EMA/395885/2021

European Medicines Agency decision

P/0331/2021

of 13 August 2021

on the agreement of a paediatric investigation plan and on the granting of a deferral for infigratinib, (EMEA-002594-PIP02-20) 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 QED Therapeutics Inc. on 7 August 2020 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 25 June 2021, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 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.

(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.

[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 infigratinib, tablet, 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 infigratinib, tablet, 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

This decision is addressed to QED Therapeutics Inc., 8000 Marina Boulevard, Suite 400, 94005 - Brisbane, CA USA.

EMA/464037/2008
Amsterdam, 25 June 2021

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

EMEA-002594-PIP02-20

Scope of the application

Active substance(s):

Infigratinib

Condition(s):

Treatment of achondroplasia

Pharmaceutical form(s):

Tablet

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

QED Therapeutics Inc.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, QED Therapeutics Inc. submitted for agreement to the European Medicines Agency on 7 August 2020 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 2020.

Supplementary information was provided by the applicant on 19 March 2021. The applicant proposed modifications to the paediatric investigation plan and withdrew its request for a waiver.

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 17(1) of said Regulation;
   - to grant a deferral in accordance with Article 21 of said Regulation.

   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 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

Not applicable

2. Paediatric investigation plan

2.1. Condition:

Treatment of achondroplasia

2.1.1. Indication(s) targeted by the PIP

Treatment of achondroplasia

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

From birth to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Tablet

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	1	**Study 1** Development of an age appropriate formulation for patients from birth.
Non-clinical studies	0	Not applicable.
Clinical studies	5	**Study 2** Observational study to evaluate the natural history of children with achondroplasia (ACH) from birth to less than 18 years of age. **Study 3** Open-label, dose finding trial to evaluate pharmacokinetics, safety and activity of infigratinibin children from 3 years to less than 12 years of age with achondroplasia. **Study 4** Double-blind, randomised, placebo controlled trial to evaluate safety, efficacy and acceptability/palatability of infigratinib in children from 3 to less than 18 years of age with achondroplasia in terms of superiority of infigratinib over placebo.

		Study 5 Open-label, long term extension study to provide long term safety and efficacy of infigratinib in patients with achondroplasia who previously participated in PIP studies 3, 4 or 6. **Study 6** Two part trial, consisting of an open label dose finding part to evaluate pharmacokinetics, safety and activity, followed by a double blind, randomised, placebo controlled part to evaluate safety, acceptability/palatability and efficacy in terms of superiority of infigratinib over placebo in children from birth to less than 3 years of age with achondroplasia.
Extrapolation, modelling and simulation studies	2	**Study 7** Modelling and simulation study to support dose finding of the product in the treatment of achondroplasia in children from birth to less than 18 years of age. **Study 8** Modelling and simulation study to evaluate the use of the product in the in children from birth to less than 18 years of age with achondroplasia.
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 February 2036
Deferral for one or more measures contained in the paediatric investigation plan:	Yes