

The waiver request does not provide evidence to support the following grounds set out in Article 11(1) of Regulation (EC) No 1901/2006 that:

- (c) the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

Because:

- measures would be justified by the expected therapeutic benefit and clinical trials may be feasible.

The waiver request is therefore refused by the PDCO.

2. Paediatric Investigation Plan

2.1. Condition: Treatment of hereditary factor X deficiency

2.1.1. Indication(s) targeted by the PIP

Treatment of hereditary factor X 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 for solution for injection

2.1.4. Studies

Area	Number of studies	Description
Quality	0	Not applicable.
Non-clinical	0	Not applicable.
Clinical	3	Study 1 Open-label, multicentre, non-controlled trial to evaluate the pharmacokinetics, efficacy and safety of Human coagulation factor X in the treatment of bleeding episodes over at least 6 months, in children from 12 to less than 18 years of age (and in adults) with moderate to severe hereditary

Area	Number of studies	Description
		factor X deficiency. Study 2 Open-label, multicentre, non-controlled trial to evaluate the efficacy, safety and pharmacokinetics of Human coagulation factor X in the prevention of bleeding when given as routine prophylaxis over 12 months, in children from birth to less than 12 years of age with severe hereditary factor X deficiency. Study 3 Open-label, multicentre, non-controlled trial to evaluate safety and efficacy of Human coagulation factor X administered by bolus infusion to prevent bleeding and achieve haemostasis in factor X deficient subjects undergoing surgery, in children from 12 to less than 18 years of age (and in adults) with mild to severe hereditary factor X deficiency.

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 2016
Deferral for one or more studies contained in the paediatric investigation plan:	Yes