EMA/475515/2010

European Medicines Agency decision

P/130/2010

of 28 July 2010

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for synthetic erythropoietin (EPO) receptor agonist (EMEA-000646-PIP01-09) 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.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7418 8670
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

European Medicines Agency decision

P/130/2010

of 28 July 2010

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for synthetic erythropoietin (EPO) receptor agonist (EMEA-000646-PIP01-09) 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 726/2004[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 Takeda Global Research & Development Centre (Europe) Ltd on 17 July 2009 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 11 June 2010, in accordance with Article 18 of Regulation (EC) No 1901/2006, and Article 21 of said Regulation 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 deferral 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 deferral.

(4) 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 synthetic erythropoietin (EPO) receptor agonist), solution for injection, intravenous use, subcutaneous 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 synthetic erythropoietin (EPO) receptor agonist), solution for injection, intravenous use, subcutaneous 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

A waiver for synthetic erythropoietin (EPO) receptor agonist), solution for injection, intravenous use, subcutaneous 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 4

This decision is addressed to Takeda Global Research & Development Centre (Europe) Ltd, 61 Aldwych, WC2B 4AE – London, United Kingdom.

Done at London, 28 July 2010

For the European Medicines Agency
Thomas Lönngren
Executive Director
(Signature on file)

EMA/475515/2010

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

EMEA-000646-PIP01-09

Scope of the application

Active substance(s):

Synthetic erythropoietin (EPO) receptor agonist

Condition(s):

Symptomatic anaemia associated with chronic kidney disease

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Intravenous use

Subcutaneous use

Name/corporate name of the PIP applicant:

Takeda Global Research & Development Centre (Europe) Ltd

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Takeda Global Research & Development Centre (Europe) Ltd submitted for agreement to the European Medicines Agency on 17 July 2009 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 20 August 2009.

Supplementary information was provided by the applicant on 1 April 2010.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom

Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

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,
   - 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)(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 Norwegian Paediatric Committee member agrees with the above-mentioned recommendation of the Paediatric Committee.

   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(es) and appendix.

London, 11 June 2010

On behalf of the Paediatric Committee
Dr Daniel Brasseur, Chairman

(Signature on file)

Annex I

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

1. Condition(s)

Symptomatic anaemia associated with chronic kidney disease

2. Waiver

2.1. Condition

Symptomatic anaemia associated with chronic kidney disease

The waiver applies to:

- The paediatric population from birth to less than 1 year of age;
- for solution for injection, intravenous route, subcutaneous route;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

3. Paediatric Investigation Plan

3.1. Condition to be investigated

Symptomatic anaemia associated with chronic kidney disease.

3.1.1. Indication targeted by the PIP

Treatment of symptomatic anaemia associated with chronic kidney disease.

3.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From 1 to less than 18 years of age.

3.1.3. Pharmaceutical form(s)

Solution for injection

3.1.4. Studies

Area	Number of studies	Description
Quality	0	Not applicable.
Non-clinical	0	Not applicable.
Clinical	6	*Study 1* Open-label, single-arm, multicentre, multiple-dose uncontrolled study to evaluate safety and pharmacokinetics (PK) of AF37702 injection for maintenance treatment of anaemia in children from 1 to less than 18 years with chronic kidney disease on haemodialysis and already receiving Erythropoietin Stimulating Agent (ESA) therapy.

Area	Number of studies	Description
		Study 2 Open-label, single arm follow-up study to evaluate the safety and tolerability of AF37702 injection for the maintenance treatment of anaemia in children with chronic kidney disease. Subjects will be those who complete the titration/evaluation periods of study 1. *Study 3* Open-label, single-arm, dose titration study to evaluate safety and pharmacokinetics of AF37702 injection for correction of anemia in children from 1 to less than 18 years with chronic kidney disease who have not received previous treatment with Erythropoietin Stimulating Agent (ESA-naïve cases), and may or may not be undergoing dialysis. *Study 4* Open-label, single arm follow-up study to evaluate the safety and tolerability of AF37702 injection for the treatment of anaemia in children with chronic kidney disease. Subjects will be those who complete the correction/titration and evaluation periods of study 3. *Study 5* Open-label, randomised, multicentre, active controlled, parallel group study to evaluate the efficacy and safety of AF37702 injection for the treatment of anaemia in children from 1 to less than 18 years with chronic kidney disease all currently receiving Erythropoietin Stimulating Agent (ESA) treatment, and either undergoing dialysis or not on dialysis. *Study 6* Open-label, single arm follow-up study to evaluate the safety and tolerability of AF37702 injection for the treatment of anaemia in children with chronic kidney disease. Subjects will be those who complete the titration/evaluation periods of study 5.

4. Follow-up, completion and deferral of PIP

Measures to address long term follow-up of potential safety issues and efficacy in relation to paediatric use:	**No**
Date of completion of the paediatric investigation plan:	By October 2024.
Deferral for one or more studies contained in the paediatric investigation plan:	Yes