EMA/484938/2024

European Medicines Agency decision

P/0383/2024

of 24 October 2024

on the acceptance of a modification of an agreed paediatric investigation plan for recombinant human A disintegrin and metalloprotease with thrombospondin type-1 Motifs 13 (Adzynma), (EMEA-001160-PIP01-11-M05) 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. P/0383/2024

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/0048/2012 issued on 29 February 2012, the decision P/0324/2017 issued on 31 October 2017, the decision P/0414/2021 issued on 29 October 2021 and the decision P/0489/2022 issued on 2 December 2022,

Having regard to the application submitted by Takeda Pharmaceuticals International AG on 3 June 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 6 September 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 recombinant human A disintegrin and metalloprotease with thrombospondin type-1 Motifs 13 (Adzynma), 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 Takeda Pharmaceuticals International AG, Block 2 Miesian Plaza, 50-58, Baggot Street Lower, D02 HW68 – Dublin, Ireland.

EMA/PDCO/281545/2024
Amsterdam, 6 September 2024

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

EMEA-001160-PIP01-11-M05

Scope of the application

Active substance(s):

Recombinant human A disintegrin and metalloprotease with thrombospondin type-1 Motifs 13

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of thrombotic thrombocytopenic purpura

Pharmaceutical form(s):

Powder and solvent for solution for injection

Route(s) of administration:

Intravenous use.

Name/corporate name of the PIP applicant:

Takeda Pharmaceuticals International AG

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Takeda Pharmaceuticals International AG submitted to the European Medicines Agency on 3 June 2024 an application for modification of the agreed paediatric investigation plan as set out in the European Medicines Agency's decision P/0048/2012 issued on 29 February 2012, the decision P/0324/2017 issued on 31 October 2017, the decision P/0414/2021 issued on 29 October 2021 and the decision P/0489/2022 issued on 2 December 2022.

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

The procedure started on 8 July 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.

   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 thrombotic thrombocytopenic purpura (TTP)

2.1.1. Indication targeted by the PIP

Treatment of hereditary thrombotic thrombocytopenic purpura (TTP)

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	**Study 1** Development of a paediatric vial
Non-clinical studies	Not applicable
Clinical studies	**Study 2** A Phase 1, prospective, uncontrolled, open-label, multicentre, dose-escalation study evaluating the safety, including immunogenicity, and pharmacokinetics of BAX 930 (rADAMTS13) in congenital thrombotic thrombocytopenic purpura (cTTP) in children from 12 years of age to less than 18 years of age (and in adults). **Study 3** Prospective, randomised, active-controlled, open-label, multicenter, 2-period crossover study (rADAMTS13 versus standard of care [SoC], 1:1 randomisation, Periods 1 and 2), followed by a single arm continuation period (rADAMTS13, Period 3) to evaluate the safety and efficacy of rADAMTS13 in the prophylactic and on-demand treatment of patients with severe congenital thrombotic thrombocytopenic purpura cTTP in children from birth to less than 18 years of age (and in adults).

3. Follow-up, completion and deferral of PIP

Concerns on potential long term efficacy and safety issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By May 2024
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 thrombotic thrombocytopenic purpura

Authorised indication(s):

- Adzynma is an enzyme replacement therapy (ERT) indicated for the treatment of ADAMTS13 deficiency in children and adult patients with congenital thrombotic thrombocytopenic purpura (cTTP). Adzynma can be used for all age groups.
  - Invented name(s): Adzynma
  - Authorised pharmaceutical form(s): Powder and solvent for solution for injection.
  - Authorised route(s) of administration: For intravenous use after reconstitution only.
  - Authorised via the centralised procedure.