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

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan and to the deferral

MHRA-100286-PIP01-21-M01

Scope of the Application

Active Substance(s)

USTEKINUMAB

Condition(s)

Treatment of Ulcerative colitis

Pharmaceutical Form(s)

Concentrate for solution for infusion; Solution for injection; Solution for injection in pre-filled syringe

Route(s) of Administration

Intravenous use, Subcutaneous 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 25/10/2021 08:00 BST an application for a Modification

The procedure started on 29/07/2022 15:25 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 accept change(s) to the agreed paediatric investigation plan and to the deferral

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-100286-PIP01-21-M01

Of 26/08/2022 14:33 BST

On the adopted decision for USTEKINUMAB (MHRA-100286-PIP01-21-M01) 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 modification of a paediatric investigation plan (including modification of a waiver or deferral included in that paediatric investigation plan)

This decision applies to a Modification for USTEKINUMAB, Concentrate for solution for infusion; Solution for injection; Solution for injection in pre-filled syringe , Intravenous use, Subcutaneous use .

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

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): Concentrate for solution for infusion; Solution for injection; Solution for injection in pre-filled syringe Route(s) of administration: Intravenous use; 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 Ulcerative colitis

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):

Concentrate for solution for infusion; Solution for injection; Solution for injection in pre-filled syringe

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable
Non-Clinical Studies	0	Not applicable
Clinical Studies	2	Study 1(CNTO1275CRD1001) Randomised, double-blind, pharmacokinetics (PK) study of intravenous (IV) ustekinumab induction followed by subcutaneous (SC) ustekinumab maintenance in children and adolescents 2 years to less than 18 years (6 years to less than 18 years in the EU) with moderately to severely active Crohn's disease (CD) who have had an inadequate response and/or intolerance to conventional therapies. Study 2 (CNTO1275PUC3001) Open-label single administration of intravenous (IV) induction dose of ustekinumab followed by a randomised, double-blind, 2-arm study of two different subcutaneous (SC) ustekinumab maintenance dose regimens to assess the PK, safety and clinical response in children and adolescents 2 years to less than 18 years with moderately to severely active Ulcerative Colitis (UC) who have had an inadequate response and/ or intolerance to biologic therapy and/or conventional therapies.
Extrapolation, Modeling & Simulation Studies	3	Study 3 Population PK model Study 4 Exposure-response model Study 5 Analysis of internal and literature data to support the assumptions

		of similarity of disease, treatment effects, and exposure-response relationship between paediatric and adult subjects with ulcerative colitis (UC).
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:	30/09/2025
Deferral of one or more studies contained in the paediatric investigation plan:	Yes