Disease registry for patients with Niemann-Pick Type C disease (NPC Registry)

First published: 30/08/2013

Last updated: 02/07/2024

Study Finalised

Administrative details

EU PAS number

EUPAS4622

Study ID

28331

DARWIN EU® study

No

Study countries

- [ ] Australia
- [ ] Austria
- [ ] Brazil
- [ ] Bulgaria
- [ ] Canada
- [ ] China

- [ ] Czechia
- [ ] France
- [ ] Germany
- [ ] Greece
- [ ] Italy
- [ ] Netherlands
- [ ] Norway
- [ ] Poland
- [ ] Portugal
- [ ] Russian Federation
- [ ] Slovakia
- [ ] Slovenia
- [ ] Spain
- [ ] Sweden
- [ ] Switzerland
- [ ] United Kingdom

Study description

International, prospective, observational, long-term, disease-specific registry for patients with Niemann-Pick Type C disease.Inclusion criteria: - Patients with NP-C disease- Patients and/or legal guardian must be willing to give written informed consent- Where necessary in order to comply with national regulatory requirements, children will be asked for written informed consent.Exclusion criteria: - NP-C patients who died before the Registry was launched

Study status

Finalised

Research institutions and networks

Institutions

Actelion Pharmaceuticals

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Institution

Multiple centres: 112 centres are involved in the study

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Eric Schoenamsgruber

Primary lead investigator

Study timelines

Date when funding contract was signed
Actual: 10/02/2009

Study start date
Actual: 09/09/2009

Date of final study report
Actual: 06/12/2017

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Actelion

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 3 (required)

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Study type:

Non-interventional study

Scope of the study:

Disease epidemiology

Drug utilisation

Safety study (incl. comparative)

Data collection methods:

Combined primary data collection and secondary use of data

Main study objective:

The objectives for all enrolled patients are to describe the natural history/disease course and clinical outcomes and to describe the treatment experience, including longitudinal assessment of outcomes. The objectives for Zavesca-treated patients only are to educate the prescribers on the Zavesca prescribing information, to observe adherence to it and to solicit adverse event reporting.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medical condition to be studied

Niemann-Pick disease

Population studied

Short description of the study population

Patients with Niemann-Pick disease. Patients and/or legal guardian must be willing to give written informed consent.

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

Niemann-Pick disease patients

Estimated number of subjects

463

Study design details

Outcomes

Measures of outcome and long-term changes in outcome will be summarized for all patients and further summarized by treatment group, by diagnosis status and severity at enrolment.- For all patients: Change in the disability status- For Zavesca treated patients: Occurrence of specific safety information and physician’s report on adherence to the prescribing information.

Data analysis plan

Modeling techniques will be used to explore the relationship between potential prognostic factors, treatment and outcomes, and the resulting inferential statistics will be presented. Potential candidate covariates include age at diagnosis, duration of disease since diagnosis, and severity of the disease.

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)

Disease registry

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

Unknown