Glybera Registry, long-term safety and efficacy follow-up in Lipoprotein Lipase deficient (LPLD) patients treated with alipogene tiparvovec (GLYBERA®)

First published: 02/04/2013

Last updated: 15/03/2024

Study Finalised

Administrative details

EU PAS number

EUPAS3750

Study ID

11889

DARWIN EU® study

No

Study countries

- [ ] Canada
- [ ] Germany
- [ ] Italy

Netherlands

Study description

The Glybera Registry is an international, prospective, non-interventional, longitudinal, observational PASS open to patients with genetically confirmed LPLD who had been treated with GLYBERA and participated in the LPLD Registry. The Glybera Registry was a continuation of this LPLD Registry, albeit with fewer assessments and decreased burden to the patient, if any. Physicians collected data from participating patients during their routine care, i.e. at entry in the LPLD Registry, on regular intervals during the first 2 years after dosing and subsequently once every 2 years, up to 15 years, or until the patients was lost to follow-up.

Study status

Finalised

Research institutions and networks

Institutions

uniQure biopharma

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Elisabeth Steinhagen-Thiessen

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 02/04/2013

Study start date

Planned: 01/10/2013

Actual: 27/06/2014

Data analysis start date

Planned: 30/06/2015

Date of interim report, if expected

Planned: 14/10/2015

Date of final study report

Planned: 02/04/2029

Actual: 12/01/2024

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

uniQure biopharma B.V.

Regulatory

Was the study required by a regulatory body?

Yes

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

Not applicable

Other study registration identification numbers and links

NCT03293810

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Disease epidemiology

Effectiveness study (incl. comparative)

Safety study (incl. comparative)

Main study objective:

To assess long-term safety of Glybera®

To assess the long-term clinical response of Glybera®

To assess the epidemiology of the disease and the demographics of LPLD patients

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Prospective, non-interventional, observational study

Study drug and medical condition

Name of medicine

GLYBERA

Medical condition to be studied

Lipoprotein deficiency

Lipid metabolism disorder

Hyperchylomicronaemia

Population studied

Age groups

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

16

Study design details

Data analysis plan

The Glybera Registry data analyses are descriptive in nature, consistent with the main objectives. As such, there was no hypothesis-driven analysis and no need for Type I error adjustment.

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)

Electronic healthcare records (EHR)

Other

Data sources (types), other

Prospective patient-based data collection

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