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

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Study Ongoing

Administrative details

EU PAS number

EUPAS29038

Study ID

32877

DARWIN EU® study

No

Study countries

- [ ] Austria
- [ ] Belgium
- [ ] Denmark

- [ ] France
- [ ] Italy
- [ ] Netherlands
- [ ] Norway
- [ ] Poland
- [ ] Spain
- [ ] Sweden
- [ ] United Kingdom

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 hypersitivity and Acute renal failure.

Study status

Ongoing

Research institutions and networks

Institutions

Julia Hennermann

Contact details

Study institution contact

Franke Chiara c.franke.consultant@chiesi.com

Study contact

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

Study start date
Planned: 31/01/2020

Actual: 10/12/2019

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

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

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

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

Non-interventional study design, other

observational prospective trial

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)

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

Data sources (types)

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