

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

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

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

MHRA-100278-PIP01-21

Scope of the Application

Active Substance(s)

venglustat

Condition(s)

Treatment of Fabry Disease

Pharmaceutical Form(s)

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 20/02/2026 16:22 GMT an application for a Paediatric Investigation Plan

The procedure started on 27/02/2026 14:29 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-100278-PIP01-21

Of 25/08/2026 16:07 BST

On the adopted decision for venglustat (MHRA-100278-PIP01-21) 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, Tablet , ORAL USE .

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

ANNEX I

1. Waiver

1.1 Condition:

Treatment of Fabry disease The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age Pharmaceutical form(s): Tablet Route(s) of administration: Oral use Reason for granting waiver: 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(s):

Treatment of Fabry disease

2.2 Indication(s) targeted by the PIP:

Treatment of Fabry disease.

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):

Tablet

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Quality study to develop an age-appropriate oral solid dosage form (small size chewable tablets).
Non-Clinical Studies	3	Study 2 (DPN0381) Pre- and postnatal developmental toxicity study in Sprague Dawley Rats. Study 3 (GT-373-TX-23) Definitive juvenile toxicity study in Sprague Dawley Rats. Study 4 (JUV0046) Definitive juvenile toxicity study in Sprague Dawley Rats.
Clinical Studies	2	Study 5 (EFC17045/ PERIDOT) Double-blind, randomised, placebo-controlled trial to evaluate effect of venglustat on neuropathic and abdominal pain symptoms in participants from 16 years of age (and adults) with Fabry disease who are treatment naïve or untreated for at least 6 months prior to screening. Study 6 Open-label, single arm trial to evaluate safety, pharmacokinetics, pharmacodynamics and activity of venglustat in male and female patients aged from 2 years to less than 18 years with Fabry disease who are treatment naïve, previously treated, or currently receiving an approved therapy for Fabry disease.
Extrapolation, Modeling & Simulation Studies	3	Study 7 (SIM0452) Population PK modelling and simulation study using data from healthy adults and adults with Fabry disease to inform paediatric dosing in Study 6. Study

		8 Population PK and PD modelling and simulation study incorporating paediatric exposure/ response data to support the extrapolation and posology recommendation for paediatric patients from 2 years to less than 18 years of age. Extrapolation Plan Studies 6, 7, 8 and 9 are part of the extrapolation plan of efficacy data from adult and adolescent patients (from 16 years of age) to the paediatric population from 2 years to less than 18 years of age with Fabry disease.
Other Studies	1	Study 9 Analysis of pooled data from completed studies including paediatric participants from 2 years to less than 18 years of age including data from all patients, irrespective of condition, having been exposed to venglustat (regardless of the dose, duration of exposure or co-mediations) to support safety assessment in paediatric participants.
Other Measures	0	Not applicable./yes.

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

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	30/09/2026
Deferral of one or more studies contained in the paediatric investigation plan:	Yes