EMA/179906/2013

European Medicines Agency decision

P/0092/2013

of 29 April 2013

on the acceptance of a modification of an agreed paediatric investigation plan for bucelipase alfa (EMEA-000822-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.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

European Medicines Agency decision

P/0092/2013

of 29 April 2013

on the acceptance of a modification of an agreed paediatric investigation plan for bucelipase alfa (EMEA-000822-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/62/2011 issued on 7 March 2011,

Having regard to the application submitted by Swedish Orphan Biovitrum AB (publ) on 14 December 2012 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 15 March 2013, 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 bucelipase alfa, powder for oral solution, oral 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 Swedish Orphan Biovitrum AB (publ), Tomtebodavägen 23a, SE-112 76 – Stockholm, Sweden.

Done at London, 29 April 2013

For the European Medicines Agency
Guido Rasi
Executive Director
(Signature on file)

EMA/PDCO/3967/2013

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

EMEA-000822-PIP01-09-M01

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 (publ)

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Swedish Orphan Biovitrum AB (publ) submitted to the European Medicines Agency on 14 December 2012 an application for modification of the agreed paediatric investigation plan with a waiver as set out in the European Medicines Agency's decision P/62/2011 issued on 7 March 2011.

The application for modification proposed changes to the agreed paediatric investigation plan.

The procedure started on 16 January 2013.

A meeting with the Paediatric Committee took place on 14 March 2013.

Scope of the modification

A measure of the Paediatric Investigation Plan has been modified.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

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 in the scope set out in the Annex I of this opinion.

   The Norwegian Paediatric Committee member agrees 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 and appendix.

London, 15 March 2013

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. Measures

Area	Number of measures	Description
Quality	1	**Measure 1:** In-use stability study to mimic clinical handling
Non-clinical	1	**Measure 2:** Repeat dose oral toxicity study in juvenile rats
Clinical	3	**Measure 3:** Randomized, double-blind, two-period crossover trial to evaluate pharmacodynamics, safety and efficacy trial in premature neonates **Measure 4:** Randomized, double-blind, two-period crossover trial to evaluate pharmacodynamics, safety and efficacy trial in premature neonates

Area	Number of measures	Description
		Measure 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

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By April 2014
Deferral for one or more measures contained in the paediatric investigation plan:	No