EMA/529962/2023

European Medicines Agency decision

P/0489/2023

of 1 December 2023

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-PIP07-22) 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.

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
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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 Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the application submitted by Sanofi B.V. on 18 November 2022 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 October 2023, 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, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

A paediatric investigation plan for venglustat, chewable 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, chewable 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, chewable 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 Sanofi B.V., Paasheuvelweg 25, 1105 BP, Amsterdam, The Netherlands.

EMA/PDCO/326582/2023
Amsterdam, 13 October 2023

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

EMEA-001716-PIP07-22

Scope of the application

Active substance(s):

Venglustat

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of Gaucher Disease type 2

Treatment of Gaucher Disease type 3

Pharmaceutical form(s):

Chewable tablet

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Sanofi B.V.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Sanofi B.V. submitted for agreement to the European Medicines Agency on 18 November 2022 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 3 January 2023.

Supplementary information was provided by the applicant on 3 July 2023. 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 Paediatric Committee member of Norway 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 annexes 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 Gaucher disease Type 2

The waiver applies to:

- all subsets of the paediatric population from birth to less than 18 years of age;
- chewable tablet, oral use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.

1.2. Condition:

Treatment of Gaucher disease Type 3

The waiver applies to:

- the paediatric population from birth to less than 2 years of age;
- chewable tablet, oral use;

  on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

2. Paediatric investigation plan

2.1. Condition:

Treatment of Gaucher disease Type 3

2.1.1. Indication(s) targeted by the PIP

Treatment of Gaucher disease Type 3

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

From 2 years to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Chewable tablet, oral use.

2.1.4. Measures

Area	Description
Quality-related studies	Not applicable

Non-clinical studies	**Study 1** Oral gavage toxicity study in the juvenile rat (GT-373-TX-23). **Study 2** Oral gavage toxicity study in the male juvenile rats with an 18-week recovery and a toxicokinetic phase (JUV0046).
Clinical studies	**Study 3** Randomized 1:1, double-blind, double-dummy study, controlled versus imiglucerase, to evaluate the efficacy and safety of venglustat in paediatric patients from 12 to less than 18 years of age (and adults) with GD3 who have been treated with enzyme replacement therapy (ERT) for at least 3 years and have reached therapeutic goals (EFC17215). **Study 4** Randomized 2:1, open-label, controlled versus imiglucerase, to evaluate the efficacy and safety of venglustat in paediatric patients from 2 years to less than 12 years of age with GD3 who have been treated with enzyme replacement therapy (ERT) for at least 2 years and have reached therapeutic goals.
Modelling and simulation studies	**Study 5** PopPK, exposure-response study of venglustat to support dose-recommendation and extrapolation of efficacy from adults to paediatric patients from 2 to less than 12 years of age based on exposure-response similarity assessment.
Other studies	**Study 6** Quantitative systems pharmacology (QSP) model of visceral manifestations of GD. **Study 7** Pooled safety analysis of venglustat in the paediatric population.
Extrapolation plan	Studies 4,5,6 are part of an extrapolation plan covering the paediatric population from 2 years to less than 18 years of age, as agreed by the PDCO.

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

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

The product is not authorised anywhere in the European Community.