



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMADOC-1700519818-2235588

European Medicines Agency decision

EMA/PE/0000245403

of 11 July 2025

on the acceptance of a modification of an agreed paediatric investigation plan for vadadustat (Vafseo)
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 vadadustat (Vafseo) 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 726/2004¹,

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/0035/2017 issued on 31 January 2017, the decision P/0074/2020 issued on 21 March 2020, the decision P/0401/2020 issued on 23 October 2020, the decision P/0024/2022 issued on 31 January 2022, the decision P/0244/2022 issued on 8 July 2022, and the decision P/0290/2024 issued on 16 August 2024,

Having regard to the application submitted by Medice Arzneimittel Puetter GmbH & Co. KG on 14 February 2025 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 23 May 2025, 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 vadadustat (Vafseo), 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 is addressed to Medice Arzneimittel Puetter GmbH & Co. KG, Kuhlweg 37, Kuhlo, 58638 – Iserlohn, North Rhine-Westphalia, Germany.



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMADOC-1700519818-1931308

Amsterdam, 23 May 2025

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA/PE/0000245403

Scope of the application

Active substance(s):

Vadadustat

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of anaemia due to chronic disorders

Pharmaceutical form(s):

Film-coated tablet

Age-appropriate oral formulation

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Medice Arzneimittel Puetter GmbH & Co. KG

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Medice Arzneimittel Puetter GmbH & Co. KG submitted to the European Medicines Agency on 14 February 2025 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/0035/2017 issued on 31 January 2017, the decision P/0074/2020 issued on 21 March 2020, the decision P/0401/2020 issued on 23 October 2020, the decision P/0024/2022 issued on 31 January 2022, the decision P/0244/2022 issued on 8 July 2022, and the decision P/0290/2024 issued on 16 August 2024.



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

The procedure started on 24 March 2025.

Scope of the modification

Some measures and 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 anaemia due to chronic disorders

The waiver applies to:

- the paediatric population from birth to less than 4 months of age;
- tablet and age-appropriate oral formulation, oral use;
- 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

Treatment of anaemia due to chronic disorders

2.1.1. Indication(s) targeted by the PIP

Treatment of anaemia secondary to chronic kidney disease

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

From 4 months to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Tablet

Age-appropriate oral formulation

2.1.4. Measures

Area	Description
Quality-related studies	Study 1 Development of an age-appropriate oral dosage form.
Non-clinical studies	Study 2 Pilot oral gavage study in juvenile rats Study 3 <i>This study was deleted as a result of procedure EMEA-001944-PIP01-16-M01.</i>

	<p>Study 9</p> <p><i>(added in procedure EMEA-001944-PIP01-16-M02)</i></p> <p>Definitive study in juvenile rats to determine the potential toxicity of vadadustat when administered daily to juvenile rats and to investigate the progression and/or reversibility of any treatment-related effects after a 6-week treatment free (recovery) period.</p>
Clinical studies	<p>Study 4</p> <p>Open-label, single-arm, externally controlled trial to evaluate the activity, safety, tolerability, PK and PD of oral vadadustat for the correction of anaemia in children from 4 months to less than 18 years of age with anaemia secondary to chronic kidney disease (CKD).</p> <p>Study 5</p> <p>Medical record review study to assess the activity and safety of erythropoiesis-stimulating agent (ESA) treatment to correct and maintain haemoglobin levels in children from 4 months to less than 18 years of age with anaemia secondary to dialysis dependent CKD and pretreated or naïve concerning ESA treatment.</p> <p>Study 6</p> <p><i>This study was deleted as a result of procedure EMA/PE/0000245403</i></p> <p>Study 7</p> <p><i>This study was deleted as a result of procedure EMA/PE/0000245403</i></p>
Extrapolation, modelling and simulation studies	<p>Study 8</p> <p>Modelling and simulation study to develop PK/PD simulations for the prediction of exposure and selection of doses to be used in children from 4 months to less than 18 years of age with anaemia secondary to CKD naïve to ESA treatment or receiving ESA treatment.</p>
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:	Yes
Date of completion of the paediatric investigation plan:	By January 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 anaemia due to chronic disorders

Authorised indication(s):

- Vafseo is indicated for the treatment of symptomatic anaemia associated with chronic kidney disease (CKD) in adults on chronic maintenance dialysis.
 - Invented name(s): Vafseo
 - Authorised pharmaceutical form(s): Film-coated tablet
 - Authorised route(s) of administration: Oral use
 - Authorised via centralised procedure