EMADOC-1700519818-1860426

European Medicines Agency decision

EMA/PE/0000227425

of 28 January 2025

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

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 726/2004[1],

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 Agency[2],

Having regard to the European Medicines Agency's decision P/0078/2022 issued on 11 March 2022 and the decision P/0252/2023 issued on 14 July 2023,

Having regard to the application submitted by Argenx on 08 September 2024 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 13 December 2024, 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 and to the waiver.

(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 and to the waiver.

[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), including changes to the deferral and to the waiver, 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 is addressed to Argenx, Industriepark-Zwijnaarde 7, 9052 - Gent, Belgium.

EMADOC-1700519818-1697151
Amsterdam, 13 December 2024

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMA/PE/0000227425

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):

Concentrate for solution for infusion

Route(s) of administration:

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 8 September 2024 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 and the decision P/0252/2023 issued on 14 July 2023.

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

The procedure started on 14 October 2024.

Scope of the modification

Some measures and timelines of the Paediatric Investigation Plan have been modified. A waiver for a new subset has been added. Pharmaceutical form and route of administration were amended.

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 and to the waiver in the scope set out in the Annex I of this opinion.

   The Paediatric Committee members of Liechtenstein and Norway agree 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 12 years of age;
- 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 12 years to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Concentrate for solution for infusion

2.1.4. Measures

Area	**Description**
Quality-related studies	Not applicable.
Non-clinical studies	Not applicable.
Clinical studies	Pharmacokinetic and pharmacodynamic study of efgartigimod i.v in paediatric patients from 12 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 2030
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 Myastenia 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.