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

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

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

MHRA-100503-PIP01-22

Scope of the Application

Active Substance(s)

efavaleukin alfa

Condition(s)

Treatment of ulcerative colitis

Pharmaceutical Form(s)

Solution for injection

Route(s) of Administration

SUBCUTANEOUS USE

Name / Corporate name of the PIP applicant

Amgen Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Amgen Limited submitted to the licensing authority on 22/04/2022 10:09 BST an application for a Paediatric Investigation Plan

The procedure started on 06/12/2022 07:43 GMT

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.

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

gov.uk/mhra

Final Decision Letter

MHRA-100503-PIP01-22

Of 23/12/2022 14:08 GMT

On the adopted decision for efavaleukin alfa (MHRA-100503-PIP01-22) 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 efavaleukin alfa, Solution for injection , SUBCUTANEOUS USE .

This decision is addressed to Amgen Limited, 216 Cambridge Science Park, Milton Road, Cambridge, UNITED KINGDOM, CB4 0WA

ANNEX I

1. Waiver

1.1 Condition:

Treatment of ulcerative colitis The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age Pharmaceutical form(s): Solution for injection Route(s) of administration: SUBCUTANEOUS USE Reason for granting waiver: on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of ulcerative colitis

2.2 Indication(s) targeted by the PIP:

Treatment of children 2 years and older with moderately to severe active ulcerative colitis (UC)

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):

Solution for injection

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable
Non-Clinical Studies	0	Not applicable
Clinical Studies	1	Study 1 Single arm, open-label study to evaluate the efficacy, pharmacokinetics (PK), pharmacodynamics (PD), safety, and tolerability of efavaleukin alfa in children and adolescents from 2 years to less than 18 years of age with moderately to severely active ulcerative colitis over 52 weeks.
Extrapolation, Modeling & Simulation Studies	1	Study 2 Modelling and simulation dose-finding study to evaluate use of efavaleukin alfa in the treatment of ulcerative colitis in children and adolescents from 2 years to less than 18 years of age.
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:	No
Date of completion of the paediatric investigation plan:	31/01/2034
Deferral of one or more studies contained in the paediatric investigation plan:	Yes