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

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Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral and grant a waiver

MHRA-101185-PIP01-23

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 20/11/2023 14:50 GMT an application for a Paediatric Investigation Plan

The procedure started on 10/01/2025 11:31 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-101185-PIP01-23

Of 12/03/2025 09:31 GMT

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

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

ANNEX I

1. Waiver

1.1 Condition:

Treatment of Myasthenia gravis

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of Myasthenia gravis

2.2 Indication(s) targeted by the PIP:

Treatment of paediatric patients with acetylcholine receptor (AChR) antibody-positive generalized myasthenia gravis

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

From 6 years to less than 18 years of age

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 efficay 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+)
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:	31/12/2030
Deferral of one or more studies contained in the paediatric investigation plan:	Yes