EMA/460032/2017

European Medicines Agency decision

P/0233/2017

of 11 August 2017

on the acceptance of a modification of an agreed paediatric investigation plan for romiplostim (Nplate), (EMEA-000653-PIP01-09-M05) 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.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Contact EMA www.ema.europa.eu/contact

European Medicines Agency decision

P/0233/2017

of 11 August 2017

on the acceptance of a modification of an agreed paediatric investigation plan for romiplostim (Nplate), (EMEA-000653-PIP01-09-M05) 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, the decision P/0037/2012 issued on 20 February 2012, the decision P/0170/2012 issued on 27 July 2012 and the decision P/0114/2014 issued on 6 May 2014,

Having regard to the application submitted by Amgen Europa B.V. on 29 March 2017 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 23 June 2017, 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 Europe B.V., Minervum 7061, 4817-ZK - Breda, The Netherlands.

EMA/PDCO/226625/2017
London, 23 June 2017

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

EMEA-000653-PIP01-09-M05

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

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5510
Send a question via our website www.ema.europa.eu/contact

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 29 March 2017 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, the decision P/0170/2012 issued on 27 July 2012 and the decision P/0114/2014 issued on 6 May 2014.

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

The procedure started on 25 April 2017.

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 and to the deferral 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 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.

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 (PIP)

1. Waiver

1.1. Condition

Treatment of immune thrombocytopenia (idiopathic thrombocytopenic purpura)

The waiver applies to:

- paediatric population from birth to less than 1 year of age;
- for powder for solution for injection, for 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 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	3	**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. **Study 4** Open-label trial to evaluate, safety in children from 1 year of age to less than 18 years of age with primary ITP regardless of splenectomy status, including a protocol supplement to implement bone marrow evaluations.
Extrapolation, modelling and simulation studies	0	Not applicable.
Other studies	0	Not applicable.
Other measures	0	Not applicable.

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 September 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) patients who are refractory to other treatments (e.g. corticosteroids, immunoglobulins).

Authorised pharmaceutical form(s):

Powder for solution for injection

Powder and solvent for solution for injection

Authorised route(s) of administration:

Subcutaneous use