

EMA/658960/2019

European Medicines Agency decision

P/0007/2020

of 6 January 2020

on the acceptance of a modification of an agreed paediatric investigation plan for avapritinib (EMA-002358-PIP02-18-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 avapritinib (EMA-002358-PIP02-18-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 726/2004¹,

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/0026/2019 issued on 22 February 2019,

Having regard to the application submitted by Blueprint Medicines (Netherlands) B.V. on 8 August 2019 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 15 November 2019, 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.
- (2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

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 avapritinib, film-coated tablet, age-appropriate oral solid dosage form, oral use, 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 Blueprint Medicines (Netherlands) B.V., Gustav Mahlerplein 2, 1082 MA – Amsterdam, The Netherlands.

EMA/PDCO/465033/2019
Amsterdam, 15 November 2019

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA-002358-PIP02-18-M01

Scope of the application

Active substance(s):

Avapritinib

Condition(s):

Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms)

Pharmaceutical form(s):

Film-coated tablet

Age-appropriate oral solid dosage form

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Blueprint Medicines (Netherlands) B.V.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Blueprint Medicines (Netherlands) B.V. submitted to the European Medicines Agency on 8 August 2019 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/0026/2019 issued on 22 February 2019.

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

The procedure started on 17 September 2019.

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 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 annex 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 all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms)

The waiver applies to:

- the paediatric population from birth to less than 2 years of age;
- film-coated tablet, age-appropriate oral solid dosage form, 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 all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue neoplasms)

2.1.1. Indication(s) targeted by the PIP

Treatment of paediatric patients from 2 to less than 18 years of age with a relapsed / refractory solid tumour harbouring mutations in either KIT or PDGFR-alpha

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

From 2 to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Film-coated tablet

Age-appropriate oral solid dosage form

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	1	Study 1 Development of an age-appropriate oral solid form
Non-clinical studies	0	Not applicable.

Area	Number of measures	Description
Clinical studies	2	Study 2 Multicentre, open-label, single arm study to evaluate safety, pharmacokinetics (part 1) and anti-tumour activity (part 2) of avapritinib in paediatric patients from 2 to less than 18 years of age with a relapsed / refractory solid tumour harbouring a mutation in KIT or PDGFR-alpha and no available alternative treatment options. (BLU-285-3101) Study 3 Multicentre, open-label, randomized study to evaluate safety, pharmacokinetics and efficacy of avapritinib compared to investigator's choice of therapy in paediatric patients from 2 to less than 18 years of age with a relapsed / refractory solid tumour harbouring a mutation in KIT or PDGFR-alpha selected based on the results of Study 2. (BLU-285-3303)
Extrapolation, modelling and simulation studies	1	Study 4 Modelling and simulation study to evaluate the use and support dosing regimen of avapritinib in children and adolescents from 2 to less than 18 years of age with a relapsed / refractory solid tumour harbouring a mutation in KIT or PDGFR-alpha. (AVA-PIP-M&S-1)
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 July 2030
Deferral for one or more measures contained in the paediatric investigation plan:	Yes