EMA/246203/2024

European Medicines Agency decision

P/0203/2024

of 14 June 2024

on the acceptance of a modification of an agreed paediatric investigation plan for quizartinib (Vanflyta), (EMEA-001821-PIP01-15-M08) 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 Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the European Medicines Agency's decision P/0203/2016 issued on 22 July 2016, the decision P/0102/2018 issued on 16 March 2018, the decision P/0268/2018 issued on 16 August 2018, the decision P/0163/2019 issued on 26 April 2019, the decision P/0343/2020 issued on 9 September 2020, the decision P/0281/2021 issued on 16 July 2021, the decision P/0091/2023 issued on 10 March 2023 and the decision P/0259/2023 issued on 13 July 2023,

Having regard to the application submitted by Daiichi Sankyo Europe GmbH on 22 January 2024 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 26 April 2024, 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, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for quizartinib (Vanflyta), film-coated tablet, powder for oral solution, 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 Daiichi Sankyo Europe GmbH, 48 Zielstattstrasse, 81379 - Munich Germany.

EMA/PDCO/46963/2024
Amsterdam, 26 April 2024

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

EMEA-001821-PIP01-15-M08

Scope of the application

Active substance(s):

Quizartinib

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of acute myeloid leukaemia

Pharmaceutical form(s):

Film-coated tablet

Powder for oral solution

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Daiichi Sankyo Europe GmbH

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Daiichi Sankyo Europe GmbH submitted to the European Medicines Agency on 22 January 2024 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/0203/2016 issued on 22 July 2016, the decision P/0102/2018 issued on 16 March 2018, the decision P/0268/2018 issued on 16 August 2018, the decision P/0163/2019 issued on 26 April 2019, the decision P/0343/2020 issued on 9 September 2020, the decision P/0281/2021 issued on 16 July 2021, the decision P/0091/2023 issued on 10 March 2023 and the decision P/0259/2023 issued on 13 July 2023.

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

The procedure started on 26 February 2024.

Scope of the modification

Some measures 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 Paediatric Committee member of Norway 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 acute myeloid leukaemia

The waiver applies to:

- the paediatric population from birth to less than 1 month of age;
- film-coated tablet, powder for oral solution, 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 acute myeloid leukaemia

2.1.1. Indication(s) targeted by the PIP

- For the treatment of paediatric patients aged from 1 month to less than 18 years of age with refractory or relapsed AML with FLT3-ITD mutations after failure of front line intensive chemotherapy regimen, in combination with standard chemotherapy.
- For the treatment of paediatric patients aged from 1 month to less than 18 years of age with newly diagnosed AML with FLT3-ITD mutations and NPM1 wild-type.

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

From 1 month to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Film-coated tablet

Powder for oral solution

2.1.4. Measures

Area	Description
Quality-related studies	Not applicable
Non-clinical studies	**Study 1** Dose range-finding juvenile toxicity study **Study 2** Juvenile toxicity study

Clinical studies	**Study 3** Open-label, single-arm trial to evaluate safety, pharmacokinetics, pharmacodynamics and efficacy of quizartinib in combination with FLA chemotherapy, with optional consolidation therapy, and as single-agent after high-dose therapy in paediatric patients with FLT3-ITD AML from 1 month to less than 18 years of age (and young adults), **Study 4** Open-label, single-arm trial to evaluate safety, pharmacokinetics, pharmacodynamics and activity of quizartinib in combination with chemotherapy and as single-agent after high-dose therapy in paediatric patients newly-diagnosed with FLT3-ITD AML and NPM1 wild-type from 1 month to less than 18 years of age (and young adults)
Extrapolation, modelling and simulation studies	**Study 5** Exposure-response model
Other measures	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 December 2027
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

Condition(s) and authorised indication(s)

1. Treatment of acute myeloid leukaemia

Authorised indication(s):

- VANFLYTA is indicated in combination with standard cytarabine and anthracycline induction and standard cytarabine consolidation chemotherapy, followed by VANFLYTA single-agent maintenance therapy for adult patients with newly diagnosed acute myeloid leukaemia (AML) that is FLT3-ITD positive
  - Invented name(s): VANFLYTA
  - Authorised pharmaceutical form(s): Film-coated tablet
  - Authorised route(s) of administration: Oral use
  - Authorised via centralised procedure.