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

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

grant a product specific waiver

MHRA-100917-PIP01-23

Scope of the Application

Active Substance(s)

efgartigimod alfa

Condition(s)

Treatment of bullous pemphigoid

Pharmaceutical Form(s)

Solution for injection, Concentrate for solution for infusion

Route(s) of Administration

SUBCUTANEOUS USE; 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 30/03/2023 11:05 BST an application for a Waiver

The procedure started on 02/08/2023 16:56 BST

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 grant a product specific 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-100917-PIP01-23

Of 14/08/2023 17:00 BST

On the adopted decision for efgartigimod alfa (MHRA-100917-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:

Granting a waiver in all age groups for the listed condition(s)

This decision applies to a Waiver for efgartigimod alfa, Solution for injection, Concentrate for solution for infusion , SUBCUTANEOUS USE; INTRAVENOUS USE .

This decision is addressed to argenx BV, Industriepark Zwijnaarde, Zwijnaarde, BELGIUM, 9210

ANNEX I

1. Waiver

1.1 Condition:

Treatment of bullous pemphigoid 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 Concentrate for solution for infusion Route(s) of administration: SUBCUTANEOUS USE INTRAVENOUS USE Reason for granting waiver: - For the paediatric population from birth to less than 2 years of age: on the grounds that the specific medicinal product is likely to be unsafe. - For the paediatric population from 2 years to less than 18 years of age: on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Not Applicable

2.2 Indication(s) targeted by the PIP:

Not Applicable

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

Not Applicable

2.4 Pharmaceutical Form(s):

Not Applicable

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures		
Non-Clinical Studies		
Clinical Studies		
Extrapolation, Modeling & Simulation Studies		
Other Studies		
Other Measures		

3. Follow-up, completion and deferral of a PIP:

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	
Date of completion of the paediatric investigation plan:	
Deferral of one or more studies contained in the paediatric investigation plan:	