EMA/391453/2014

European Medicines Agency decision

P/0179/2014

of 11 July 2014

on the acceptance of a modification of an agreed paediatric investigation plan for recombinant human lysosomal acid lipase (EMEA-001331-PIP01-12-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.

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on the acceptance of a modification of an agreed paediatric investigation plan for recombinant human lysosomal acid lipase (EMEA-001331-PIP01-12-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/0112/2013 issued on 30 April 2013,

Having regard to the application submitted by Synageva BioPharma Ltd on 27 March 2014 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 20 June 2014, 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 recombinant human lysosomal acid lipase, concentrate for solution for infusion, intravenous 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 Synageva BioPharma Ltd, c/o Kevin Beare & Co., 3-5 Horndean Road, RG12 0XQ - Bracknell, Berkshire, United Kingdom.

Done at London, 11 July 2014

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

EMA/PDCO/218717/2014

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

EMEA-001331-PIP01-12-M01

Scope of the application

Active substance(s):

Recombinant human lysosomal acid lipase

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 27 March 2014 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.

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

The procedure started on 23 March 2014.

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

   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 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.

London, 20 June 2014

On behalf of the Paediatric Committee
Dr Dirk Mentzer, 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 (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 recombinant human lysosomal acid lipase 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 recombinant human lysosomal acid lipase 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 recombinant human lysosomal acid lipase 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 April 2016
Deferral for one or more measures contained in the paediatric investigation plan:	Yes