EMA/2090/2012

European Medicines Agency decision

P/0037/2012

of 20 February 2012

on the acceptance of a modification of an agreed paediatric investigation plan for romiplostim (Nplate), (EMEA-000653-PIP01-09-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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on the acceptance of a modification of an agreed paediatric investigation plan for romiplostim (Nplate), (EMEA-000653-PIP01-09-M01) 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 European Medicines Agency's decision P/173/2010 issued on 17 September 2010,

Having regard to the application submitted by Amgen Europe B.V on 26 August 2011 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 10 February 2012, 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, following a re-examination procedure of the Paediatric Committee's opinion according to Article 25(3) Regulation (EC) No 1901/2006, an opinion on the acceptance of changes to the agreed paediatric investigation plan and to the waiver.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan, including changes to the 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

Changes to the agreed paediatric investigation plan for romiplostim (Nplate), powder for solution for injection, powder and solvent for solution for injection, subcutaneous use, including changes to the waiver, 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 Amgen Europe B.V, Minervum 7061, Breda - 4817-ZK, The Netherlands.

Done at London, 20 February 2012

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

EMA/PDCO/994709/2011

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan (after re-examination)

EMEA-000653-PIP01-09-M01

Scope of the application

Active substance(s):

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 22 of Regulation (EC) No 1901/2006 as amended, Amgen Europe B.V. submitted to the European Medicines Agency on 26 August 2011 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/173/2010 of 17 September 2010. The application for modification proposed changes to the agreed paediatric investigation plan and to the waiver.

An Opinion was adopted by the Paediatric Committee on 11 November 2011, for the above mentioned product. Amgen Europe B.V. received the Paediatric Committee Opinion on 21 November 2011.

On 22 December 2011, Amgen Europe B.V. submitted to the European Medicines Agency a written request including detailed grounds for a re-examination of the Opinion.

The re-examination procedure started on 12 January 2012.

A meeting with the Paediatric Committee took place on 09 February 2012.

Scope of the modification

The paediatric investigation plan for one condition was replaced by a full waiver.

Final Opinion

1. The Paediatric Committee, having assessed the detailed grounds for re-examination, in accordance with Article 25(3) of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

1.1. to maintain its opinion and

- to amend the scope of the waiver 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 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, 10 February 2012

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:

- All subsets of the paediatric population from birth to less than 18 years 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 the specific medicinal product is likely to be unsafe.

2. Paediatric Investigation Plan

2.1. Condition: 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

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By June 2014
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	Nplate	500 µg	Powder and	Subcutaneo	powder:	powder:	1 vial + 1

EU Number	Invented name	Strength	Pharmaceutical form	Route of administration	Packaging	Content (concentration)	Package size
/497/007			solvent for solution for injection	us use	vial (glass); solvent: pre-filled syringe: (glass)	500 µg (500µg/ml); solvent: 1.2 ml	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)