VPRIV® Non-Interventional Study in Patients Previously Treated with Other Enzyme Replacement Therapies (ERTs)/Substrate Reduction Therapies (SRTs) (SHP669-405)

First published: 11/08/2021

Last updated: 25/03/2024

Study | Finalised

Administrative details

EU PAS number

EUPAS42338

Study ID

47175

DARWIN EU® study

No

Study countries

- ☐ Canada
- ☐ Germany

- [ ] Israel
- [ ] Spain

Study description

The main aim of this study is to describe the safety profile of velaglucerase alfa (VPRIV) in participants with Gaucher disease type 1. Participants will be transitioning from other enzyme replacement therapies or substrate reduction therapies to VPRIV. Some participants may have already transitioned to treatment with VPRIV before this study started. In this study, data on VPRIV will be collected from the medical records of participants who already transitioned to VPRIV before this study started. Other participants will join this study when they transition to VPRIV. All participants will be followed to allow for 12 months of observation from time of transition to VPRIV. The study sponsor will not be involved in how participants are treated but will provide instructions on how the clinics will record what happens during the study.

Study status

Finalised

Research institutions and networks

Institutions

Shire

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Multiple centres: 4 centres are involved in the study

Contact details

Study institution contact

Study Contact Shire ClinicalTransparency@shire.com

Study contact

ClinicalTransparency@shire.com

Primary lead investigator

Study Contact Shire

Primary lead investigator

Study timelines

Date when funding contract was signed

Actual: 06/05/2019

Study start date

Actual: 19/09/2019

Data analysis start date

Planned: 01/08/2023

Actual: 17/11/2021

Date of final study report
Planned: 01/02/2024

Actual: 30/03/2022

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Shire

Study protocol

SHP669-405-protocol-original-redact.pdf (1.28 MB)

SHP669-405-protocol-amendment1-redact.pdf (1.26 MB)

Regulatory

Was the study required by a regulatory body?

No

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

Not applicable

Other study registration identification numbers and links

NCT04094181,https://www.clinicaltrials.gov/ct2/show/NCT04094181

Methodological aspects

Study type

Study topic:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Other

If ‘other’, further details on the scope of the study

Treatment patterns

Data collection methods:

Combined primary data collection and secondary use of data

Main study objective:

The main objective is to describe the safety in patients with GD1 transitioning from other ERTs/SRTs to VPRIV across all age groups.

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Phase 4, observational, retrospective/prospective, non-controlled, non-comparative, multicenter study

Study drug and medical condition

Anatomical Therapeutic Chemical (ATC) code

(A16AB10) velaglucerase alfa

velaglucerase alfa

Medical condition to be studied

Gaucher's disease

Population studied

Short description of the study population

Patients with GD1 transitioning from other ERTs/SRTs to VPRIV in a routine clinical practice setting in Canada.

Inclusion Criteria

1. Patients with GD1 and currently being treated with another ERT/SRT for at least 6 months before baseline enrolment.
2. Patient or legally authorized representative has provided written informed consent.

Exclusion Criteria

Patients are excluded from the study if any of the following criteria are met.

1. Patient is at high risk of non-compliance in the investigator's opinion.
2. Patient is in the opinion of the investigator, unsuitable in any other way to participate in this study.
3. Patient is pregnant

Age groups

- Preterm newborn infants (0 – 27 days)

- 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)

Special population of interest

Other

Special population of interest, other

Gaucher disease patients

Estimated number of subjects

24

Study design details

Outcomes

1. Number of Participants with Adverse Events (AEs) Following the Transition From Other ERTs/SRTs to VPRIV, 1. Describe VPRIV treatment dosing and administration patterns for all patients 2. Change From Baseline in Use of Glucosylspingosine (Lyso-Gb1) Biomarker 3. Change From Baseline in Gaucher Disease Questionnaire Score at Month 12

Data analysis plan

The statistical evaluation of all collected data will be done on a descriptive basis. No statistical hypotheses will be tested.

Documents

Study results

SHP669-405-clinical-study-report-redact.pdf (679.82 KB)

Data management

ENCePP Seal

The use of the ENCePP Seal has been discontinued since February 2025. The ENCePP Seal fields are retained in the display mode for transparency but are no longer maintained.

Data sources

Data sources (types)

Other

Data sources (types), other

Prospective patient-based data collection, Retrospective patient-based data collection, Medical records

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