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 change(s) to the agreed paediatric investigation plan (MHRA-100066-PIP01-21-M01)

MHRA-100066-PIP01-21-M02

Scope of the Application

Active Substance(s)

EFGARTIGIMOD ALFA

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

argenx BV

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, argenx BV submitted to the licensing authority on 09/10/2024 14:53 BST an application for a Modification

The procedure started on 02/12/2024 07:46 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 accept change(s) to the agreed paediatric investigation plan.

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-100066-PIP01-21-M02

Of 02/04/2025 07:10 BST

On the adopted decision for EFGARTIGIMOD ALFA (MHRA-100066-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 EFGARTIGIMOD ALFA, Solution for injection , SUBCUTANEOUS USE .

This decision is addressed to argenx BV, Industriepark Zwijnaarde 7, Gent, BELGIUM, 9052

ANNEX I

1. Waiver

1.1 Condition:

Treatment of myasthenia gravis (MG) 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 Route(s) of administration: SUBCUTANEOUS USE Reason for granting waiver: on the grounds that the specific medicinal product is likely to be unsafe

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of myasthenia gravis (MG)

2.2 Indication(s) targeted by the PIP:

Treatment of patients with 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

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, uncontrolled study to evaluate pharmacokinetics and pharmacodynamics of subcutaneous efgartigimod alfa in children from 2 years to less than 18 years of age with generalised myasthenia gravis.
Extrapolation, Modeling & Simulation Studies	2	Study 2 A modelling and simulation study to support the use of subcutaneous efgartigimod alfa for the treatment of generalised myasthenia gravis in children from 2 years to less than 18 years of age. Study 3 An extrapolation study to support the use of subcutaneous efgartigimod alfa for the treatment of generalised myasthenia gravis in children from 2 years to less than 18 years of age.
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:	31/03/2028
Deferral of one or more studies contained in the paediatric investigation plan:	Yes