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 (MHRA-100043-PIP01-21-M01) and to the deferral

MHRA-100043-PIP01-21-M02

Scope of the Application

Active Substance(s)

ZILUCOPLAN SODIUM

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 Ltd.

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, UCB Pharma Ltd. submitted to the licensing authority on 11/03/2026 11:57 GMT an application for a Modification

The procedure started on 30/03/2026 09:23 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.

MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

gov.uk/mhra

Final Decision Letter

MHRA-100043-PIP01-21-M02

Of 03/07/2026 07:17 BST

On the adopted decision for ZILUCOPLAN SODIUM (MHRA-100043-PIP01-21-M02) 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 ZILUCOPLAN SODIUM, Solution for injection Age appropriate dosage form for parenteral use , SUBCUTANEOUS USE .

This decision is addressed to UCB Pharma Ltd., 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, including development of a medical administration device with suitable graduation appropriate for children with body weight from 10 to less than 30 kg.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	1	Study 2 (MG0014) Open label, uncontrolled trial to evaluate pharmacokinetics, pharmacodynamics, safety, tolerability and activity of zilucoplan in children from 2 years to less than 18 years with generalised myasthenia gravis.
Extrapolation, Modeling & Simulation Studies	2	Study 3 (CL0515 / CL0544) Modelling and simulation study to establish the doses for zilucoplan in children from 2 years to less than 18 years of age with generalised myasthenia gravis. Study 4 (CL0516) 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:	30/06/2027
Deferral of one or more studies contained in the paediatric investigation plan:	Yes