



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/422220/2011

European Medicines Agency decision P/123/2011

of 7 June 2011

on the agreement of a paediatric investigation plan and on the granting of a deferral for recombinant fusion protein consisting of human coagulation factor IX attached to the Fc domain of human IgG1 (rFIXFc) (EMEA-000914-PIP01-10) 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 application submitted by Biogen Idec Ltd. on 7 June 2010 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 20 April 2011, in accordance with Article 18 of Regulation (EC) No 1901/2006, and Article 21 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 deferral.
- (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 deferral.

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 recombinant fusion protein consisting of human coagulation factor IX attached to the Fc domain of human IgG1 (rFIXFc), 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 deferral for recombinant fusion protein consisting of human coagulation factor IX attached to the Fc domain of human IgG1 (rFIXFc), 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 Biogen Idec Ltd., Innovation House, 70 Norden Road, SL6 4AY, Maidenhead, United Kingdom.

Done at London, 7 June 2011

For the European Medicines Agency
Andreas Pott
Acting Executive Director
(Signature on file)



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EMA/PDCO/176679/2011

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a deferral

EMA-000914-PIP01-10

Scope of the application

Active substance(s):

Recombinant fusion protein consisting of human coagulation factor IX attached to the Fc domain of human IgG1 (rFIXFc)

Condition(s):

Treatment of hereditary factor IX deficiency

Pharmaceutical form(s):

Powder and solvent for solution for injection

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Biogen Idec Ltd.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Biogen Idec Ltd. submitted for agreement to the European Medicines Agency on 7 June 2010 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 15 July 2010.

Supplementary information was provided by the applicant on 7 February 2011. The applicant proposed modifications to the paediatric investigation plan and withdrew its request for a waiver.



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 deferral in accordance with Article 21 of said Regulation.

The Norwegian Paediatric Committee member agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed 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, 20 April 2011

On behalf of the Paediatric Committee
Dr Daniel Brasseur, 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

Not applicable.

2. Paediatric Investigation Plan

2.1. Condition: Treatment of hereditary factor IX deficiency

2.1.1. Indication(s) targeted by the PIP

Treatment and prophylaxis of bleeding in patients with Haemophilia B (hereditary factor IX 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	1	Study 1: Development of a 250 IU/vial formulation (powder and solvent for solution for injection).
Non-clinical	0	Not applicable.
Clinical	3	- Study 2: Open-label, multicentre, uncontrolled study to evaluate the safety, pharmacokinetics, and efficacy of recombinant human coagulation factor IX attached to the Fc domain of human IgG1 (rFIXFc) in the prevention and treatment of bleeding in previously treated subjects, 12 years of age or above, with Haemophilia B. - Study 3: Open-label, multicenter, uncontrolled trial to evaluate the safety, pharmacokinetics, and efficacy of rFIXFc in the prevention and treatment of bleeding in previously treated patients less than 12 years of age with Haemophilia B. - Study 4: Open-label, multicenter, uncontrolled trial to evaluate the safety and efficacy of rFIXFc in the prevention and treatment of bleeding in previously untreated patients (PUPs) with Haemophilia B.

3. Follow-up, completion and deferral of PIP

Measures to address long term follow-up of potential safety and efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By June 2019
Deferral for one or more studies contained in the paediatric investigation plan:	Yes