



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/303410/2023

European Medicines Agency decision P/0252/2023

of 14 July 2023

on the acceptance of a modification of an agreed paediatric investigation plan for efgartigimod alfa (Vyvgart), (EMA-002597-PIP04-21-M01) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

Disclaimer

This decision does not constitute entitlement to the rewards and incentives referred to in Title V of Regulation (EC) No 1901/2006.

Only the English text is authentic.



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The European Medicines Agency,

Having regard to the Treaty on the Functioning of the European Union,

Having regard to Regulation (EC) No 1901/2006 of the European Parliament and of the Council of 12 December 2006 on medicinal products for paediatric use and amending Regulation (EEC) No 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004¹,

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency²,

Having regard to the European Medicines Agency's decision P/0078/2022 issued on 11 March 2022,

Having regard to the application submitted by argenx BV on 17 February 2023 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 26 May 2023, in accordance with Article 22 of Regulation (EC) No 1901/2006,

Having regard to Article 25 of Regulation (EC) No 1901/2006,

Whereas:

- (1) The Paediatric Committee of the European Medicines Agency has given an opinion on the acceptance of changes to the agreed paediatric investigation plan and to the deferral.
- (2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan, including changes to the deferral.

¹ OJ L 378, 27.12.2006, p.1, as amended.

² OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for efgartigimod alfa (Vyvgart), solution for injection, concentrate for solution for infusion, subcutaneous use, intravenous use, including changes to the deferral, are hereby accepted in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

Article 2

This decision covers all conditions, indications, pharmaceutical forms, routes of administration, measures, timelines, waivers and deferrals, as agreed in the decision P/0097/2020 issued on 18/3/2020, including subsequent modifications thereof.

Article 3

This decision is addressed to argenx BV, Industriepark Zwijnaarde 7, B-9052 – Zwijnaarde, Belgium.



EUROPEAN MEDICINES AGENCY
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EMA/PDCO/101667/2023 Corr¹
Amsterdam, 26 May 2023

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA-002597-PIP04-21-M01

Scope of the application

Active substance(s):

Efgartigimod alfa

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of immune thrombocytopenia

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 opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, argenx BV submitted to the European Medicines Agency on 17 February 2023 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/0078/2022 issued on 11 March 2022.

¹ 9 June 2023.



The application for modification proposed changes to the agreed paediatric investigation plan and to the deferral.

The procedure started on 27 March 2023.

Scope of the modification

Some timelines of the Paediatric Investigation Plan have been modified.

Opinion

1. The Paediatric Committee, having assessed the application in accordance with Article 22 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:
 - to agree to changes to the paediatric investigation plan and to the deferral in the scope set out in the Annex I of this opinion.

The Paediatric Committee member of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the paediatric investigation plan and the subset(s) of the paediatric population and condition(s) covered by the waiver are set out in the Annex I.

This opinion is forwarded to the applicant and the Executive Director of the European Medicines Agency, together with its annexes and appendix.

Annex I

The subset(s) of the paediatric population and condition(s) covered by the waiver and the measures and timelines of the agreed paediatric investigation plan (PIP)

1. Waiver

1.1. Condition:

Treatment of immune thrombocytopenia

The waiver applies to:

- the paediatric population from birth to less than 2 years of age;
- solution for injection, subcutaneous use; concentrate for solution for infusion, intravenous use;
- 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:

Treatment of immune thrombocytopenia

2.1.1. Indication(s) targeted by the PIP

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

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From 2 years to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Solution for injection

Concentrate for solution for infusion

2.1.4. Measures

Area	Description
Quality-related studies	Not applicable.
Non-clinical studies	Not applicable.
Clinical studies	Two-part pharmacokinetic and pharmacodynamic study of efgartigimod in paediatric patients from 2 years to less than 18 years of age with ITP.
Extrapolation, modelling and simulation studies	Adult derived pharmacokinetic (PK)/pharmacodynamic (PD) model supplemented with data from paediatric dosing in patients with myasthenia gravis for dose predictions in children with ITP.
Other studies	Not applicable.
Other measures	Not applicable.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By June 2031
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

Condition(s) and authorised indication(s)

1. Treatment of Myasthenia Gravis

Authorised indication(s): Vyvgart is indicated as an add-on to standard therapy for the treatment of adult patients with generalised Myasthenia Gravis (gMG) who are anti-acetylcholine receptor (AChR) antibody positive.

- Invented name(s): Vyvgart.
- Authorised pharmaceutical form(s): concentrate for solution for infusion.
- Authorised route(s) of administration: IV infusion.
- Authorised via centralised procedure.