EMA/558114/2010

European Medicines Agency decision

P/173/2010

of 17 September 2010

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for romiplostim (Nplate), (EMEA-000653-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.

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on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for romiplostim (Nplate), (EMEA-000653-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 Amgen Europe B.V on 14 August 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 06 August 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 romiplostim (Nplate), powder for solution for injection, powder and solvent for solution for injection, 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 romiplostim (Nplate), powder for solution for injection, powder and solvent for solution for injection, 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 romiplostim (Nplate), powder for solution for injection, powder and solvent for solution for injection, 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 Amgen Europe B.V, Minervum 7061, 4817-ZK Breda, The Netherlands.

Done at London, 17 September 2010

For the European Medicines Agency
Thomas Lönngren
Executive Director

(Signature on file)

EMA/PDCO/473277/2010

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

EMEA-000653-PIP01-09

Scope of the application

Active substance:

Romiplostim

Invented name:

Nplate

Condition(s):

Treatment of immune thrombocytopenia (idiopathic thrombocytopenic purpura)

Treatment of disease-related thrombocytopenia in myelodysplastic syndrome

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Powder for solution for injection

Powder and solvent for solution for injection

Route(s) of administration:

Subcutaneous use

Name/corporate name of the PIP applicant:

Amgen Europe B.V

Information about the authorised medicinal product: see Annex II

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Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Amgen Europe B.V submitted for agreement to the European Medicines Agency on 14 August 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 17 September 2009.

Supplementary information was provided by the applicant on 28 May 2010.

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 Articles 11(1)(b) of said Regulation, on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified subset(s) of the paediatric population, and 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.

2. 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 annexes and appendix.

London, 6 August 2010

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

1.1. Condition treatment of immune thrombocytopenia (idiopathic thrombocytopenic purpura)

The waiver applies to:

- Children from birth to less than 1 year of age
- for powder for solution for injection for subcutaneous use and powder and solvent for solution for injection for subcutaneous use
- on the grounds that clinical studies cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the paediatric population.

1.2. Condition treatment of disease-related thrombocytopenia in myelodysplastic syndrome

The waiver applies to:

- Preterm and term newborn infants
- for powder for solution for injection for subcutaneous use and powder and solvent for solution for injection for subcutaneous use
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

2. Paediatric Investigation Plan

2.1. Condition treatment of immune thrombocytopenia (idiopathic thrombocytopenic purpura)

2.1.1. Indication(s) targeted by the PIP

Treatment of chronic immune thrombocytopenia (idiopathic thrombocytopenic purpura; ITP) in paediatric patients who are refractory or intolerant to other treatments (e.g., glucocorticosteroids, immunoglobulins, splenectomy)

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

From 1 year to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

- Powder for solution for injection for subcutaneous use
- Powder and solvent for solution for injection for subcutaneous use

2.1.4. Studies

Area	Number of studies	Description
Quality	1	Study 1: Development of an age-appropriate strength
Non-clinical	0	Not applicable
Clinical	2	Study 2: Double-blind, randomised, placebo-controlled, multi-centre trial to evaluate safety and efficacy of romiplostim in children from 1 year to less than 18 years of age with immune thrombocytopenia refractory to a prior therapy, relapsed after at least one prior ITP therapy or ineligible for other therapies Study 3: Open-label extension trial to evaluate safety and efficacy of romiplostim in children from 1 year to less than 18 years of age with immune thrombocytopenia

2.2. Condition treatment of disease-related thrombocytopenia in myelodysplastic syndrome

2.2.1. Indication(s) targeted by the PIP

Treatment of thrombocytopenia resulting from myelodysplastic syndrome (disease-related) in children

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

From 28 days to less than 18 years of age.

2.2.3. Pharmaceutical form(s)

- Powder for solution for injection for subcutaneous use
- Powder and solvent for solution for injection for subcutaneous use

2.2.4. Studies

Area	Number of studies	Description
Quality	1	Study 1: As per condition treatment of immune thrombocytopenia (idiopathic thrombocytopenic purpura)
Non-clinical	0	Not applicable
Clinical	2	Study 4: Double-blind, randomised, placebo-controlled, multi-centre trial to evaluate safety and efficacy of romiplostim in children from 28 days to less than 18 years of age with thrombocytopenia secondary to myelodysplastic syndrome Study 5: Open-label extension trial to evaluate safety and efficacy of romiplostim in children from 28 days to less than 18 years of age with thrombocytopenia secondary to myelodysplastic syndrome

3. Follow-up, completion and deferral of PIP

Measures to address long term follow-up of potential safety issues in relation to paediatric use:	**Yes**
Date of completion of the paediatric investigation plan:	By September 2015
Deferral for one or more studies contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

1) Treatment of immune thrombocytopenia (idiopathic thrombocytopenic purpura)

Authorised indications:

- Nplate is indicated for adult chronic immune (idiopathic) thrombocytopenic purpura (ITP) splenectomised patients who are refractory to other treatments (e.g. corticosteroids, immunoglobulins)
- Nplate may be considered as second line treatment for adult non-splenectomised patients where surgery is contra-indicated

EU Number	Invented name	Strength	Pharmaceutical form	Route of administration	Packaging	Content (concentration)	Package size
EU/1/08/497/001	Nplate	250 µg	Powder for solution for injection	Subcutaneous use	vial (glass)		1 vial
EU/1/08/497/002	Nplate	500 µg	Powder for solution for injection	Subcutaneous use	vial (glass)		1 vial
EU/1/08/497/003	Nplate	250 µg	Powder for solution for injection	Subcutaneous use	vial (glass)		4 vials
EU/1/08/497/004	Nplate	500 µg	Powder for solution for injection	Subcutaneous use	vial (glass)		4 vials
EU/1/08/497/005	Nplate	250 µg	Powder and solvent for solution for injection	Subcutaneous use	powder: vial (glass); solvent: pre-filled syringe (glass)	powder: 250 µg (500µg/ml); solvent: 0.72 ml	1 vial + 1 pre-filled syringe + 1 vial adapter + 1 needle + 1 syringe + 4 alcohol pads
EU/1/08/497/006	Nplate	250 µg	Powder and solvent for solution for injection	Subcutaneous use	powder: vial (glass); solvent: pre-filled syringe (glass)	powder: 250 µg (500µg/ml); solvent: 0.72 ml	4 x (1 vial + 1 pre-filled syringe + 1 vial adapter + 1 needle + 1 syringe + 4 alcohol pads)
EU/1/08/497/007	Nplate	500 µg	Powder and solvent for solution for injection	Subcutaneous use	powder: vial (glass); solvent: pre-filled syringe: (glass)	powder: 500 µg (500µg/ml); solvent: 1.2 ml	1 vial + 1 pre-filled syringe + 1 vial adapter + 1 needle + 1 syringe + 4 alcohol pads
EU/1/08/497/008	Nplate	500 µg	Powder and solvent for solution for injection	Subcutaneous use	powder: vial (glass); solvent: pre-filled syringe: (glass)	powder: 500 µg (500µg/ml); solvent: 1.2 ml	4 x (1 vial + 1 pre-filled syringe + 1 vial adapter + 1 needle + 1 syringe + 4 alcohol pads)