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

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

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

MHRA-100513-PIP01-22

Scope of the Application

Active Substance(s)

efgartigimod alfa

Condition(s)

Treatment of dermatomyositis, Treatment of immune-mediated necrotizing myopathy , Treatment of polymyositis (including antisynthetase syndrome)

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 04/07/2022 11:06 BST an application for a Paediatric Investigation Plan

The procedure started on 23/01/2023 07:28 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-100513-PIP01-22

Of 15/06/2023 18:08 BST

On the adopted decision for efgartigimod alfa (MHRA-100513-PIP01-22) 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 efgartigimod alfa, Solution for injection , SUBCUTANEOUS USE .

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

ANNEX I

1. Waiver

1.1 Condition:

Condition1: Treatment of dermatomyositis 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. Condition 2: Treatment of immune-mediated necrotizing myopathy The waiver applies / applied to: Paediatric Subset(s): All subsets of the paediatric population from birth to less than 18 years of age Pharmaceutical form(s): Solution for injection Route(s) of administration: SUBCUTANEOUS USE Reason for granting waiver: on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s). Condition 3: Treatment of polymyositis (including antisynthetase syndrome) The waiver applies / applied to: Paediatric Subset(s): All subsets of the paediatric population from birth to less than 18 years of age Pharmaceutical form(s): Solution for injection Route(s) of administration: SUBCUTANEOUS USE Reason for granting waiver: on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of dermatomyositis

2.2 Indication(s) targeted by the PIP:

Treatment of patients with juvenile dermatomyositis aged 2 years to less than 18 years

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	0	Not applicable.
Extrapolation, Modeling & Simulation Studies	2	Study 1 Modelling and simulation study to evaluate the use of the product in the treatment of juvenile dermatomyositis (JDM) in children from 2 years to less than 18 years of age. Extrapolation Plan PIP Study 1 is part of an extrapolation plan covering the population from 2 years to less than 18 years, as agreed by the Regulatory Agency.
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:	31/12/2028
Deferral of one or more studies contained in the paediatric investigation plan:	Yes