BONAPH1DE, A Prospective Observational Study of Patients With Primary Hyperoxaluria Type 1 (PH1)

First published: 27/09/2021

Last updated: 07/05/2025

Study Ongoing

Administrative details

PURI

https://redirect.ema.europa.eu/resource/44283

EU PAS number

EUPAS43242

Study ID

44283

DARWIN EU® study

No

Study countries

☐ Belgium

- [ ] Canada
- [ ] France
- [ ] Germany
- [ ] Israel
- [ ] Italy
- [ ] Netherlands
- [ ] Spain
- [ ] Switzerland
- [ ] United Kingdom
- [ ] United States

Study description

The purpose of this study is to describe the natural history and progression of patients diagnosed with PH1, and to characterize the long-term real-world safety and effectiveness of lumasiran.

Study status

Ongoing

Research institutions and networks

Institutions

United BioSource Corporation (UBC)

- [ ] Switzerland

First published: 25/04/2013

Last updated: 06/03/2024

Institution | Non-Pharmaceutical company | ENCePP partner

Multiple centres: 32 centres are involved in the study

Contact details

Study institution contact

Karien Verhulst

Study contact

kverhulst@alnylam.com

Primary lead investigator

Emily Brouwer

Primary lead investigator

Study timelines

Date when funding contract was signed

Actual: 19/11/2020

Study start date

Planned: 17/12/2021

Actual: 13/12/2021

Data analysis start date
Planned: 30/11/2028

Date of final study report
Planned: 01/02/2030

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Alnylam

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)

Other study registration identification numbers and links

ALN-GO1-007,CT.gov NCT04982393

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Disease epidemiology

Effectiveness study (incl. comparative)

Main study objective:

The main objective of this trial is to describe the natural history and progression of patients diagnosed with PH1, and to characterize the long-term real-world safety and effectiveness of lumasiran.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Name of medicine

OXLUMO

Study drug International non-proprietary name (INN) or common name

LUMASIRAN

Anatomical Therapeutic Chemical (ATC) code

(A16AX18) lumasiran

lumasiran

Medical condition to be studied

Primary hyperoxaluria

Additional medical condition(s)

Primary hyperoxaluria type 1 PH1

Population studied

Short description of the study population

Study will enroll PH1 patients who will be managed and treated per routine clinical practice.

Patients will be enrolled regardless of treatment status.

The target is to enroll 100 lumasiran treated patients.

Age groups

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

Hepatic impaired

Immunocompromised

Pregnant women

Renal impaired

Estimated number of subjects

200

Study design details

Outcomes

The primary outcome of interest is the incidence of adverse events in lumasiran treated patients.

• Incidence of selected events of interest: hepatic events, kidney stones, acute kidney injury events, nephrocalcinosis, chromic kidney disease, kidney failure, and any cardiac, bone, skin, eye, hematological, or neuropathic manifestations due to oxalosis

• 12-Item Short Form Health Survey Version 2 (SF-12 V2) (Standard Version)

• Change in urinary oxalate excretion

• Change in plasma oxalate

Data analysis plan

Statistical analyses will be primarily descriptive in nature. Descriptive statistics for continuous variables will be reported.

Categorical variables will be summarized as number and proportion of the relevant population, and by subgroups where appropriate.

Summary statistics will be presented for the full study population and separately by subgroups.

There will be no pre-defined hypotheses.

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