**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-101953-PIP01-25-M01

# Scope of the Application

## Active Substance(s)

Nipocalimab

## Condition(s)

Treatment of myasthenia gravis

## Pharmaceutical Form(s)

Solution for infusion, Concentrate for solution for infusion

## Route(s) of Administration

INTRAVENOUS 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 19/05/2025 08:07 BST an application for a Modification

The procedure started on 14/07/2025 10:20 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.

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# Final Decision Letter

MHRA-101953-PIP01-25-M01

Of 31/07/2025 14:56 BST

On the adopted decision for Nipocalimab (MHRA-101953-PIP01-25-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 Nipocalimab, Solution for infusion, Concentrate for solution for infusion , INTRAVENOUS USE .

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

## ANNEX I

### 1. Waiver

### 1.1 Condition:

| |
|---|
| Treatment of myasthenia gravis 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; Concentrate for solution for infusion Route(s) of administration: INTRAVENOUS USE Reason for granting waiver: On the grounds the specific medicinal product is likely to be unsafe. |

### 2. Paediatric Investigation Plan:

### 2.1 Condition(s):

| |
|---|
| Treatment of myasthenia gravis |

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

| |
|---|
| Treatment of generalised myasthenia gravis |

## 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 Concentrate for solution for infusion |

## 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 Open-label uncontrolled trial to evaluate pharmacokinetics, pharmacodynamics, safety and activity of anti-neonatal Fc receptor human monoclonal antibody (hereafter referred to as M281) in children from 2 years to less than 18 years of age with generalised myasthenia gravis. |
| **Extrapolation, Modeling & Simulation Studies** | 2 | Study 2 Modelling and simulation study to support the use of M281 for the treatment of generalised myasthenia gravis in children from 2 years to less than 18 years of age. Study 3 Extrapolation study to support the use of M281 for the treatment of generalised myasthenia gravis in children 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:** | Yes |
| **Date of completion of the paediatric investigation plan:** | 31/12/2026 |
| **Deferral of one or more studies contained in the paediatric investigation plan:** | Yes |