EMA/227590/2015

European Medicines Agency decision

P/0082/2015

of 10 April 2015

on the agreement of a paediatric investigation plan for recombinant human N-acetylglucosaminidase (rhNAGLU) (EMEA-001653-PIP01-14) 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 Synageva BioPharma Ltd. on 9 June 2014 under Article 16(1) of Regulation (EC) No 1901/2006,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 20 March 2015, in accordance with Article 18 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 agreement of a paediatric investigation plan.

(2) It is therefore appropriate to adopt a decision agreeing a 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

A paediatric investigation plan for recombinant human N-acetylglucosaminidase (rhNAGLU), concentrate for solution for injection/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

This decision is addressed to Synageva BioPharma Ltd., 1A Local Board Road, WD17 2JP - Watford, Hertfordshire, United Kingdom.

Done at London, 10 April 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/21963/2015
London, 20 March 2015

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan

EMEA-001653-PIP01-14

Scope of the application

Active substance(s):

Recombinant human N-acetylglucosaminidase (rhNAGLU)

Condition(s):

Treatment of mucopolysaccharidosis IIIB (Sanfilippo B)

Pharmaceutical form(s):

Concentrate for solution for injection/infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Synageva BioPharma Ltd.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Synageva BioPharma Ltd. submitted for agreement to the European Medicines Agency on 9 June 2014 an application for a paediatric investigation plan for the above mentioned medicinal product.

The procedure started on 16 July 2014.

Supplementary information was provided by the applicant on 19 December 2014. The applicant proposed modifications to the paediatric investigation plan.

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.

   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 agreed 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 mucopolysaccharidosis IIIB (Sanfilippo B)

2.1.1. Indication(s) targeted by the PIP

Treatment of mucopolysaccharidosis IIIB (Sanfilippo B)

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 injection/infusion

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	1	**Study 1** Development of higher concentration formulation resulting in administration volumes appropriate to the paediatric population.
Non-clinical studies	1	**Study 2** Intravenous infusion study of SBC-103 in the juvenile cynomolgus monkey
Clinical studies	6	**Study 3 NGLU-CL02** Open-label, dose escalation study to evaluate safety, tolerability, PK, PD and activity of recombinant human N-acetylglucosaminidase (rhNAGLU) administered intravenously. **Study 4 NGLU-CL06** Open-label, dose escalation study to assess safety, tolerability, PK, PET biodistribution, PD and activity of recombinant human N-acetylglucosaminidase (rhNAGLU) **Study 5 NGLU-CL03** Randomized, double-blind, placebo-controlled study to evaluate efficacy of recombinant human N-

		acetylglucosaminidase (rhNAGLU) in children from birth to less than 6 years of age for the treatment of mucopolysaccharidosis IIIB (Sanfilippo B). **Study 6** Randomized, double-blind, placebo-controlled study to evaluate efficacy of recombinant human N-acetylglucosaminidase (rhNAGLU) in children from 6 to less than 18 years of age for the treatment of mucopolysaccharidosis IIIB (MPS IIIB; Sanfilippo B). **Study 7 NGLU-NH01** Retrospective chart review of deceased MPS IIIB patients to generate data to evaluate the clinical characteristics and course of disease progression of MPS IIIB. **Study 8 NGLU-NH02** Observational study to describe the clinical and biochemical characteristics and course of disease progression in patients with MPS IIIB.
Extrapolation, modelling and simulation studies	0	Not applicable.
Other studies	0	Not applicable.
Other measures	0	Not applicable.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By April 2022
Deferral for one or more measures contained in the paediatric investigation plan:	No