EMA/13274/2011

European Medicines Agency decision

P/28/2011

of 28 January 2011

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the refusal of a waiver for pegloticase (EMEA-000293-PIP02-10) 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 Savient Pharmaceuticals, Inc. on 12 April 2010 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 10 December 2010, in accordance with Article 18 of Regulation (EC) No 1901/2006, and Article 21 of said Regulation and Article 13 of said Regulation,

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 and on the granting of a deferral and on the refusal of a waiver.

(2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.

(3) It is therefore appropriate to adopt a decision granting a deferral.

(4) It is therefore appropriate to adopt a decision refusing a waiver.

[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 pegloticase (EMEA-000293-PIP02-10), 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

A deferral for pegloticase (EMEA-000293-PIP02-10), 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 granted.

Article 3

A waiver for pegloticase (EMEA-000293-PIP02-10), 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 refused.

Article 4

This decision is addressed to Savient Pharmaceuticals, Inc., One Tower Center Blvd, 14th Floor, East Brunswick NJ 08816, United States.

Done at London, 28 January 2011

For the European Medicines Agency
Andreas Pott
Acting Executive Director

(Signature on file)

EMA/PDCO/705768/2010

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a deferral and on the refusal of a waiver

EMEA-000293-PIP02-10

Scope of the application

Active substance(s):

Pegloticase

Condition(s):

Treatment of hyperuricaemia

Prevention of hyperuricaemia

Pharmaceutical form(s):

Solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Savient Pharmaceuticals, Inc.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Savient Pharmaceuticals, Inc. submitted for agreement to the European Medicines Agency on 12 April 2010 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 20 May 2010.

Supplementary information was provided by the applicant on 4 October 2010.

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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,
   - to grant a deferral in accordance with Article 21 of said Regulation,
   - to refuse the granting of a waiver in accordance with Article 13 of said Regulation, for some of the subsets of the paediatric population and some of the above mentioned conditions as it does not meet the grounds detailed in Article 11(1) of said Regulation.

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

2. 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, 10 December 2010

On behalf of the Paediatric Committee
Dr Daniel Brasseur, Chairman

(Signature on file)

Annex I

The measures and timelines of the agreed Paediatric Investigation Plan

1. Waiver

Not applicable.

2. Paediatric Investigation Plan

2.1. Condition: Treatment of hyperuricaemia

2.1.1. Indication(s) targeted by the PIP

Treatment and/or prevention of hyperuricaemia related to Tumour Lysis Syndrome

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)

Solution for infusion, intravenous use.

Age-appropriate strength to be developed: A vial containing less than 10-fold the lowest dose to be administered in the paediatric population.

2.1.4. Studies

Area	Number of studies	Description
Quality	1	Age-appropriate strength to be developed: A vial containing less than 10-fold the lowest dose to be administered in the paediatric population.
Non-clinical	0	Not applicable.
Clinical	2	Study 1: Open-label, non-randomised, multicentre, single dose trial to determine the dose of intravenous pegloticase required to control plasma uric acid levels in children from birth to less than 18 years of age at high risk for Tumour Lysis Syndrome. Study 2: Open-label, randomised, multicentre, single dose active controlled trial to evaluate efficacy and safety of pegloticase versus rasburicase, in children from birth to less than 18 years of age for the prevention and treatment of Tumour Lysis Syndrome.

2.2. Condition: Prevention of hyperuricaemia

2.2.1. Indication(s) targeted by the PIP

Treatment and/or prevention of hyperuricaemia related to Tumour Lysis Syndrome

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

From birth to less than 18 years of age.

2.2.3. Pharmaceutical form(s)

Solution for infusion, intravenous use.

Age-appropriate strength to be developed: A vial containing less than 10-fold the lowest dose to be administered in the paediatric population.

2.2.4. Studies

Area	Number of studies	Description
Quality	1	Same as for the condition Treatment of hyperuricaemia
Non-clinical	0	Not applicable.
Clinical	2	Study 1: Same as for the condition Treatment of hyperuricaemia Study 2: Same as for the condition Treatment of hyperuricaemia

3. Follow-up, completion and deferral of PIP

Measures to address long term follow-up of potential safety and efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By June 2017
Deferral for one or more studies contained in the paediatric investigation plan:	Yes