



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/232690/2014

European Medicines Agency decision

P/0114/2014

of 6 May 2014

on the acceptance of a modification of an agreed paediatric investigation plan for romiplostim (Nplate) (EMA-000653-PIP01-09-M04) 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 European Medicines Agency's decision P/173/2010 issued on 17 September 2010, the decision P/0037/2012 issued on 20 February 2012, and the decision P/0170/2012 issued on 27 July 2012,

Having regard to the application submitted by Amgen Europa B.V on 20 December 2013 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 21 March 2014, 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 and to the deferral.
- (2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan, including changes to the 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

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 deferral, 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 Europa B.V, Minervum 7061, 4817-ZK - BREDA, The Netherlands.

Done at London, 6 May 2014

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

EMA/PDCO/15676/2014

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

EMA-000653-PIP01-09-M04

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 Europa B.V

Information about the authorised medicinal product:

See Annex II

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Amgen Europa B.V. submitted to the European Medicines Agency on 20 December 2013 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 issued on 17 September 2010, the decision P/0037/2012 issued on 20 February 2012, and the decision P/0170/2012 issued on 27 July 2012.

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

The procedure started on 21 January 2014.

Scope of the modification

Some measures and/or 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 and to the deferral 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 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, 21 March 2014

On behalf of the Paediatric Committee
Dr Dirk Mentzer, 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: 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 January 2019
Deferral for one or more measures 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.

Authorised pharmaceutical form(s):

Powder for solution for injection

Powder and solvent for solution for injection

Authorised route(s) of administration:

Subcutaneous use