EMA/483690/2013

European Medicines Agency decision

P/0198/2013

of 2 September 2013

on the acceptance of a modification of an agreed paediatric investigation plan for recombinant fusion protein consisting of human coagulation factor IX attached to the Fc domain of human IgG1 (rFIXFc), (EMEA-000914-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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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 European Medicines Agency's decision P/123/2011 issued on 7 June 2011,

Having regard to the application submitted by Biogen Idec Ltd on 29 April 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 19 July 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.

(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 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, 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 Biogen Idec Ltd, Innovation House, 70 Norden Road, SL6 4AY – Maidenhead, United Kingdom.

Done at London, 2 September 2013

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

EMA/PDCO/273283/2013

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

EMEA-000914-PIP01-10-M01

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 22 of Regulation (EC) No 1901/2006 as amended, Biogen Idec Ltd submitted to the European Medicines Agency on 29 April 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/123/2011 issued on 7 June 2011.

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

The procedure started on 22 May 2013.

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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 Norwegian Paediatric Committee member agrees 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, 19 July 2013

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, multicentre, 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, multicentre, 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