EMA/158091/2011

# European Medicines Agency decision

P/62/2011

of 7 March 2011

on the agreement of a paediatric investigation plan and on the granting of a waiver for bucelipase alfa (EMEA-000822-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 Swedish Orphan Biovitrum AB on 11 February 2010 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 14 January 2011, in accordance with Article 18 of Regulation (EC) No 1901/2006, 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 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 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 bucelipase alfa, powder for oral solution, oral 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 waiver for bucelipase alfa, powder for oral solution, oral 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***

This decision is addressed to Swedish Orphan Biovitrum AB (publ), Tomtebodavägen 23a, SE-112 76 Stockholm, Sweden.

Done at London, 7 March 2011

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

EMA/PDCO/783722/2010

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

EMEA-000822-PIP01-09

## Scope of the application

**Active substance(s):**

Bucelipase alfa

**Condition(s):**

Prevention of growth retardation due to lack of bile salt-stimulated lipase in enteral nutrition

**Pharmaceutical form(s):**

Powder for oral solution

**Route(s) of administration:**

Oral use

**Name/corporate name of the PIP applicant:**

Swedish Orphan Biovitrum AB

## Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Swedish Biovitrum Orphan AB submitted for agreement to the European Medicines Agency on 11 February 2010 an application for a paediatric investigation plan for the above mentioned medicinal product and a waiver under Article 13 of said Regulation.

The procedure started on 23 March 2010.

Supplementary information was provided by the applicant on 5 November 2010.

A meeting with the Paediatric Committee took place on 12 January 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,

   - 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)(b) of said Regulation, on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified subset(s) of the paediatric population.

   The Norwegian Paediatric Committee member agrees 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, 14 January 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: Prevention of growth retardation due to lack of bile salt-stimulated lipase in enteral nutrition*

The waiver applies to:

- Term newborn infants, infants, toddlers and children to less than 18 years of age
- for powder for oral solution for oral use
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

# 2. Paediatric Investigation Plan

## *2.1. Condition: Prevention of growth retardation due to lack of bile salt-stimulated lipase in enteral nutrition*

### 2.1.1. Indication(s) targeted by the PIP

Bucelipase alfa is indicated for the supplementation of pasteurized breast milk and fortified formula for four weeks to improve growth by increasing fat absorption in enterally fed preterm infants

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

Preterm neonates

### 2.1.3. Pharmaceutical form(s)

Powder for oral solution for oral use

### 2.1.4. Studies

| Area | Number of studies | Description |
|---|---|---|
| Quality | 1 | Study 1: In-use stability study to mimic clinical handling |
| Non-clinical | 1 | Study 2: Repeat dose oral toxicity study in juvenile rats |
| Clinical | 3 | Study 3: Randomized, double-blind, two-period crossover trial to evaluate pharmacodynamics, safety and efficacy trial in premature neonates<br><br>Study 4: Randomized, double-blind, two-period crossover trial to evaluate pharmacodynamics, safety and efficacy trial in premature neonates<br><br>Study 5: Randomized, double-blind, placebo-controlled, parallel-group, multi-centre trial to evaluate the pharmacodynamics, safety and efficacy of bucelipase alfa in premature neonates |

## 3. Follow-up, completion and deferral of PIP

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