EMA/434301/2015

European Medicines Agency decision

P/0173/2015

of 7 August 2015

on the acceptance of a modification of an agreed paediatric investigation plan for sebelipase alfa (EMEA-001331-PIP01-12-M02) 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 European Medicines Agency's decision P/0112/2013 issued on 30 April 2013, and the decision P/0179/2014 issued on 11 July 2014,

Having regard to the application submitted by Synageva BioPharma Ltd. on 26 March 2015 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 19 June 2015, 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 and to the deferral.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan, including changes to the deferral.

[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 sebelipase alfa, concentrate for solution for infusion, intravenous use, including changes to the deferral, 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 Synageva BioPharma Ltd., 1A Local Board Road, WD17 2JP - Watford, Hertfordshire, United Kingdom.

Done at London, 7 August 2015

For the European Medicines Agency
Jordi Llinares Garcia
Head of Division (ad interim)
Human Medicines Research and Development Support
(Signature on file)

EMA/PDCO/237262/2015
London, 19 June 2015

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

EMEA-001331-PIP01-12-M02

Scope of the application

Active substance(s):

Sebelipase alfa

Condition(s):

Treatment of lysosomal acid lipase deficiency

Pharmaceutical form(s):

Concentrate for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Synageva BioPharma Ltd.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Synageva BioPharma Ltd. submitted to the European Medicines Agency on 26 March 2015 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0112/2013 issued on 30 April 2013, and the decision P/0179/2014 issued on 11 July 2014.

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

The procedure started on 24 April 2015.

Scope of the modification

Some measures and timelines of the Paediatric Investigation Plan have been modified.

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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 and to the deferral 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 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.

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 (PIP)

1. Waiver

Not applicable.

2. Paediatric Investigation Plan

2.1. Condition:

Treatment of lysosomal acid lipase deficiency

2.1.1. Indication(s) targeted by the PIP

Treatment of early onset lysosomal acid lipase deficiency/ wolman disease

Treatment of late onset lysosomal acid lipase deficiency/ cholesteryl ester storage disease

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

From birth to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Concentrate for solution for infusion

2.1.4. Measures

Area	Number of studies	Description
Quality-related studies	0	Not applicable.
Non-clinical studies	3	**Study 1** Rat Developmental Toxicity and Toxicokinetic Intravenous Infusion Study (Seg II). **Study 2** Rabbit Developmental Toxicity and Toxicokinetic Intravenous Infusion Study (Seg II). **Study 3** Intravenous Infusion Pre- and Post-Natal Study in the Rat.
Clinical studies	3	**Study 4** Open-label, multi-centre, dose escalating, historical controlled trial to evaluate, pharmacokinetics, safety, efficacy, immunogenicity of sebelipase alfa in children from birth to 2 years of age with Lysosomal Acid Lipase Deficiency.

		Study 5 Double-blind, randomised, placebo controlled trial to evaluate, pharmacokinetics, safety, efficacy, immunogenicity of sebelipase alfa in children from 4 years of age and adults with Late Onset Lysosomal Acid Lipase Deficiency or Cholesteryl ester storage disease (including adults). **Study 6** Open-label, multi-centre, trial to evaluate the safety, tolerability, and immunogenicity of sebelipase alfa in a broad population of subjects with LAL Deficiency (including adults).

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By March 2017
Deferral for one or more measures contained in the paediatric investigation plan:	Yes