

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-101264-PIP01-23

Scope of the Application

Active Substance(s)

cemdisiran

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

Regeneron Ireland DAC

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Regeneron Ireland DAC submitted to the licensing authority on 11/12/2023 13:10 GMT an application for a Paediatric Investigation Plan

The procedure started on 12/02/2024 11:43 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-101264-PIP01-23

Of 24/04/2024 13:13 BST

On the adopted decision for cemdisiran (MHRA-101264-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 cemdisiran, Solution for injection ,
SUBCUTANEOUS USE .

This decision is addressed to Regeneron Ireland DAC, One Warrington Place, Dublin, IRELAND, D02
HH27

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 of age 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).

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 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, uncontrolled trial to evaluate pharmacokinetics, safety and descriptive efficacy of cemdisiran and pozelimab in combination, in children from 6 years to less than 18 years of age with symptomatic generalised juvenile myasthenia gravis (gJMG).
Extrapolation, Modeling & Simulation Studies	0	Not applicable.
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:	31/12/2030
Deferral of one or more studies contained in the paediatric investigation plan:	Yes

