

EMA/72931/2021

European Medicines Agency decision P/0084/2021

of 19 March 2021

on the acceptance of a modification of an agreed paediatric investigation plan for ustekinumab (Stelara), (EMA-000311-PIP05-17-M02) 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 European Medicines Agency's decision P/0190/2018 issued on 17 July 2018 and the decision P/0281/2020 issued on 12 August 2020,

Having regard to the application submitted by Janssen-Cilag International NV on 23 October 2020 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 29 January 2021, 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.

² OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for ustekinumab (Stelara), concentrate for solution for infusion, solution for injection, intravenous use, subcutaneous 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 covers all conditions, indications, pharmaceutical forms, routes of administration, measures, timelines, waivers and deferrals, as agreed in the decision P/19/2009 issued on 4 February 2009, P/0062/2012 issued on 28 March 2012, P/0045/2014 issued on 7 March 2014 including subsequent modifications thereof.

Article 3

This decision is addressed to Janssen-Cilag International NV, Turnhoutseweg 30, B2340 - Beerse, Belgium.

EMA/PDCO/577738/2020
Amsterdam, 29 January 2021

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA-000311-PIP05-17-M02

Scope of the application

Active substance(s):

Ustekinumab

Invented name:

Stelara

Condition(s):

Treatment of ulcerative colitis

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Concentrate for solution for infusion

Solution for injection

Route(s) of administration:

Intravenous use

Subcutaneous use

Name/corporate name of the PIP applicant:

Janssen-Cilag International NV

Information about the authorised medicinal product:

See Annex II

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Janssen-Cilag International NV submitted to the European Medicines Agency on 23 October 2020 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/0190/2018 issued on 17 July 2018 and the decision P/0281/2020 issued on 12 August 2020.

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

The procedure started on 1 December 2020.

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 Norwegian Paediatric Committee member 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 annexes 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 ulcerative colitis

The waiver applies to:

- the paediatric population from birth to less than 2 years;
- concentrate for solution for infusion, solution for injection, intravenous use, subcutaneous use;
- 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:

Treatment of ulcerative colitis

2.1.1. Indication(s) targeted by the PIP

Treatment of ulcerative colitis

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

From 2 to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Concentrate for solution for infusion

Solution for injection

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable
Non-clinical studies	0	Not applicable
Clinical studies	2	Study 1 Randomized, double-blind, pharmacokinetics (PK) study of intravenous (IV) ustekinumab induction followed by subcutaneous (SC) ustekinumab maintenance in children and adolescents 2 to less than 18 years (6 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. (CNT01275CRD1001)

		Study 2 Open-label single administration of intravenous (IV) induction dose of ustekinumab followed by a randomized, 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 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. (CNT01275PUC3001)
Extrapolation, modelling and 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 PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By September 2025
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

- Crohn's disease

Stelara is indicated for the treatment of adult patients with moderately to severely active Crohn's disease who have had an inadequate response with, lost response to, or were intolerant to either conventional therapy or a TNF α antagonist or have medical contraindications to such therapies.

- Plaque psoriasis

Stelara is indicated for the treatment of moderate to severe plaque psoriasis in adults who failed to respond to, or who have a contraindication to, or are intolerant to other systemic therapies including ciclosporin, methotrexate (MTX) or PUVA (psoralen and ultraviolet A).

- Paediatric plaque psoriasis

Stelara is indicated for the treatment of moderate to severe plaque psoriasis in children and adolescent patients from the age of 6 years and older, who are inadequately controlled by, or are intolerant to, other systemic therapies or phototherapies.

- Psoriatic arthritis (PsA)

Stelara, alone or in combination with MTX, is indicated for the treatment of active psoriatic arthritis in adult patients when the response to previous non-biological disease-modifying anti-rheumatic drug (DMARD) therapy has been inadequate.

Authorised pharmaceutical form(s):

Concentrate for solution for infusion

Solution for injection

Solution for injection in pre-filled syringe

Authorised route(s) of administration:

Intravenous use

Subcutaneous use