EMA/251362/2022

European Medicines Agency decision

P/0190/2022

of 10 June 2022

on the acceptance of a modification of an agreed paediatric investigation plan for erdafitinib (EMEA-002042-PIP02-20-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 erdafitinib (EMEA-002042-PIP02-20-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 726/2004[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/0223/2021 issued on 8 June 2021,

Having regard to the application submitted by Janssen-Cilag International N.V. on 24 January 2022 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 22 April 2022, 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 erdafitinib, film-coated tablet, age-appropriate 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 Janssen-Cilag International N.V., Turnhoutseweg 30, B-2340 – Beerse, Belgium.

EMA/PDCO/63507/2022
Amsterdam, 22 April 2022

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

EMEA-002042-PIP02-20-M01

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 22 of Regulation (EC) No 1901/2006 as amended, Janssen-Cilag International N.V. submitted to the European Medicines Agency on 24 January 2022 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/0223/2021 issued on 8 June 2021.

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

The procedure started on 21 February 2022.

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 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 of age;
- 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	Description
Quality-related studies	**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 studies	Not applicable.

Clinical studies	**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	Not applicable.
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 June 2029
Deferral for one or more measures contained in the paediatric investigation plan:	Yes