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 and to the deferral

MHRA-100743-PIP01-22-M01

Scope of the Application

Active Substance(s)

efgartigimod alfa

Condition(s)

Treatment of generalised myasthenia gravis

Pharmaceutical Form(s)

Concentrate for solution for infusion

Route(s) of Administration

INTRAVENOUS 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 07/11/2022 08:27 GMT an application for a Modification

The procedure started on 28/11/2022 12:50 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 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-100743-PIP01-22-M01

Of 05/12/2022 09:24 GMT

On the adopted decision for efgartigimod alfa (MHRA-100743-PIP01-22-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 Modification for efgartigimod alfa, Concentrate for solution for infusion , INTRAVENOUS USE .

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

ANNEX I

1. Waiver

1.1 Condition:

Treatment of generalised 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): Concentrate for solution for infusion Route(s) of administration: INTRAVENOUS 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 generalised 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 2 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Concentrate for solution for infusion

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 trial to evaluate pharmacokinetics, pharmacodynamics, safety and activity of 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 Modelling and simulation study to support the use of efgartigimod alfa for the treatment of generalised myasthenia gravis in children from 2 years to less than 18 years of age. Study 3 Extrapolation study to support the use of 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:	31/12/2025
Deferral of one or more studies contained in the paediatric investigation plan:	Yes