

# THE ALPHA-MANNOSIDOSIS REGISTRY: A multi-centre, multi-country, non interventional, prospective cohort, in alpha-mannosidosis patients (SPARKLE)

**First published:** 22/03/2019

**Last updated:** 02/07/2024

Study

Ongoing

## Administrative details

### EU PAS number

EUPAS29038

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### Study ID

32877


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### DARWIN EU® study

No

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### Study countries

 Austria

 Belgium

 Denmark

-  France
  -  Italy
  -  Netherlands
  -  Norway
  -  Poland
  -  Spain
  -  Sweden
  -  United Kingdom
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## **Study description**

The Registry is a multi-centre, multi-country, non interventional, study in alpha mannosidosis patients. In connection of Lamzede marketing authorization and its Risk Management Plan, this registry is requested to obtain long term data on effectiveness and safety, furthermore, it will expand the current understanding of alpha-mannosidosis by collecting additional data on alpha-mannosidosis patients despite the therapeutic treatment they are receiving. The enrolment in the Registry will occur during an indefinite timeframe. The duration of the observation period for each patient will be of 15 years. Patients with alpha-mannosidosis receiving and not receiving treatment with Lamzede will be enrolled. If applicable in accordance with routine clinical practice, the following schedule of visits is recommended: Inclusion Visit, approximately 7 days prior to the Baseline visit, in which eligibility criteria will be checked and the informed consent form signed. Baseline Visit, corresponding to the time in which the observational period will start and when the site can also evaluate to possibly start or continue the treatment with Lamzede according to clinical practice, Six-month and Yearly follow-up visits for all patients included in the registry, Unscheduled follow-up visits, such as but not limited to, three months after Lamzede treatment start, or whenever deemed appropriate, according to treating physician's judgement for patients that start Lamzede treatment within one year prior to Registry inclusion. During the following years, routine clinical

visits are recommended to take place at least annually, for all patients. Effectiveness will be evaluated through a Global Treatment Response by aggregating single endpoints in 3 disease relevant domains: pharmacodynamics, functional and quality of life. Safety variables will be also assessed including but limiting to SAEs, ADRs, AEs leading to treatment stop and to death, ADA, IRRs hypersensitivity and Acute renal failure.

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### **Study status**

Ongoing

## Research institutions and networks

### Institutions

[Julia Hennermann](#)

## Contact details

### **Study institution contact**

Franke Chiara [c.franke.consultant@chiesi.com](mailto:c.franke.consultant@chiesi.com)

**Study contact**

[c.franke.consultant@chiesi.com](mailto:c.franke.consultant@chiesi.com)

### **Primary lead investigator**

Julia Hennermann

**Primary lead investigator**

# Study timelines

## **Date when funding contract was signed**

Planned: 31/05/2019

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## **Study start date**

Planned: 31/01/2020

Actual: 10/12/2019

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## **Date of final study report**

Planned: 30/10/2034

# Sources of funding

- Pharmaceutical company and other private sector

# More details on funding

Chiesi Farmaceutici spa

# Study protocol

[CLI-LMZYMAA1-12-APP 16.1.1-00465.pdf](#) (566.24 KB)

[CLI-LMZYMAA1-12-APP 16.1.1-00465\\_Final version approved.pdf](#) (537.25 KB)

# Regulatory

## **Was the study required by a regulatory body?**

Yes

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

EU RMP category 2 (specific obligation of marketing authorisation)

## Other study registration identification numbers and links

Study Code: CLI-LMZYMAA1-12 Procedure number: EMEA/H/C/003922

## Methodological aspects

### Study type

### Study type list

**Study type:**

Non-interventional study

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**Scope of the study:**

Assessment of risk minimisation measure implementation or effectiveness

Effectiveness study (incl. comparative)

**Main study objective:**

To assess the long-term effectiveness and safety of treatment with Lamzede under conditions of routine clinical care. Effectiveness: to estimate the Global Treatment Response rate as percentage of patients qualified as responders by aggregately assessing oligosaccharides in serum, 3MSCT, 6MWT, FVC and QoL. Safety: the rate of AEs in the treated patients.

## Study Design

## **Non-interventional study design**

Other

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### **Non-interventional study design, other**

observational prospective trial

## Population studied

### **Age groups**

- Adolescents (12 to < 18 years)
  - Children (2 to < 12 years)
  - Infants and toddlers (28 days - 23 months)
  - Preterm newborn infants (0 - 27 days)
  - Term newborn infants (0 - 27 days)
  - 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)
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### **Estimated number of subjects**

70

## Study design details

### **Data analysis plan**

Categorical variables will be described by means of absolute and relative frequencies, while continuous variables by means of mean, standard deviation, quartiles, min and max. Analysis will consider data collected at available

observational point, according to clinical practice and clinical judgment. In order to summarise data by time point (e.g. one year after baseline, two years after baseline, etc.), the nearest available evaluation/measurement will be considered (acceptable range).

## 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](#)

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### 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

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**Check completeness**

Unknown

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**Check stability**

Unknown

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**Check logical consistency**

Unknown

## Data characterisation

**Data characterisation conducted**

No