European Medicines Agency

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EUROPEAN MEDICINES AGENCY DECISION

of 27 November 2009

on the agreement of a Paediatric Investigation Plan and on the granting of a deferral and on the granting of a waiver for velaglucerase alfa (EMEA-000556-PIP01-09) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council as amended

(ONLY THE ENGLISH TEXT IS AUTHENTIC)

DISCLAIMER: This Decision does not entitle to the rewards and incentives referred to in Title V of Regulation (EC) No 1901/2006, as amended.

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THE EUROPEAN MEDICINES AGENCY,

Having regard to the Treaty establishing the European Community,

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 as amended 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 Shire Pharmaceuticals Ireland Limited on 27 March 2009 under Article 16(1) of Regulation (EC) No 1901/2006 as amended also requesting a waiver under Article 13 of said Regulation and a deferral under Article 20 of said Regulation,

Having regard to the Opinion of the Paediatric Committee of the European Medicines Agency, issued on 13 November 2009, in accordance with Article 18 of Regulation (EC) No 1901/2006 as amended, and Article 13 of said Regulation and Article 21 of said Regulation**,**

Having regard to Article 25 of Regulation (EC) No 1901/2006 as amended,

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 granting 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 granting 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 velaglucerase alfa, lyophilisate 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

A deferral for velaglucerase alfa, lyophilisate 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 granted.

Article 3

A waiver for velaglucerase alfa, lyophilisate 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 granted.

Article 4

This decision is addressed to Shire Pharmaceuticals Ireland Limited, 5 Riverside Walk, Citywest Business Campus, Dublin 24, Ireland.

Done at London, 27 November 2009

For the European Medicines Agency
Thomas Lönngren
Executive Director

(Signature on file)

European Medicines Agency

Doc. Ref. EMEA/PDCO/702425/2009
EMEA-000556-PIP01-09

OPINION OF THE PAEDIATRIC COMMITTEE ON THE AGREEMENT OF A PAEDIATRIC INVESTIGATION PLAN AND A DEFERRAL AND A WAIVER

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 16(1) of Regulation (EC) No 1901/2006 as amended, Shire Pharmaceuticals Ireland Limited submitted for agreement to the EMEA on 27 March 2009 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 30 April 2009.

Supplementary information was provided by the applicant on 14 August 2009.

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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 grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(a) of said Regulation, on the grounds that the specific medicinal product is likely to be ineffective or unsafe in part or all of the paediatric population, and with Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

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 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 Agency, together with its annex(es) and appendix.

London, 13 November 2009

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 AND THE SUBSET(S) OF THE PAEDIATRIC POPULATION AND CONDITION(S) COVERED BY THE WAIVER

A. CONDITION(S)

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

B. WAIVER

- **Condition**

  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.

- **Condition**

  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

C. PAEDIATRIC INVESTIGATION PLAN

- **Condition to be investigated**

  Gaucher Disease, types 1 and 3

- **Proposed PIP indication**

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

- **Subset(s) of the paediatric population concerned by the paediatric development**

  From 2 to less than 18 years.

- **Formulation(s)**

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

- **Studies**

Area	Number of studies	Description
Quality	0	Not applicable.
Non-clinical	0	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

Measures to address long term follow-up of potential safety issues and efficacy in relation to paediatric use:	**No**
Date of completion of the paediatric investigation plan:	**By December 2015**
Deferral for some or all studies contained in the paediatric investigation plan:	**Yes**