EMA/697750/2022

European Medicines Agency decision

P/0363/2022

of 7 September 2022

on the acceptance of a modification of an agreed paediatric investigation plan for vonicog alfa (Veyvondi), (EMEA-001164-PIP01-11-M06) 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, decision P/0214/2015 issued on 2 October 2015, decision P/0225/2018 issued on 17 July 2018, decision P/0394/2019 issued on 4 December 2019, decision P/0463/2020 issued on 4 December 2020, and decision P/0491/2021 issued on 3 December,

Having regard to the application submitted by Baxalta Innovations GmBH on 22 April 2022 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 22 July 2022, 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.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

[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, 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/243324/2022
Amsterdam, 22 July 2022

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

EMEA-001164-PIP01-11-M06

Scope of the application

Active substance(s):

Vonicog alfa

Invented name:

Veyvondi

Condition(s):

Treatment of von Willebrand disease

Authorised indication(s):

See Annex II

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

Information about the authorised medicinal product:

See Annex II

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 22 April 2022 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, decision P/0214/2015 issued on 2 October 2015,

decision P/0225/2018 issued on 17 July 2018, decision P/0394/2019 issued on 4 December 2019, decision P/0463/2020 issued on 4 December 2020, and decision P/0491/2021 issued on 3 December 2021.

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

The procedure started on 23 May 2022.

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 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 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) Open-label study to assess the safety and efficacy of vonicog alfa (rVWF), with or without ADVATE, in the treatment of bleeding episodes, the efficacy and safety of 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.
Extrapolation, modelling and simulation studies	Not applicable

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 December 2025
Deferral for one or more studies contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

1. Treatment of von Willebrand disease

Authorised indication(s):

- VEYVONDI is indicated in adults (age 18 and older) with von Willebrand Disease (VWD), when desmopressin (DDAVP) treatment alone is ineffective or not indicated for the
  - Treatment of haemorrhage and surgical bleeding.
  - Prevention of surgical bleeding.

Authorised pharmaceutical form(s):

Powder and solvent for solution for injection

Authorised route(s) of administration:

Intravenous use