EMA/259113/2011

European Medicines Agency decision

P/86/2011

of 8 April 2011

on the acceptance of a modification of an agreed paediatric investigation plan for velaglucerase alfa (EMEA-000556-PIP01-09-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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on the acceptance of a modification of an agreed paediatric investigation plan for velaglucerase alfa (EMEA-000556-PIP01-09-M01) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

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 European Medicines Agency's decision P/245/2009 issued on 27 November 2009,

Having regard to the application submitted by Shire Pharmaceuticals Ireland Limited on 3 December 2010 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 18 February 2011, 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 velaglucerase alfa, lyophilisate for solution for infusion, 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 Shire Pharmaceuticals Ireland Limited, 5 Riverwalk Citiwest Business Campus, 24 Dublin, Ireland.

Done at London, 8 April 2011

For the European Medicines Agency
Andreas Pott
Acting Executive Director
(Signature on file)

EMA/PDCO/25454/2011

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

EMEA-000556-PIP01-09-M01

Scope of the application

Active substance(s):

Velaglucerase alfa

Condition(s):

Gaucher Disease, Types 1 and 3
Gaucher Disease, Type 2

Pharmaceutical form(s):

Lyophilisate for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Shire Pharmaceuticals Ireland Limited

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Shire Pharmaceuticals Ireland Limited submitted to the European Medicines Agency on 03 December 2010 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/245/2009 issued on 27 November 2009.
The application for modification proposed changes to the agreed paediatric investigation plan.

The procedure started on 22 December 2010.

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Scope of the modification

The initiation timelines in two studies have been modified along with a change in inclusion criteria in one study.

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

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

2. The measures and timelines of the paediatric investigation plan and the subset(s) of the paediatric population and condition(s) covered by the waiver 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, 18 February 2011

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

1.1. Condition: Treatment of Gaucher Disease, types 1 and 3

The waiver applies to:

- Preterm newborn infants, Term newborn infants (from birth to less than 28 days), Infants and toddlers (from 28 days to less than 24 months)
- for lyophilisate for solution for infusion, for intravenous use
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

1.2. Condition: Treatment of Gaucher Disease, Type 2

The waiver applies to:

- All subsets of the paediatric population from birth to less than 18 years of age
- for lyophilisate for solution for infusion, for intravenous use
- on the grounds that the specific medicinal product is likely to be ineffective

2. Paediatric Investigation Plan

2.1. Condition: Treatment of Gaucher Disease, types 1 and 3

2.1.1. Indication(s) targeted by the PIP

Long-term enzyme replacement therapy (ERT) for paediatric patients with type 1 and 3 Gaucher disease

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

From 2 to less than 18 years.

2.1.3. Pharmaceutical form(s)

Lyophilisate for solution for infusion, intravenous use, 40U/ml

2.1.4. Studies

Area	Number of studies	Description
Quality		Not applicable.
Non-clinical		Not applicable.
Clinical	**3**	1. Open-label extension study of velaglucerase alfa enzyme replacement therapy in adults and children with Type 1 Gaucher Disease 2. Open-label, severity-stratified, efficacy and safety study of two doses of velaglucerase alfa enzyme replacement therapy in children and adolescents with Type 1 Gaucher Disease 3. Open-label efficacy and safety study of velaglucerase alfa enzyme replacement therapy in children and adolescents with Type 3 Gaucher Disease

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 December 2015
Deferral for one or more studies contained in the paediatric investigation plan:	Yes