MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

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Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral and grant a waiver

MHRA-101314-PIP01-23

Scope of the Application

Active Substance(s)

Venglustat

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 the Decision

Pursuant to the Human Medicines Regulations 2012, Sanofi B.V. submitted to the licensing authority on 16/04/2024 14:51 BST an application for a Paediatric Investigation Plan

The procedure started on 13/01/2025 11:26 GMT

1. The licensing authority, having assessed the application in accordance with the Human Medicines Regulations 2012, decides, as set out in the appended summary report:

to agree a paediatric investigation plan and grant a deferral and grant a waiver

2. The measures and timelines of the paediatric investigation plan are set out in the Annex I.

This decision is forwarded to the applicant, together with its annex and appendix.

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Final Decision Letter

MHRA-101314-PIP01-23

Of 19/02/2025 17:06 GMT

On the adopted decision for Venglustat (MHRA-101314-PIP01-23) in accordance with the Human Medicines Regulations 2012.

The licensing authority, in accordance with the Human Medicines Regulations 2012, has adopted this decision:

Agreement on a paediatric investigation plan

This decision applies to a Paediatric Investigation Plan for Venglustat, Chewable tablet , ORAL USE .

This decision is addressed to Sanofi B.V., Paasheuvelweg 25, Amsterdam, NETHERLANDS, 1105 BP

ANNEX I

1. Waiver

1.1 Condition:

Condition 1: Treatment of Gaucher disease type 2 The waiver applies / applied to: Paediatric Subset(s): All subsets of the paediatric population from birth to less than 18 years of age Pharmaceutical form(s): Chewable tablet Route(s) of administration: ORAL USE Reason for granting waiver: on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible. Reason for Refusing Waiver: Not Applicable Condition 2: Treatment of Gaucher disease type 3 The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age Pharmaceutical form(s): Chewable tablet Route(s) of administration: ORAL USE Reason for granting waiver: 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(s):

Treatment of Gaucher disease type 3

2.2 Indication(s) targeted by the PIP:

Treatment of Gaucher disease type 3

2.3 Subset(s) of the paediatric population concerned by the paediatric development:

The paediatric population from 2 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Chewable tablet

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	2	Study 1 (GT-373-TX-23) Oral gavage toxicity study in the juvenile rat. Study 2 (JUV0046) Oral gavage toxicity study in the male juvenile rats with an 18-week recovery and a toxicokinetic phase.
Clinical Studies	2	Study 3 (EFC17215) Randomised 1:1, double-blind, double-dummy study, controlled versus imiglucerase, to evaluate the efficacy and safety of venglustat in paediatric patients from 12 years 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. Study 4 Randomised 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.
Extrapolation, Modeling & Simulation Studies	2	Study 5 PopPK, exposure-response study of venglustat to support dose recommendation and extrapolation of efficacy from adults to paediatric

		patients from 2 years to less than 12 years of age based on exposure-response similarity assessment. 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.
Other Studies	2	Study 6 Quantitative systems pharmacology (QSP) model of visceral manifestations of GD. Study 7 Pooled safety analysis of venglustat in the paediatric population.
Other Measures	0	Not applicable.

3. Follow-up, completion and deferral of a PIP:

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	31/03/2031
Deferral of one or more studies contained in the paediatric investigation plan:	Yes