EMA/631901/2020

European Medicines Agency decision

P/0482/2020

of 9 December 2020

on the acceptance of a modification of an agreed paediatric investigation plan for avatrombopag (Doptelet), (EMEA-001136-PIP02-19-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.

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
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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/0096/2020 issued on 18 March 2020,

Having regard to the application submitted by Dova Pharmaceuticals Ireland Limited on 7 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 avatrombopag (Doptelet), 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/0309/2011 issued on 20 December 2011, including subsequent modifications thereof.

Article 3

This decision is addressed to Dova Pharmaceuticals Ireland Limited, 6th Floor, 2 Grand Canal Square, D02 A342 - Dublin, Ireland.

EMA/PDCO/439169/2020
Amsterdam, 13 November 2020

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

EMEA-001136-PIP02-19-M01

Scope of the application

Active substance(s):

Avatrombopag

Invented name:

Doptelet

Condition(s):

Treatment of chemotherapy-induced thrombocytopenia

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:

Dova Pharmaceuticals Ireland 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, Dova Pharmaceuticals Ireland Limited submitted to the European Medicines Agency on 7 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/0096/2020 issued on 18 March 2020.

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 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 chemotherapy-induced thrombocytopenia

The waiver applies to:

- the paediatric population from birth to less than 28 days;
- film-coated tablet, powder for oral suspension, oral use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2. Paediatric investigation plan

2.1. Condition:

Treatment of chemotherapy-induced thrombocytopenia

2.1.1. Indication(s) targeted by the PIP

Treatment of chemotherapy-induced thrombocytopenia in paediatric patients from 28 days to less than 18 years of age receiving myelosuppressive chemotherapy for treatment of solid tumours

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

From 28 days to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Film-coated tablet

Powder for oral suspension

2.1.4. Measures

Area	**Number of measures**	**Description**
Quality-related studies	1	**Study 1** Development of an age appropriate pharmaceutical form (powder for oral suspension) for oral use
Non-clinical studies	0	Not applicable
Clinical studies	1	**Study 2** Open-label multi-centre study to evaluate the pharmacokinetics, pharmacodynamics and safety of avatrombopag including a dose-

		finding phase and a safety assessment phase in paediatric patients from 28 days to less than 18 years of age with chemotherapy-induced thrombocytopenia
Extrapolation, modelling and simulation studies	2	**Study 3** Modelling and simulation study to determine the initial dose of avatrombopag to be used in the clinical trial in paediatric patients from 28 days to less than 18 years of age with chemotherapy-induced thrombocytopenia **Study 4** Extrapolation study to evaluate the use of avatrombopag and to extrapolate efficacy from the adult to the paediatric population from 28 days to less than 18 years of age with chemotherapy -induced thrombocytopenia
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:	No
Date of completion of the paediatric investigation plan:	By December 2024
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 thrombocytopenia purpura secondary to liver disease

Authorised indication(s):

- Doptelet is indicated for the treatment of severe thrombocytopenia in adult patients with chronic liver disease who are scheduled to undergo an invasive procedure.

Authorised pharmaceutical form(s):

Film-coated tablet

Authorised route(s) of administration:

Oral use