MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

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Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral and grant a waiver

MHRA-100335-PIP01-21

Scope of the Application

Active Substance(s)

erdafitinib

Condition(s)

Malignant neoplasms (except urothelial carcinoma, haematopoietic and lymphoid tissue)

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 Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Janssen-Cilag Limited submitted to the licensing authority on 26/11/2021 09:12 GMT an application for a Paediatric Investigation Plan

The procedure started on 22/07/2022 07:26 BST

1. The licensing authority, having assessed the application in accordance with the Human Medicines Regulations 2012, decides, as set out in the appended summary report:

to agree a paediatric investigation plan and grant a deferral and grant a waiver

2. The measures and timelines of the paediatric investigation plan are set out in the Annex I.

This decision is forwarded to the applicant, together with its annex and appendix.

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Final Decision Letter

MHRA-100335-PIP01-21

Of 04/08/2022 07:29 BST

On the adopted decision for erdafitinib (MHRA-100335-PIP01-21) in accordance with the Human Medicines Regulations 2012.

The licensing authority, in accordance with the Human Medicines Regulations 2012, has adopted this decision:

Agreement on a paediatric investigation plan

This decision applies to a Paediatric Investigation Plan for erdafitinib , Film-coated tablet; Age-appropriate dosage form , Oral use .

This decision is addressed to Janssen-Cilag Limited, 50-100 Holmers Farm Way, Wycombe, Buckinghamshire, United Kingdom, HP12 4EG

ANNEX I

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 / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age Pharmaceutical form(s): Film-coated tablet; Age-appropriate dosage form Route(s) of administration: Oral use Reason for granting waiver: on the grounds that the specific medicinal product is likely to be unsafe.

2. Paediatric Investigation Plan:

2.1 Condition(s):

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

2.2 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 alternations and have no acceptable standard therapies

2.3 Subset(s) of the paediatric population concerned by the paediatric development:

The paediatric population from 2 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Film-coated tablet; Age-appropriate dosage form

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Development of an age-appropriate dosage form (solid dosage or liquid dosage form) for children from 2 years to less than 6 years of age.
Non-Clinical Studies	0	Not applicable
Clinical Studies	2	Study 2 (APEC1621B, one arm of the Pediatric MATCH Treatment Trial) 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. Study 3 (42756493CAN2002, RAGNAR) 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.

Extrapolation, Modeling & Simulation Studies	0	Not applicable
Other Studies	0	Not applicable
Other Measures	0	Not applicable

3. Follow-up, completion and deferral of a PIP:

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	30/06/2029
Deferral of one or more studies contained in the paediatric investigation plan:	Yes