EMA/632794/2020

European Medicines Agency decision

P/0505/2020

of 22 December 2020

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for venglustat (EMEA-001716-PIP05-20) 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 21 February 2020 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 13 November 2020, in accordance with Article 17 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 agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver.

(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.

(4) It is therefore appropriate to adopt a decision granting a waiver.

[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 venglustat, tablet, oral 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 venglustat, tablet, oral 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

A waiver for venglustat, tablet, oral 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 4

This decision is addressed to Genzyme Europe B.V., Paasheuvelweg 25, 1105 BP – Amsterdam, The Netherlands.

EMA/PDCO/490349/2020 Corr
Amsterdam, 13 November 2020

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

EMEA-001716-PIP05-20

Scope of the application

Active substance(s):

Venglustat

Condition(s):

Treatment of autosomal dominant polycystic kidney disease

Pharmaceutical form(s):

Tablet

Route(s) of administration:

Oral 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 21 February 2020 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 31 March 2020.

Supplementary information was provided by the applicant on 5 August 2020. 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 17(1) of said Regulation;
   - to grant a deferral in accordance with Article 21 of said Regulation;
   - to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

   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 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 autosomal dominant polycystic kidney disease

The waiver applies to:

- the paediatric population from birth to less than 12 years of age;
- tablet, oral use;
- on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2. Paediatric investigation plan

2.1. Condition:

Treatment of autosomal dominant polycystic kidney disease

2.1.1. Indication(s) targeted by the PIP

Long-term treatment to slow progression of cyst development in paediatric patients from 12 years to less than 18 years of age with autosomal dominant polycystic kidney disease.

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

From 12 years to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Tablet

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	2	**Study 1** (same as study 1 in EMEA-001716-PIP04-19) Development of small size chewable tablets of 4 mg, 6 mg and 15 mg dosage strengths appropriate to be administered in paediatric patients from 12 years of age and older using doses based on body weight. **Study 2** (same as study 2 in EMEA-001716-PIP04-19) Generation of data in adult subjects on the palatability of the tablets (ACC15856)

Area	Number of measures	Description
Non-clinical studies	1	**Study 3** (same as study 1 in EMEA-001716-PIP04-19) Enhanced pre- and postnatal development study in Sprague-Dawley rats to evaluate the potential toxic effects of venglustat on the pregnant/lactating female and on development of the conceptus and the offspring through sexual maturity.
Clinical studies	1	**Study 4** Randomized, double-blind, placebo-controlled study to evaluate the efficacy, safety, tolerability and pharmacokinetics of venglustat compared to placebo in adolescents from 12 to less than 18 years of age with autosomal dominant polycystic kidney disease
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 March 2031
Deferral for one or more measures contained in the paediatric investigation plan:	Yes