



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMADOC-1700519818-2038993

European Medicines Agency decision

EMEA-003385-PIP01-22

of 15 April 2025

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for axatilimab 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 agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for axatilimab 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 726/2004¹,

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 Agency²,

Having regard to the application submitted by Incyte Biosciences Distribution B.V. on 19 December 2022 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 28 February 2025, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 of said Regulation and Article 13 of said Regulation,

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 agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver.
- (2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.
- (3) It is therefore appropriate to adopt a decision granting a deferral.
- (4) It is therefore appropriate to adopt a decision granting a waiver.

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

A paediatric investigation plan for axatilimab, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

Article 2

A deferral for axatilimab, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

A waiver for axatilimab, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 4

This decision is addressed to Incyte Biosciences Distribution B.V., Paasheuvelweg 25, 1105 - BP, Amsterdam Noord-Holland, Netherlands.



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMADOC-1700519818-1811340
Amsterdam, 28 February 2025

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a deferral and a waiver

EMA-003385-PIP01-22

Scope of the application

Active substance(s):

Axatilimab

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of chronic graft-versus-host disease

Pharmaceutical form(s):

Solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Incyte Biosciences Distribution B.V.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Incyte Biosciences Distribution B.V. submitted for agreement to the European Medicines Agency on 19 December 2022 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 30 January 2023.

Supplementary information was provided by the applicant on 22 November 2024. The applicant proposed modifications to the paediatric investigation plan.



Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

- to agree the paediatric investigation plan in accordance with Article 17(1) of said Regulation;
- to grant a deferral in accordance with Article 21 of said Regulation;
- to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

The Paediatric Committee member of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed 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 chronic graft-versus-host disease

The waiver applies to:

- the paediatric population from birth to less than 2 years of age;
- solution for infusion, intravenous use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2. Paediatric investigation plan

2.1. Condition:

Treatment of chronic graft-versus-host disease

2.1.1. Indication(s) targeted by the PIP

Treatment of patients with chronic graft-versus-host disease after failure of 2 or more lines of systemic therapy.

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

From 2 years of age to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Solution for infusion

2.1.4. Measures

Area	Description
Quality-related studies	Not applicable
Non-clinical studies	Study 1 (8465308/T22-07-07) 26-week toxicity study in juvenile monkeys
Clinical studies	Study 2 (SNDX-6352-0504 (AGAVE-201)) Open-label, randomized, multicenter study to evaluate the effect, safety and tolerability of axatilimab at 3 different doses in paediatric patients from 7 years to less than 18 years of age (and adults) with recurrent or refractory active chronic graft-versus-host disease (GVHD) who have received at least 2 prior lines of systemic therapy.

	<p>Study 3 (INCA034176-256)</p> <p>Open-label, randomized, multicenter study of axatilimab versus Best Available Therapy (BAT) to determine the effect, safety, tolerability, and pharmacokinetics of axatilimab single agent in paediatric patients from 2 years to less than 18 years of age with moderate or severe cGVHD after failure of 2 or more lines of systemic therapy.</p>
Modelling and simulation analyses	<p>Study 4</p> <p>Population pharmacokinetic/pharmacodynamics modelling study, to inform dose selection in paediatric patients from 2 years to less than 18 years of age with chronic graft-versus-host disease.</p>
Other studies	Not applicable
Extrapolation plan	Studies 2, 3 and 4 are part of an extrapolation plan covering the paediatric population from 2 years to less than 18 years of age, as agreed by the PDCO.

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 2034
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

The product is not authorised anywhere in the European Community.