EMA/513173/2023

European Medicines Agency decision

P/0529/2023

of 29 December 2023

on the acceptance of a modification of an agreed paediatric investigation plan for venglustat (EMEA-001716-PIP04-19-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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on the acceptance of a modification of an agreed paediatric investigation plan for venglustat (EMEA-001716-PIP04-19-M01) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

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 European Medicines Agency's decision P/0457/2020 issued on 4 December 2020,

Having regard to the application submitted by Sanofi B.V. on 4 August 2023 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a waiver and proposing a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 10 November 2023, 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 on the refusal of a deferral.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

(3) It is therefore appropriate to adopt a decision refusing a deferral.

[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

Changes to the agreed paediatric investigation plan for venglustat, tablet, oral use, 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

A deferral for venglustat, tablet, oral use, the details of which are set out in the opinion of the Paediatric Committee the European Medicines Agency annexed hereto, together with its appendices, is hereby refused.

Article 3

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

EMA/PDCO/362983/2023
Amsterdam, 10 November 2023

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

EMEA-001716-PIP04-19-M01

Scope of the application

Active substance(s):

Venglustat

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of GM2 gangliosidosis

Treatment of GM1 gangliosidosis

Treatment of galactosialidosis

Treatment of sialidosis

Pharmaceutical form(s):

Tablet

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Sanofi B.V.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Sanofi B.V. submitted to the European Medicines Agency on 4 August 2023 an application for modification of the agreed paediatric investigation plan with a waiver as set out in the European Medicines Agency's decision P/0457/2020 issued on 4 December 2020.

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

The procedure started on 11 September 2023.

Scope of the modification

Some measures and 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 in the scope set out in the Annex I of this opinion;
   - to refuse a deferral as it does not meet the grounds detailed in Article 20(1) of said Regulation.

   The Paediatric Committee member of Norway 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 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 GM2 gangliosidosis

The waiver applies to:

- all subsets of the paediatric population from birth to less than 2 years of age;
- tablet, oral use;
- on the grounds that the specific medicinal product is likely to be ineffective.

1.2. Condition:

Treatment of GM1 gangliosidosis

The waiver applies to:

- all subsets of the paediatric population from birth to less than 2 years of age;
- tablet, oral use;
- on the grounds that the specific medicinal product is likely to be ineffective.

1.3. Condition:

Treatment of galactosialidosis

The waiver applies to:

- all subsets of the paediatric population from birth to less than 2 years of age;
- tablet, oral use;
- on the grounds that the specific medicinal product is likely to be ineffective.

1.4. Condition:

Treatment of sialidosis

The waiver applies to:

- all subsets of the paediatric population from birth to less than 2 years of age;
- tablet, oral use;
- on the grounds that the specific medicinal product is likely to be ineffective.

2. Paediatric investigation plan

2.1. Condition:

Treatment of GM2 gangliosidosis

2.1.1. Indication(s) targeted by the PIP

Long term treatment of patients from 2 years of age to less than 18 years of age with juvenile (subacute) and adolescent (late-onset) GM2 gangliosidosis

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

From 2 years of age to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Tablet

2.1.4. Measures

Area	Description
Quality-related studies	**Study 1** Development of small size chewable tablets of 4 mg, 6 mg and 15 mg dosage strengths appropriate to be administered in paediatric patients from 2 years of age and older using doses based on body weight **Study 2** Generation of data in adult subjects on the palatability of the tablets (ACC15856)
Non-clinical studies	**Study 3** 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	**Study 4** Randomised, double-blind, placebo-controlled study to evaluate the efficacy, pharmacokinetics, pharmacodynamics, safety and tolerability of venglustat in adult patients with (late onset) GM2 gangliosidosis (primary population) with an open-label single-arm part to evaluate the efficacy, pharmacokinetics, pharmacodynamics, safety and tolerability of venglustat in paediatric patients from 2 years to less than 18 years of age with (juvenile and late onset) GM2 gangliosidosis, GM1 gangliosidosis, galactosialidosis and sialidosis (secondary population) (EFC15299 - AMETHIST)
Extrapolation, modelling and simulation studies	**Study 5** Modelling and simulation study to determine the dose of venglustat to be used in the paediatric population from 2 years of age to less than 18 years of age (SIM0452)
Other studies	Not applicable.
Other measures	Not applicable.

2.2. Condition:

Treatment of GM1 gangliosidosis

2.2.1. Indication(s) targeted by the PIP

Long term treatment of patients from 2 years of age to less than 18 years of age with GM1 gangliosidosis

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

From 2 years of age to less than 18 years of age

2.2.3. Pharmaceutical form(s)

Tablet

2.2.4. Measures

Area	Description
Quality-related studies	**Study 1** (same as study 1 for the condition treatment of GM2 gangliosidosis) **Study 2** (same as study 2 for the condition treatment of GM2 gangliosidosis)
Non-clinical studies	**Study 3** (same as study 3 for the condition treatment of GM2 gangliosidosis)
Clinical studies	**Study 4** (same as study 4 for the condition treatment of GM2 gangliosidosis)
Extrapolation, modelling and simulation studies	**Study 5** (same as study 5 for the condition treatment of GM2 gangliosidosis)
Other studies	Not applicable.
Other measures	Not applicable.

2.3. Condition:

Treatment of galactosialidosis

2.3.1. Indication(s) targeted by the PIP

Long term treatment of patients from 2 years of age to less than 18 years of age with galactosialidosis

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

From 2 years of age to less than 18 years of age

2.3.3. Pharmaceutical form(s)

Tablet

2.3.4. Measures

Area	Description
Quality-related studies	**Study 1** (same as study 1 for the condition treatment of GM2 gangliosidosis) **Study 2** (same as study 2 for the condition treatment of GM2 gangliosidosis)
Non-clinical studies	**Study 3** (same as study 3 for the condition treatment of GM2 gangliosidosis)
Clinical studies	**Study 4** (same as study 4 for the condition treatment of GM2 gangliosidosis)
Extrapolation, modelling and simulation studies	**Study 5** (same as study 5 for the condition treatment of GM2 gangliosidosis)
Other studies	Not applicable.
Other measures	Not applicable.

2.4. Condition:

Treatment of sialidosis

2.4.1. Indication(s) targeted by the PIP

Long term treatment of patients from 2 years of age to less than 18 years of age with sialidosis

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

From 2 years of age to less than 18 years of age

2.4.3. Pharmaceutical form(s)

Tablet

2.4.4. Measures

Area	Description
Quality-related studies	**Study 1** (same as study 1 for the condition treatment of GM2 gangliosidosis) **Study 2** (same as study 2 for the condition treatment of GM2 gangliosidosis)
Non-clinical studies	**Study 3** (same as study 3 for the condition treatment of GM2 gangliosidosis)
Clinical studies	**Study 4** (same as study 4 for the condition treatment of GM2 gangliosidosis)

Extrapolation, modelling and simulation studies	**Study 5** (same as study 5 for the condition treatment of GM2 gangliosidosis)
Other studies	Not applicable.
Other measures	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 May 2024
Deferral for one or more measures contained in the paediatric investigation plan:	No

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

The product is not authorised anywhere in the European Community.