EMA/413457/2020

European Medicines Agency decision

P/0318/2020

of 12 August 2020

on the acceptance of a modification of an agreed paediatric investigation plan for recombinant human glutamic acid decarboxylase (rhGAD65) (EMEA-000609-PIP01-09-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/167/2010 issued on 3 September 2010 and the decision P/0400/2017 issued on 19 December 2017,

Having regard to the application submitted by Diamyd Medical AB on 23 March 2020 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 26 June 2020, 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 recombinant human glutamic acid decarboxylase (rhGAD65), suspension for injection, intralymphatic 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 Diamyd Medical AB, Kungsgatan 29, 111 56 – Stockholm, Sweden.

EMA/PDCO/200184/2020
Amsterdam, 26 June 2020

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

EMEA-000609-PIP01-09-M02

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:

Intralymphatic 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 23 March 2020 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 and the decision P/0400/2017 issued on 19 December 2017.

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

The procedure started on 30 April 2020.

Scope of the modification

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

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 and the subset(s) of the paediatric population and condition(s) covered by the waiver 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

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, intralymphatic 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	0	Not applicable
Non-clinical	0	Not applicable
Clinical	*7*	**Study 1 (D/P2/04/3)** 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 (Protocol # D/P3/07/4)** 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 (Protocol # D/P3/07/5) Deleted in procedure EMEA-000609-PIP01-09-M02 **Study 4 (Protocol # D/P4/11/6)** Deleted in procedure EMEA-000609-PIP01-09-M02 **Study 5 (Protocol # D/P4/14/7)** Deleted in procedure EMEA-000609-PIP01-09-M02 **Study 6 (DIAGNODE-1)** Added in procedure EMEA-000609-PIP01-09-M02 Open-label study to evaluate the safety of rhGAD65 administered into lymph nodes combined with an oral Vitamin D regimen in patients with newly diagnosed type 1 diabetes mellitus (T1DM). **Study 7 (DIAGNODE-2)** Added in procedure EMEA-000609-PIP01-09-M02 Randomised, double-blind, placebo-controlled study to evaluate the efficacy of rhGAD65 administered into lymph nodes with or without oral Vitamin D supplementation in adolescents and young adults with newly diagnosed T1DM. **Study 8 (DIAGNODE-3)** Added in procedure EMEA-000609-PIP01-09-M02 Randomised, double-blind, placebo-controlled study to evaluate the efficacy of 3 versus 4 doses of rhGAD65 administered into lymph nodes with oral Vitamin D supplementation in adolescents and young adults with newly diagnosed T1DM. **Study 9 (DIAGNODE-4)** Added in procedure EMEA-000609-PIP01-09-M02 Randomised, double-blind, placebo-controlled study to evaluate the efficacy of rhGAD65 administered into lymph nodes with oral Vitamin D supplementation in children from 4 to less than 12 years of age with newly diagnosed T1DM. **Study 10 (DIAGNODE-5)** Added in procedure EMEA-000609-PIP01-09-M02 Randomised, double-blind, placebo-controlled study to evaluate the efficacy of rhGAD65 administered into lymph nodes with oral Vitamin D supplementation in children from 1 to less than 4 years of age with newly diagnosed T1DM.

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 September 2034
Deferral for one or more studies contained in the paediatric investigation plan:	Yes