EMA/507354/2016

European Medicines Agency decision

P/0203/2016

of 22 July 2016

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for quizartinib (EMEA-001821-PIP01-15) 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 application submitted by Daiichi Sankyo Europe GmbH on 10 August 2015 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 24 June 2016, in accordance with Article 18 of Regulation (EC) No 1901/2006, and Article 21 of said Regulation and Article 13 of said Regulation,

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 agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver.

(2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.

(3) It is therefore appropriate to adopt a decision granting a deferral.

(4) It is therefore appropriate to adopt a decision granting a waiver.

[1] OJ L 378, 27.12.2006, p.1.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

A paediatric investigation plan for quizartinib, film-coated tablet, powder for oral solution, oral use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

Article 2

A deferral for quizartinib, film-coated tablet, powder for oral solution, oral use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

A waiver for quizartinib, film-coated tablet, powder for oral solution, oral use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 4

This decision is addressed to Daiichi Sankyo Europe GmbH, Zielstattstrasse 48, 81379 – Munchen, Germany

Done at London, 22 July 2016

For the European Medicines Agency
Zaïde Frias
Head of Division
Human Medicines Research and Development Support
(Signature on file)

EMA/PDCO/256115/2016
London, 24 June 2016

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a deferral and a waiver

EMEA-001821-PIP01-15

Scope of the application

Active substance(s):

Quizartinib

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 16(1) of Regulation (EC) No 1901/2006 as amended, Daiichi Sankyo Europe GmbH submitted for agreement to the European Medicines Agency on 10 August 2015 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 15 September 2015.

Supplementary information was provided by the applicant on 1 April 2016. The applicant proposed modifications to the paediatric investigation plan.

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Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree the paediatric investigation plan in accordance with Article 18 of said Regulation;
   - to grant a deferral in accordance with Article 21 of said Regulation;

     to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

   The Norwegian Paediatric Committee member agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed 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 acute myeloid leukaemia

The waiver applies to:

- the paediatric population from birth to less than 1 month of age;
- for film-coated tablet and 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 FLT3-ITD(+) AML 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 FLT3-ITD(+) AML.

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	Number of measures	Description
Quality-related studies	0	Not applicable.
Non-clinical studies	2	**Study 1** Dose range-finding juvenile toxicity study. **Study 2** Juvenile toxicity study.

Clinical studies	2	**Study 3** Open-label, single-arm trial to evaluate safety, pharmacokinetics, pharmacodynamics and activity of quizartinib in combination with FLAG/DNX chemotherapy 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), with expansion cohort. **Study 4** Open-label, single-arm trial to evaluate safety, pharmacokinetics, pharmacodynamics and activity of quizartinib in combination with ADE chemotherapy and as single-agent after high-dose therapy in paediatric patients newly-diagnosed with FLT3-ITD AML from 1 month to less than 18 years of age (and young adults).
Extrapolation, modelling and simulation studies	1	**Study 5** Exposure-response model.
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 June 2022
Deferral for one or more measures contained in the paediatric investigation plan:	Yes