



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/713540/2014

European Medicines Agency decision

P/0335/2014

of 22 December 2014

on the acceptance of a modification of an agreed paediatric investigation plan for recombinant single chain coagulation factor VIII (EMEA-001215-PIP01-11-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.



European Medicines Agency decision

P/0335/2014

of 22 December 2014

on the acceptance of a modification of an agreed paediatric investigation plan for recombinant single chain coagulation factor VIII (EMA-001215-PIP01-11-M02) 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/0215/2012 issued on 28 September 2012, and the decision P/0189/2014 issued on 6 August 2014,

Having regard to the application submitted by CSL Behring GmbH on 15 August 2014 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 14 November 2014, 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 recombinant single chain coagulation factor VIII, 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 CSL Behring GmbH, Emil-von-Behring Str. 76, 35041 - Marburg, Germany.

Done at London, 22 December 2014

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



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/PDCO/505716/2014

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

EMA-001215-PIP01-11-M02

Scope of the application

Active substance(s):

Recombinant single chain coagulation factor VIII

Condition(s):

Treatment of congenital factor VIII 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 22 of Regulation (EC) No 1901/2006 as amended, CSL Behring GmbH submitted to the European Medicines Agency on 15 August 2014 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0215/2012 issued on 28 September 2012, and the decision P/0189/2014 issued on 6 August 2014.

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

The procedure started on 16 September 2014.

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 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, 14 November 2014

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

Not applicable.

2. Paediatric Investigation Plan

2.1. Condition

Treatment of congenital factor VIII Deficiency

2.1.1. Indication(s) targeted by the PIP

Treatment and prophylaxis of bleeding in patients with congenital factor VIII 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. Studies

Area	Number of studies	Description
Quality-related studies	0	Not applicable.
Non-clinical studies	0	Not applicable.
Clinical studies	3	Study 1 Open-label, pharmacokinetic, safety, efficacy study for on-demand and prophylaxis treatment of haemophilia including a surgical sub-study in previously treated patients with severe haemophilia A from 12 to less than 18 years of age and adults (CSL627_1001). Study 2 Open-label, pharmacokinetic, safety and efficacy study for on-demand and prophylaxis treatment of haemophilia in previously treated patients with severe haemophilia A below 12 years of age (CSL627_3002). Study 3 Open-label, safety and efficacy study for on-demand and prophylaxis treatment of haemophilia in previously untreated patients (PUPs) from birth to less than 18 years of age and adults with haemophilia A (CSL627_3004).

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 and efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By May 2018
Deferral for one or more studies contained in the paediatric investigation plan:	Yes