

EMA/671585/2017

European Medicines Agency decision

P/0324/2017

of 31 October 2017

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 (EMEA-001160-PIP01-11-M01) 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 726/2004¹,

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency²,

Having regard to the European Medicines Agency's decision P/0048/2012 issued on 29 February 2012,

Having regard to the application submitted by Baxalta Innovations GmbH on 22 June 2017 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 15 September 2017, 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.

² OJ L 136, 30.4.2004, p. 1.

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, 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/423458/2017
London, 15 September 2017

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA-001160-PIP01-11-M01

Scope of the application

Active substance(s):

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

Condition(s):

Treatment of thrombotic thrombocytopenic purpura (TTP)

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 22 June 2017 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 application for modification proposed changes to the agreed paediatric investigation plan.

The procedure started on 18 July 2017.

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 in the scope set out in the Annex I of this opinion.

The Norwegian Paediatric Committee member 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 annex 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 infusion

2.1.4. Measures

Area	Number of studies	Description
Quality-related studies	1	Study 1: Development of a paediatric vial.
Non-clinical studies	0	Not applicable.
Clinical studies	2	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 December 2021
Deferral for one or more studies contained in the paediatric investigation plan:	No