EMA/110877/2017

European Medicines Agency decision

P/0075/2017

of 17 March 2017

on the agreement of a paediatric investigation plan and on the granting of a deferral for recombinant human alpha-glucosidase conjugated with synthetic bismannose-6-phosphate-tetramannose glycan (neoGAA) (EMEA-001945-PIP01-16) 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 Genzyme Europe B.V. on 14 April 2016 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 27 January 2017, in accordance with Article 18 of Regulation (EC) No 1901/2006 and Article 21 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 deferral.

(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 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

A paediatric investigation plan for recombinant human alpha-glucosidase conjugated with synthetic bismannose-6-phosphate-tetramannose glycan (neoGAA), powder for concentrate for solution for 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

A deferral for recombinant human alpha-glucosidase conjugated with synthetic bismannose-6-phosphate-tetramannose glycan (neoGAA), powder for concentrate for solution for 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 granted.

Article 3

This decision is addressed to Genzyme Europe B.V., Gooimeer 10, NL-1411 DD – Naarden, The Netherlands.

EMA/PDCO/393061/2016
London, 27 January 2017

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

EMEA-001945-PIP01-16

Scope of the application

Active substance(s):

Recombinant human alpha-glucosidase conjugated with synthetic bismannose-6-phosphate-tetra-mannose glycan (neoGAA)

Condition(s):

Treatment of Pompe disease

Pharmaceutical form(s):

Powder for concentrate for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Genzyme Europe B.V.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Genzyme Europe B.V. submitted for agreement to the European Medicines Agency on 14 April 2016 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation.

The procedure started on 24 May 2016.

Supplementary information was provided by the applicant on 27 October 2016.

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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 deferral in accordance with Article 21 of said Regulation.

   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 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 Pompe disease

2.1.1. Indication(s) targeted by the PIP

Long-term ERT treatment of patients with Pompe disease (acid α-glucosidase deficiency)

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)

Powder for concentrate for solution for infusion

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable.
Non-clinical studies	0	Not applicable.
Clinical studies	3	Study 1 Randomized, multicentre, multinational, double-blinded study comparing the efficacy and safety of Recombinant human alpha-glucosidase conjugated with synthetic bismannose-6-phosphate-tetra-mannose glycan (neoGAA) and alglucosidase alfa in treatment-naïve patients from 3 years of age with late onset Pompe disease. Study 2 Open-label, multicentre, multinational, ascending dose, repeated intravenous infusion study of neoGAA in treatment experienced paediatric patients from 6 months to less than 18 years of age with infantile-onset Pompe disease (IOPD) to evaluate the safety profile of and the pharmacokinetic profile of neoGAA and to evaluate the preliminary efficacy of neoGAA in comparison to alglucosidase alfa.

		Study 3 Open-label, multinational, multicentre study of neoGAA in treatment-naïve paediatric patients from birth to less than 7 months of age with infantile onset Pompe disease (IOPD) to determine the safety, tolerability and effect of neoGAA treatment.
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:	No
Date of completion of the paediatric investigation plan:	By December 2024
Deferral for one or more measures contained in the paediatric investigation plan:	Yes