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

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Decision Cover Letter

Decision of the licensing authority to:

accept of change(s) to the agreed paediatric investigation plan and to the deferral

MHRA-101185-PIP01-23-M01

Scope of the Application

Active Substance(s)

gefurulimab

Condition(s)

Treatment of Myasthenia gravis

Pharmaceutical Form(s)

Solution for injection

Route(s) of Administration

SUBCUTANEOUS USE

Name / Corporate name of the PIP applicant

Alexion Europe SAS

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Alexion Europe SAS submitted to the licensing authority on 02/04/2026 13:28 BST an application for a

The procedure started on 13/04/2026 14:30 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-101185-PIP01-23-M01

Of 01/06/2026 10:26 BST

On the adopted decision for gefurulimab (MHRA-101185-PIP01-23-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 for gefurulimab, Solution for injection , SUBCUTANEOUS USE .

This decision is addressed to Alexion Europe SAS, 103-105 rue Anatole France, Levallois-Perret, FRANCE, 92300

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 6 years Pharmaceutical form(s): Solution for injection 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). Reason for Refusing Waiver: Not Applicable

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 generalised myasthenia gravis

2.3 Subset(s) of the paediatric population concerned by the paediatric development:

The paediatric population from 6 years to less than 18 years

2.4 Pharmaceutical Form(s):

Solution for injection

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, single-arm study to evaluate pharmacokinetics (PK), pharmacodynamics (PD), safety and activity of gefurulimab in paediatric patients from 6 years to less than 18 years of age with generalised myasthenia gravis (gMG) who express acetylcholine receptor-antibodies (AChR+) (ALXN1720-MG-302)
Extrapolation, Modeling & Simulation Studies	1	Study 2 Modelling and simulation study using population target-mediated drug disposition (TMDD) to determine (adult) and paediatric doses.
Other Studies	0	Not applicable
Other Measures	1	Extrapolation Plan Studies 1 and 2 are part of an extrapolation plan covering the paediatric population from 6 years to less than 18 years of age, as agreed by the Regulatory Agency.

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:	30/12/2026

Deferral of one or more studies contained in the paediatric investigation plan:	Yes