EMA/797359/2017

European Medicines Agency decision

P/0400/2017

of 19 December 2017

on the refusal of a modification of an agreed paediatric investigation plan for recombinant human glutamic acid decarboxylase (rhGAD65) (EMEA-000609-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.

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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/167/2010 issued on 3 September 2010,

Having regard to the application submitted by Diamyd Medical AB on 28 July 2017 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 10 November 2017, in accordance with Article 22 of Regulation (EC) No 1901/2006, and Article 21 of said Regulation and Article 13 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 refusal of changes to the agreed paediatric investigation plan and to the deferral.

(2) It is therefore appropriate to adopt a decision on the refusal 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 recombinant human glutamic acid decarboxylase (rhGAD65), suspension for injection, subcutaneous use, including changes to the deferral, 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, are hereby refused.

Article 2

This decision is addressed to Diamyd Medical AB, Kungsgatan 29, 11156 – Stockholm, Sweden.

EMA/PDCO/537494/2017
London, 10 November 2017

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

EMEA-000609-PIP01-09-M01

Scope of the application

Active substance(s):

Recombinant human glutamic acid decarboxylase (rhGAD65)

Condition(s):

Treatment of type I diabetes mellitus

Pharmaceutical form(s):

Suspension for injection

Route(s) of administration:

Subcutaneous use

Name/corporate name of the PIP applicant:

Diamyd Medical AB

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Diamyd Medical AB submitted to the European Medicines Agency on 28 July 2017 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/167/2010 issued on 3 September 2010.

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

The procedure started on 12 September 2017.

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 refuse the changes proposed by the applicant regarding the paediatric investigation plan and the deferral.

   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 and the subset(s) of the paediatric population and condition(s)) covered by the waiver remain unchanged and are set out in the Annex I.

3. The scientific conclusions and the grounds for refusal are set out in the summary report appended to this opinion.

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

1.1. Condition:

Treatment of type I diabetes mellitus

The waiver applies to:

- The paediatric population from birth to less than 1 year of age;
- suspension for injection, subcutaneous use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2. Paediatric investigation plan

2.1. Condition:

Treatment of type I diabetes mellitus

2.1.1. Indication(s) targeted by the PIP

Treatment of recent-onset type I diabetes mellitus

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

From 1 to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Suspension for injection

2.1.4. Measures

Area	Number of studies	Description
Quality		Not applicable
Non-clinical		Not applicable
Clinical	5	**Study 1** Randomised, double-blind, parallel-group, placebo-controlled, multicentre study, in children and adolescents between 10 and 18 years with type I diabetes mellitus, of two 20-microgram doses of rhGAD65, administered SC 4 weeks apart **Study 2** 3-arm, randomized, double-blind, placebo-controlled, multicenter, clinical trial of a prime-and-boost regimen of rhGAD65 in children, adolescents and adults between 10 and 20 years old, with type I diabetes mellitus

		Study 3 3-arm, randomized, double-blind, placebo-controlled, multicenter, clinical trial of a prime-and-boost regimen of rhGAD65 in children, adolescents and adults between 10 and 20 years old, with type I diabetes mellitus **Study 4** 2-arm, randomized, double-blind, placebo-controlled, multicenter, clinical trial of a prime-and-boost regimen of rhGAD65 in children, between 4 and less than 10 years old, with type I diabetes mellitus **Study 5** 2-arm, randomized, double-blind, placebo-controlled, multicenter, clinical trial of a prime-and-boost regimen of rhGAD65 in children, between 1 and less than 4 years old, with type I diabetes mellitus

3. Follow-up, completion and deferral of PIP

Measures to address long term follow-up of potential safety and efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By December 2017
Deferral for one or more studies contained in the paediatric investigation plan:	Yes