**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-101861-PIP01-25

# Scope of the Application

## Active Substance(s)

Axatilimab

## 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 UK LIMITED

## Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, INCYTE BIOSCIENCES UK LIMITED submitted to the licensing authority on 14/05/2025 19:50 BST an application for a Paediatric Investigation Plan

The procedure started on 10/06/2025 14:21 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-101861-PIP01-25

Of 29/01/2026 08:40 GMT

On the adopted decision for Axatilimab (MHRA-101861-PIP01-25) 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 Axatilimab, Solution for infusion , INTRAVENOUS USE .

This decision is addressed to INCYTE BIOSCIENCES UK LIMITED, First Floor 1, Q1 The Square, Randalls Way, Leatherhead, UNITED KINGDOM, KT22 7TW

## ANNEX I

### 1. Waiver

### 1.1 Condition:

| |
|---|
| Treatment of chronic graft-versus-host disease 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 infusion Route(s) of administration: INTRAVENOUS 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 chronic graft-versus-host disease |

## 2.2 Indication(s) targeted by the PIP:

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

## 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 infusion |

## 2.5 Studies:

| Study Type | Number of Studies | Study Description |
|---|---|---|
| **Quality Measures** | 0 | Not applicable. |
| **Non-Clinical Studies** | 1 | Study 1 (8465308/T22-07-07) Definitive 26-week toxicity study in juvenile monkeys. |
| **Clinical Studies** | 2 | Study 2 (SNDX-6352-0504 [AGAVE-201]) Open-label, randomised, multicentre study to evaluate the safety, activity 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. Study 3 (INCA034176-256) Open-label, randomised multicentre study to evaluate the activity, safety, tolerability, and pharmacokinetics of axatilimab compared to best available therapy (BAT) 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. |
| **Extrapolation, Modeling & Simulation Studies** | 1 | Study 4 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. |
| **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:** | 30/06/2034 |
| **Deferral of one or more studies contained in the paediatric investigation plan:** | Yes |