EMA/633039/2020

European Medicines Agency decision

P/0516/2020

of 22 December 2020

on the acceptance of a modification of an agreed paediatric investigation plan for eltrombopag (Revolade), (EMEA-000170-PIP03-13-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 726/2004[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/0262/2014 issued on 3 October 2014, the decision P/0181/2016 issued on 15 July 2016, the decision P/0007/2017 issued on 31 January 2017, and the decision P/0280/2017 issued on 4 October 2017,

Having regard to the application submitted by Novartis Europharm Limited on 3 August 2020 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 13 November 2020, 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 eltrombopag (Revolade), film-coated tablet, powder for oral suspension, oral 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 covers all conditions, indications, pharmaceutical forms, routes of administration, measures, timelines, waivers and deferrals, as agreed in the decision P/83/2008 issued on 14 October 2008, including subsequent modifications thereof, and in the decision P/234/2011 issued on 30 September 2011, including subsequent modifications thereof.

Article 3

This decision is addressed to Novartis Europharm Limited, Vista Building, Elm Park, Merrion Road, 4 – Dublin, Ireland.

EMA/PDCO/486060/2020
Amsterdam, 13 November 2020

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

EMEA-000170-PIP03-13-M04

Scope of the application

Active substance(s):

Eltrombopag

Invented name:

Revolade

Condition(s):

Treatment of aplastic anaemia

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Film-coated tablet

Powder for oral suspension

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Novartis Europharm Limited

Information about the authorised medicinal product:

See Annex II

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Novartis Europharm Limited submitted to the European Medicines Agency on 3 August 2020 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/0262/2014 issued on 3 October 2014, the decision P/0181/2016 issued on 15 July 2016, the decision P/0007/2017 issued on 31 January 2017, and the decision P/0280/2017 issued on 4 October 2017.

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

The procedure started on 15 September 2020.

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 aplastic anaemia

The waiver applies to:

- the paediatric population from birth to less than 1 year;
- for film-coated tablet and powder for oral suspension, for oral use;
- on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2. Paediatric investigation plan

2.1. Condition:

Treatment of aplastic anaemia.

2.1.1. Indication(s) targeted by the PIP

Treatment of cytopenias in paediatric patients with severe aplastic anaemia who are not receiving haematopoietic stem cell transplant.

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)

Film-coated tablet for oral use.

Powder for oral suspension for oral use.

2.1.4. Measures

Area	**Number of measures**	**Description**
Quality-related studies	1	**Study 1** Development of a powder for oral suspension in fixed single dose sachets.
Non-clinical studies	0	Not applicable.

Clinical studies	2	**Study 2** Open-label, non-controlled, multiple dose trial to evaluate pharmacokinetics, safety, activity and acceptability/palatability of eltrombopag in children from 1 year to less than 18 years of age with severe aplastic anaemia that is refractory to, or has relapsed after immunosuppressive therapy or with newly-diagnosed severe aplastic anaemia in combination with immunosuppressive therapy. **Study 3** Open-label, non-controlled, multiple dose trial to evaluate pharmacokinetics, safety and activity of eltrombopag in combination with immunosuppressive therapy in adults (and children from 2 years to less than 18 years of age) with newly-diagnosed severe aplastic anaemia.
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/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By May 2022.
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 aplastic anaemia

Authorised indication(s):

- Revolade is indicated in adult patients with acquired severe aplastic anaemia (SAA) who were either refractory to prior immunosuppressive therapy or heavily pretreated and are unsuitable for haematopoietic stem cell transplantation (see section 5.1).

2. Treatment of immune (idiopathic) thrombocytopenic purpura

Authorised indication(s):

- Revolade is indicated for chronic immune (idiopathic) thrombocytopenic purpura (ITP) patients aged 1 year and above who are refractory to other treatments (e.g. corticosteroids, immunoglobulins).

3. Treatment of secondary thrombocytopenia

Authorised indication(s):

- Revolade is indicated in adult patients with chronic hepatitis C virus (HCV) infection for the treatment of thrombocytopenia, where the degree of thrombocytopenia is the main factor preventing the initiation or limiting the ability to maintain optimal interferon-based therapy (see sections 4.4 and 5.1).

Authorised pharmaceutical form(s):

Film-coated tablet.

Powder for oral suspension.

Authorised route(s) of administration:

Oral use.