A Post-Authorization Safety Study (PASS) to Assess Long-term Safety in Patients with Pompe Disease Treated with Avalglucosidase alfa in the Real-World Setting: the SAVANT (Safety assessment of AValglucosidase Alfa as a Novel Treatment) study

First published: 15/11/2023

Last updated: 18/02/2025

Study Ongoing

Administrative details

PURI

https://redirect.ema.europa.eu/resource/107473

EU PAS number

EUPAS107472

Study ID

107473

DARWIN EU® study

No

Study countries

- [ ] Belgium
- [ ] Canada
- [ ] France
- [ ] Germany
- [ ] Italy
- [ ] Netherlands
- [ ] United Kingdom
- [ ] United States

Study description

This PASS is a non-interventional, multicenter, longitudinal study of safety with prospectively collected data in participants with Pompe disease who are receiving ERT with avalglucosidase alfa as per SoC determined by their treating physician and will be followed for safety reporting using the PASS Recommended Schedule of Assessments provided in the protocol as a guidance. The site selection will be worldwide, with participating sites from multiple geographical regions, particularly sites participating in the Pompe Registry and in countries where home infusion is allowed per local regulation. The Pompe Registry (www.registrynxt.com) is a global, multi-center, multi-national, longitudinal, observational, and voluntary program that is currently conducted to track the disease history and outcomes in patients with Pompe disease

Study status

Ongoing

Research institutions and networks

Institutions

Sanofi

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Institution

Contact details

Study institution contact

Trial Transparency Team Trial Transparency Team

Study contact

contact-us@sanofi.com

Primary lead investigator

Trial Transparency Team Trial Transparency Team

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 06/10/2023

Actual: 06/10/2023

Study start date

Planned: 30/06/2024

Actual: 15/07/2024

Date of final study report
Planned: 31/12/2029

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Sanofi

Regulatory

Was the study required by a regulatory body?

No

Is the study required by a Risk Management Plan (RMP)?

EU RMP category 3 (required)

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Main study objective:

Evaluate the important identified risk of IARs including hypersensitivity and anaphylactic reactions

Study drug and medical condition

Name of medicine, other

Nexviazyme

Population studied

Age groups

Term newborn infants (0 – 27 days)

Infants and toddlers (28 days – 23 months)

Children (2 to < 12 years)

Adolescents (12 to < 18 years)

Adults (18 to < 46 years)

Adults (46 to < 65 years)

Adults (65 to < 75 years)

Adults (75 to < 85 years)

Adults (85 years and over)

Estimated number of subjects

100

Study design details

Outcomes

Incidence rate and event rate of participants with IARs Incidence rate and event rate of IARs Cumulative incidence of IARs leading to treatment discontinuation Incidence rate and event rate of medication errors Cumulative incidence of medication errors leading to AE/SAE/treatment discontinuation, Incidence of all AEs during study participation, including but not limited to AESIs and AEs leading to permanent treatment discontinuations, Incidence of severe hypersensitivity reactions, anaphylactic reactions, and severe immune mediated reactions, Incidence of AEs with permanent treatment discontinuation as outcome, Incidence of ADA/NAb over time

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Data analysis plan

The primary analysis approach is descriptive, and the same approach will apply to both the primary and secondary endpoints. No hypothesis testing is planned. Stratification by prior exposure category (ie, avalglucosidase alfa use prior to enrollment, prior use of other ERTs), region (Europe versus rest of the world) and infusion setting (clinic/hospital versus home) will also be performed, given adequate representations in each stratum.

Data management

Data sources

Data sources (types)

Other

Use of a Common Data Model (CDM)

CDM mapping

No

Data quality specifications

Check conformance

Unknown

Check completeness

Unknown

Check stability

Unknown

Check logical consistency

Unknown

Data characterisation

Data characterisation conducted

No