An observational disease and clinical outcomes registry of patients with lysosomal acid lipase (LAL) deficiency (ALX-LALD-501)

First published: 26/04/2016

Last updated: 24/06/2025

Study Ongoing

Administrative details

EU PAS number

EUPAS13276

Study ID

47904

DARWIN EU® study

No

Study countries

Australia

Belgium

Brazil

Canada
Croatia
Czechia
Denmark
France
Germany
Greece
Ireland
Israel
Italy
Japan
Mexico
Netherlands
Poland
Portugal
Russian Federation
Saudi Arabia
Slovenia
Spain
United Kingdom
United States

Study description

This is an observational, multi-center, international registry designed to collect longitudinal data and create a knowledge base that will be utilized to improve the care and treatment of patients with LAL deficiency.

Study status

Ongoing

Contact details

Study institution contact

Florian Abel adeline.merlet@alexion.com

Study contact

adeline.merlet@alexion.com

Primary lead investigator

Florian Abel

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 30/05/2013

Actual: 30/05/2013

Study start date

Planned: 30/05/2013

Actual: 30/05/2013

Date of final study report

Planned: 30/01/2027

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Alexion Pharmaceuticals Inc.

Study protocol

riskmgtsystem redacted-annex-6-alx-lald-501-3.pdf (1.22 MB)

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 1 (imposed as condition of marketing authorisation)

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Drug utilisation

Effectiveness study (incl. comparative)

Safety study (incl. comparative)

Main study objective:

Further understand the disease, its progression and any associated complications and evaluate the long-term effectiveness and safety of sebelipase alfa.

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Observational registry

Study drug and medical condition

Anatomical Therapeutic Chemical (ATC) code

(A16AB14) sebelipase alfa

sebelipase alfa

Medical condition to be studied

Lysosomal acid lipase deficiency

Population studied

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

Pregnant women

Estimated number of subjects

297

Study design details

Data analysis plan

Data analyses will be periodically conducted to meet requirements (or upon request) of regulatory agencies and reimbursement authorities and for support of scientific manuscripts and/or conference abstracts. This is an observational Registry intended to descriptively document the clinical course of LAL deficiency, thus, no single statistical hypothesis test is planned. Several variables are taken into account like LAL deficiency diagnostic test results, medical/clinical history, clinical chemistry results and quality of life.

Documents

Study publications

Francis M, Balwani M, Balistreri W, D'Antiga L, Fang S, Jones S, Ros E, Abel F,...

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

Subject 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