EMA/320605/2024

European Medicines Agency decision

P/0236/2024

of 18 July 2024

on the acceptance of a modification of an agreed paediatric investigation plan for vonicog alfa (Veyvondi), (EMEA-001164-PIP01-11-M08) 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 726/2004[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/0091/2012 issued on 29 May 2012, the decision P/0214/2015 issued on 2 October 2015, the decision P/0225/2018 issued on 17 July 2018, the decision P/0394/2019 issued on 4 December 2019, the decision P/0463/2020 issued on 4 December 2020, the decision P/0491/2021 issued on 3 December 2021, the decision P/0363/2022 issued on 7 September 2022 and the decision P/0416/2023 issued on 25 October 2023,

Having regard to the application submitted by Baxalta Innovations GmBH on 26 February 2024 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 31 May 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.

(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 vonicog alfa (Veyvondi), powder and solvent for solution for injection, 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 is addressed to Baxalta Innovations GmBH, Industriestrasse 67, 1221 – Vienna, Austria.

EMA/PDCO/93794/2024
Amsterdam, 31 May 2024

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

EMEA-001164-PIP01-11-M08

Scope of the application

Active substance(s):

Vonicog alfa

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of von Willebrand disease

Pharmaceutical form(s):

Powder and solvent for solution for injection

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Baxalta Innovations GmbH

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Baxalta Innovations GmbH submitted to the European Medicines Agency on 26 February 2024 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0091/2012 issued on 29 May 2012, the decision P/0214/2015 issued on 2 October 2015, the decision P/0225/2018 issued on 17 July 2018, the decision P/0394/2019 issued on 4 December 2019, the decision P/0463/2020 issued on 4 December 2020, the decision P/0491/2021 issued on 3 December 2021, the decision P/0363/2022 issued on 7 September 2022 and the decision P/0416/2023 issued on 25 October 2023.

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

The procedure started on 2 April 2024.

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.

2. The measures and timelines of the paediatric investigation plan 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

Not applicable

2. Paediatric Investigation Plan

2.1. Condition

Treatment of von Willebrand disease (VWD)

2.1.1. Indication(s) targeted by the PIP

Prevention and treatment of bleeding episodes and for surgical and invasive procedures in paediatric patients (less than 18 years of age) with von Willebrand disease.

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

From birth to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Powder and solvent for solution for injection

2.1.4. Measures

Area	Description
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	**Study 1 (071102)- Surgery arm** Open-label study to assess the efficacy and safety of vonicog alfa (rVWF) in elective and emergency surgeries in children diagnosed with severe hereditary VWD and to determine the pharmacokinetics (PK) of rVWF. **Study 2** *Study deleted in procedure number EMEA-001164-PIP01-11-M01.* **Study 3 (TAK-577-3001)** *Study added in procedure number EMEA-001164-PIP01-11-M04.* Open-label, uncontrolled study to assess the efficacy and safety vonicog alfa for prophylaxis to prevent or reduce the frequency and/or severity of bleeding episodes in children with von Willebrand disease. **Study 5 (071102) – On-demand arm** *Study added in procedure number EMEA-001164-PIP01-11-M08.* Open-label study to assess the safety and efficacy of vonicog alfa (rVWF), with or without rhFVIII product in the treatment of bleeding episodes in children diagnosed with severe hereditary VWD, and to determine the PK of rVWF.

Extrapolation, modelling and simulation studies	**Study 4** *Study added in procedure number EMEA-001164-PIP01-11-M07.* Population PK and PK/PD model to assess the similarity of exposure and PK/PD between adults and children, and to support the use of vonicog alfa across all paediatric age cohorts.
Other studies	Not applicable
Other measures	Not applicable

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By November 2027
Deferral for one or more studies 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 von Willebrand disease

Authorised indication(s):

- Prevention and treatment of haemorrhage or surgical bleeding in adults (age 18 years and older) with von Willebrand disease (VWD), when desmopressin (DDAVP) treatment alone is ineffective or contraindicated. Veyvondi should not be used in the treatment of haemophilia Invented name(s): Veyvondi
- Authorised pharmaceutical form(s): powder and solvent for solution for injection
- Authorised route(s) of administration: intravenous use
- Authorised via centralised procedure