



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/299951/2021

European Medicines Agency decision P/0223/2021

of 8 June 2021

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for erdafitinib (EMA-002042-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 726/2004¹,

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 application submitted by Janssen-Cilag International N.V. on 5 June 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 23 April 2021, in accordance with Article 17 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 erdafitinib, film-coated tablet, age-appropriate dosage form, 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 erdafitinib, film-coated tablet, age-appropriate dosage form, 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 erdafitinib, film-coated tablet, age-appropriate dosage form, 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 Janssen-Cilag International N.V., Turnhoutseweg 30, B-2340 – Beerse, Belgium.



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/PDCO/91601/2021
Amsterdam, 23 April 2021

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

EMA-002042-PIP02-20

Scope of the application

Active substance(s):

Erdafitinib

Condition(s):

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

Pharmaceutical form(s):

Film-coated tablet

Age-appropriate dosage form

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Janssen-Cilag International N.V.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Janssen-Cilag International N.V. submitted for agreement to the European Medicines Agency on 5 June 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 6 July 2020.

Supplementary information was provided by the applicant on 18 January 2021. The applicant proposed modifications to the paediatric investigation plan.



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;
 - 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)(a) of said Regulation, on the grounds that the specific medicinal product is likely to be ineffective or unsafe in part or all of the paediatric population.

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

The waiver applies to:

- the paediatric population from birth to less than 2 years;
- film-coated tablet, age-appropriate dosage form, oral use;
- on the grounds that the specific medicinal product is likely to be unsafe.

2. Paediatric investigation plan

2.1. Condition:

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

2.1.1. Indication(s) targeted by the PIP

Treatment of locally advanced or metastatic solid tumours harbouring susceptible FGFR alterations in paediatric patients from 2 years to less than 18 years of age who have either progressed following prior therapies and who have no acceptable standard therapies or who have a newly-diagnosed solid tumour harbouring susceptible FGFR alterations and have no acceptable standard therapies.

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

From 2 years to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Film-coated tablet

Age-appropriate dosage form

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	1	Study 1 Development of an age-appropriate dosage (solid dosage form or liquid dosage) form for children from 2 years to less than 6 years of age.
Non-clinical	0	Not applicable.

Area	Number of measures	Description
studies		
Clinical studies	2	Study 2 Open-label, single-arm trial to evaluate the safety, pharmacokinetics and anti-tumour activity of erdafitinib in paediatric patients from 2 years to less than 18 years of age (and young adults) with a recurrent or refractory solid tumour harbouring select FGFR1/2/3/4 alterations (APEC1621B, one arm of the Pediatric MATCH Treatment Trial) Study 3 Open-label, single-arm trial to assess the safety, pharmacokinetics and efficacy of erdafitinib in paediatric patients from 2 years to less than 18 years of age, paediatric panel cohort (and adults, broad panel cohort) with a solid tumour harbouring select FGFR1/2/3/4 alterations and who have either progressed following prior therapies and who have no acceptable standard therapy or with newly diagnosed tumours with FGFR alterations who have no acceptable standard therapies (42756493CAN2002 , RAGNAR).
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 June 2029
Deferral for one or more measures contained in the paediatric investigation plan:	Yes