EMA/208668/2010

European Medicines Agency decision

P/57/2010

of 9 April 2010

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for taliglucerase alfa (EMEA-000648-PIP01-09) 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 Protalix B.V. on 17 July 2009 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 19 February 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 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 taliglucerase alfa, powder 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 taliglucerase alfa, powder 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 taliglucerase alfa, powder 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 Protalix B.V., Locatellikade 1, 1076 AZ Amsterdam, The Netherlands.

Done at London, 9 April 2010

For the European Medicines Agency
Thomas Lönngren
Executive Director

(Signature on file)

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

19 February 2010
EMA/PDCO/73447/2010

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

EMEA-000648-PIP01-09

Scope of the application

Active substance(s):

Taliglucerase alfa

Condition(s):

Gaucher Disease (except acute neuronopathic)

Gaucher Disease, acute neuronopathic

Pharmaceutical form(s):

Powder for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Protalix B.V.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Protalix B.V. submitted for agreement to the European Medicines Agency on 17 July 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 20 August 2009.

Supplementary information was provided by the applicant on 7 December 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:

   - 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 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 member(s) agree(s) 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 European Medicines Agency, together with its annex and appendix.

London, 19 February 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 and the subset(s) of the paediatric population and condition(s) covered by the waiver

1. Condition(s)

Gaucher Disease (except acute neuronopathic)

Gaucher Disease, acute neuronopathic

2. Waiver

2.1. Condition

Gaucher Disease (except acute neuronopathic)

The waiver applies to:

- neonates, infants and toddlers (from birth to less than 24 months)
- for powder for solution for infusion, intravenous use
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.

2.2. Condition

Gaucher Disease, acute neuronopathic

The waiver applies to:

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

3. Paediatric Investigation Plan

3.1. Condition to be investigated

Gaucher Disease (except acute neuronopathic)

3.2. Indication targeted by the PIP

Long term enzyme replacement therapy for the treatment of systemic symptoms in patients with a confirmed diagnosis of Gaucher Disease

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

From 2 to less than 18 years of age.

3.4. Pharmaceutical form(s)

Powder for solution for infusion.

3.5. Studies

Area	**Number of studies**	**Description**
Quality	0	Not applicable.
Non-clinical	0	Not applicable.
Clinical	3	1. Double-blind, randomised, efficacy and safety study of two doses of taliglucerase alfa enzyme replacement therapy in children and adolescents with Gaucher Disease (non-neuronopathic and chronic neuronopathic). 2. Open-label switchover trial to assess the safety and efficacy of taliglucerase alfa in adult and paediatric patients with Gaucher Disease treated with imiglucerase. 3. Extension trial to assess the long term safety and efficacy of taliglucerase alfa in adult and paediatric patients with Gaucher Disease.

4. Follow-up, completion and deferral of PIP

Measures to address long term follow-up of potential safety issues or efficacy in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By December 2014
Deferral for one or more studies contained in the paediatric investigation plan:	Yes