

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-101943-PIP01-25

Scope of the Application

Active Substance(s)

Humanised IgG1 monoclonal antibody against muscle specific kinase; Adimanebart

Condition(s)

Treatment of spinal muscular atrophy

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 16/05/2025 16:56 BST an application for a Paediatric Investigation Plan

The procedure started on 10/10/2025 15:03 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 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-101943-PIP01-25

Of 25/08/2026 10:46 BST

On the adopted decision for Adimanebart (MHRA-101943-PIP01-25) 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 Adimanebart, Solution for injection Concentrate for solution for infusion , Subcutaneous use Intravenous use .

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

ANNEX I

1. Waiver

1.1 Condition:

Treatment of spinal muscular atrophy The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 months 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: 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 spinal muscular atrophy

2.2 Indication(s) targeted by the PIP:

Treatment of spinal muscular atrophy

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

The paediatric population from 2 months to less than 18 years of age.

2.4 Pharmaceutical Form(s):

Solution for injection Concentrate for solution for infusion

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	1	Study 1 (ARGX-NC-369) Pre- and post-weaning juvenile toxicity study in Wistar Han IGS rats.
Clinical Studies	3	Study 2 (ARGX-119-24-SMA-2001) Double-blinded, randomised, multiple dose, placebo-controlled study to evaluate the safety, tolerability, efficacy, pharmacokinetics, and immunogenicity of intravenously administered adimanebart in addition to a survival motor neuron gene (SMN)-upregulating therapy in paediatric participants from 5 years to less than 18 years of age with spinal muscular atrophy (SMA). Study 3 Double-blinded, randomised, placebo-controlled study to evaluate the efficacy, safety, PK, and immunogenicity of adimanebart co-formulated with recombinant human hyaluronidase PH20 (rHuPH20) and administered subcutaneously (adimanebart PH20 SC) in addition to an SMN-upregulating therapy in paediatric participants from 2 years to less than 18 years of age (and adults) with SMA. Study 4 Open-label study to evaluate PK, safety, and tolerability of adimanebart PH20 SC in addition to an SMN-upregulating therapy in paediatric

		participants from 2 months to less than 2 years of age with SMA.
Extrapolation, Modeling & Simulation Studies	3	Study 5 Modelling and simulation study to support dose selection of adimanebart for the treatment of spinal muscular atrophy (SMA) in children from 2 years to less than 18 years of age. Study 6 Modelling and simulation study to support adimanebart dose selection for the treatment of SMA in children from 2 months to less than 2 years of age. Extrapolation plan Studies 2, 3, and 4 are part of the extrapolation plan of efficacy data from paediatric participants aged 2 years to less than 18 years of age and adults to the paediatric population aged 2 months to less than 2 years with the condition of spinal muscular atrophy.
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:	31/03/2033
Deferral of one or more studies contained in the paediatric investigation plan:	Yes