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-100050-PIP01-21) and to the deferral.

MHRA-100050-PIP01-21-M01

Scope of the Application

Active Substance(s)

efgartigimod alfa

Condition(s)

Treatment of immune thrombocytopenia.

Pharmaceutical Form(s)

Concentrate for solution for infusion, Solution for injection

Route(s) of Administration

INTRAVENOUS USE; 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 20/02/2023 16:02 GMT an application for a Modification

The procedure started on 05/06/2023 20:21 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 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-100050-PIP01-21-M01

Of 21/07/2023 16:26 BST

On the adopted decision for efgartigimod alfa (MHRA-100050-PIP01-21-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, Solution for injection , INTRAVENOUS, SUBCUTANEOUS USE .

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

ANNEX I

1. Waiver

1.1 Condition:

Treatment of Immune Thrombocytopenia. 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. Solution for injection. Route(s) of administration: INTRAVENOUS USE. SUBCUTANEOUS USE. Reason for granting waiver: on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of Immune Thrombocytopenia.

2.2 Indication(s) targeted by the PIP:

Treatment of patients with chronic immune thrombocytopenia (or idiopathic immune thrombocytopenia, ITP) who have had insufficient response to a previous treatment.

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. 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 Two part pharmacokinetic and pharmacodynamic study of efgartigimod in paediatric patients from 2 years to less than 18 years of age with ITP.
Extrapolation, Modeling & Simulation Studies	1	Study 2 Adult derived pharmacokinetics (PK)/ Pharmacodynamic (PD) model supplemented with data from paediatric dosing in patients with myasthenia gravis for dose predictions in children with ITP.
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:	No
Date of completion of the paediatric investigation plan:	30/09/2031
Deferral of one or more studies contained in the paediatric investigation plan:	Yes