**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-100043-PIP01-21

# Scope of the Application

## Active Substance(s)

zilucoplan

## Condition(s)

Treatment of myasthenia gravis

## Pharmaceutical Form(s)

Solution for injection; Age appropriate dosage form for parenteral use.

## Route(s) of Administration

Subcutaneous use

## Name / Corporate name of the PIP applicant

UCB Pharma Limited

## Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, UCB Pharma Limited submitted to the licensing authority on 14/04/2021 12:40 BST an application for a Paediatric Investigation Plan

The procedure started on 23/12/2021 17:11 GMT

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-100043-PIP01-21

Of 13/01/2022 15:26 GMT

On the adopted decision for zilucoplan (MHRA-100043-PIP01-21) 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 zilucoplan, Solution for injection; Age appropriate dosage form for parenteral use. , Subcutaneous use .

This decision is addressed to UCB Pharma Limited, 208 Bath Road, Slough, United Kingdom, SL1 3WE

## 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 injection; Age appropriate dosage form for parenteral use Route(s) of administration: Subcutaneous use Reason for granting waiver: on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s). |

### 2. Paediatric Investigation Plan:

### 2.1 Condition(s):

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

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

| |
|---|
| Treatment of acetylcholine receptor antibody positive (AChR Ab +) 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 injection; Age appropriate dosage form for parenteral use |

### 2.5 Studies:

| Study Type | Number of Studies | Study Description |
|---|---|---|
| **Quality Measures** | 1 | Study 1 Development of an age-appropriate dosage form for parenteral use appropriate for children from 2 years to less than 12 years of age. |
| **Non-Clinical Studies** | 0 | Not applicable |
| **Clinical Studies** | 1 | Study 2 Open label, uncontrolled trial to evaluate pharmacokinetics, safety , tolerability and activity of zilucoplan in children from 2 years to less than 18 years with myasthenia gravis. |
| **Extrapolation, Modeling & Simulation Studies** | 2 | Study 3 Modelling and simulation study to establish the doses for zilucoplan in children from 2 years to less than 18 years of age with myasthenia gravis. Study 4 Analysis of existing data to extrapolate efficacy of zilucoplan from adults with generalised myasthenia gravis (gMG) to the paediatric population. |
| **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:** | 30/09/2026 |
| **Deferral of one or more studies contained in the paediatric investigation plan:** | Yes |