EMA/PDCO/54526/2012

European Medicines Agency decision

P/0048/2012

of 29 February 2012

on the agreement of a paediatric investigation plan for recombinant human A Disintegrin and Metalloprotease with Thrombospind Type-1 Motifs 13 (EMEA-001160-PIP01-11) 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 Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency[2],

Having regard to the application submitted by Baxter Innovations GmbH on 10 May 2011 under Article 16(1) of Regulation (EC) No 1901/2006,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 13 January 2012, in accordance with Article 18 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 agreement of a paediatric investigation plan.

(2) It is therefore appropriate to adopt a decision agreeing a 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

A paediatric investigation plan for recombinant human A Disintegrin and Metalloprotease with Thrombospind Type-1 Motifs 13, powder and solvent for solution for infusion, intravenous use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

Article 2

This decision is addressed to Baxter Innovations GmbH, Industriestrasse 67, 1221 – Vienna, Austria.

Done at London, 29 February 2012

For the European Medicines Agency
Guido Rasi
Executive Director
(Signature on file)

EMA/PDCO/549592/2011

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan

EMEA-001160-PIP01-11

Scope of the application

Active substance(s):

Recombinant human A Disintegrin and Metalloprotease with Thrombospind Type-1 Motifs 13

Condition(s):

Treatment of thrombotic thrombocytopenic purpura (TTP)

Pharmaceutical form(s):

Powder and solvent for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Baxter Innovations GmbH

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Baxter Innovations GmbH submitted for agreement to the European Medicines Agency on 10 May 2011 an application for a paediatric investigation plan for the above mentioned medicinal product.

The procedure started on 15 June 2011.

Supplementary information was provided by the applicant on 14 October 2011.

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Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree the paediatric investigation plan in accordance with Article 18 of said Regulation.

   The Icelandic and the Norwegian Paediatric Committee members agree with the above-mentioned recommendation of the Paediatric Committee.

   The measures and timelines of the agreed 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(es) and appendix.

London, 13 January 2012

On behalf of the Paediatric Committee
Dr Daniel Brasseur, Chairman
(Signature on file)

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

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. Studies

Area	**Number of studies**	**Description**
Quality	Total number of quality studies	**Study 1:** Development of a paediatric vial.
Non-clinical	Total number of studies	Not applicable.
Clinical	Total number of studies	**Study 2:** Open-label, multicentre, single dose, dose escalation trial to evaluate pharmacokinetics, safety, immunogenicity of Recombinant human A Disintegrin and Metalloprotease with Thrombospind Type-1 Motifs 13 in children from 12 years of age to less than 18 years of age (and in adults) with severe hereditary ADAMTS13 deficiency. **Study 3:** Open-label, randomised multicentre, active controlled, trial to evaluate safety, efficacy, pharmacokinetics immunogenicity of Recombinant human A Disintegrin and Metalloprotease with Thrombospind Type-1 Motifs 13 compared to Fresh Frozen Plasma in children from Birth to less than 18 years of age (and in adults) with with severe hereditary ADAMTS13 deficiency.

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 June 2016
Deferral for one or more studies contained in the paediatric investigation plan:	No