LOWER: Lomitapide Observational Worldwide Evaluation Registry (AEGR-733-025)

First published: 18/02/2014

Last updated: 14/03/2025

Study Ongoing

Administrative details

EU PAS number

EUPAS5326

Study ID

47071

DARWIN EU® study

No

Study countries

- [ ] Argentina
- [ ] Austria
- [ ] Canada
- [ ] France

- [ ] Greece
- [ ] Italy
- [ ] Netherlands
- [ ] United Kingdom
- [ ] United States

Study description

The registry is designed to evaluate the long-term safety and effectiveness of lomitapide in clinical practice. The objectives of the registry are:
• To evaluate the occurrence of the following in patients treated with lomitapide: Hepatic abnormalities, Gastrointestinal (GI) events, Small bowel, hepatic, colorectal and pancreatic tumours, Events associated with coagulopathy, Major Adverse Cardiovascular Events (MACE) events, Death, including cause of death,
• To evaluate the occurrence and outcomes of pregnancy in females of reproductive potential treated with lomitapide, with or without consultation with a teratologist and regardless of the outcome of the pregnancy (live birth, elective or spontaneous abortion or still birth). The outcomes of primary interest are major congenital anomalies. Refer to Section 7.2.3 for more detail.,
• To evaluate the long-term effectiveness of lomitapide in maintaining control of serum lipid levels in clinical practice.,
• To evaluate whether prescribers of lomitapide enroled at registry sites are following the screening and monitoring recommendations as specified in the product information (PI) and the prescriber educational materials aimed at risk minimisation.

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

Metropolitan Hospital

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Radboud university medical center (Radboudumc)

Netherlands

First published: 30/06/2022

Last updated: 21/03/2025

Institution | Educational Institution | Hospital/Clinic/Other health care facility | ENCePP partner

Assitance Publique des Hopitaux de Marseille (APHM)

France

First published: 01/02/2024

Last updated: 01/02/2024

Institution | Hospital/Clinic/Other health care facility

Erasmus Medical Centre Rotterdam

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Klinikum der Universitaet Muenchen-DEU;

Umberto I' Hospital-ITA;

Azienda Ospedaliera Universitaria â œPoliclinico Paolo Giacconeâ di Palermo-ITA;

Azienda Ospedaliero Universitaria Policlinico di Bari-ITA;

CNR - Regione Toscana-ITA;

Azienda Sanitaria Ospedaliera San Luigi Gonzaga-

ITA;

Ospedale Monaldi-ITA;

Azienda Ospedaliera Universitaria Integrata di Verona-ITA;

Policlinico Federico II-ITA;

Dipartimento Clinica E Terapia Medica Applicata - ITA;

Azienda Ospedaliera "G.Brotzu"-ITA;

Azienda Ospedaliera Universitaria Padova -ITA;

Policlinico S. Orsola-Malpighi-ITA;

UniversitÃ degli Studi di Genova/Policlinico S. Martino-ITA;

Ospedale Bassini-ITA;

Azienda Ospedaliero-Universitaria - Mater Domini-ITA;

Azienda Ospedaliero-Universitaria S. Anna di Ferrara-ITA;

Centre Hospitalier Universitaire Strasbourg-FRA;

Centre Hospitalier Regional Universitaire de Lille-

FRA;

Hopital Louis Pradel-FRA;

Hopital de la Pitie-Salpetriere-FRA

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Beatriz Borredá

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 02/12/2013

Actual: 20/12/2013

Study start date

Planned: 01/03/2014

Actual: 18/03/2014

Data analysis start date
Planned: 18/03/2028

Date of interim report, if expected
Planned: 01/07/2024

Date of final study report
Planned: 13/07/2028

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Amryt, DAC

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)

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:

The registry is designed to evaluate the long-term safety and effectiveness of lomitapide in clinical practice. Objectives: To evaluate the occurrence of the following in patients treated with lomitapide: Hepatic abnormalities, GI events, Small bowel, hepatic, colorectal and pancreatic tumours, Events associated with coagulopathy, MACE events, Death (including cause of death) and Pregnancy.

Study Design

Non-interventional study design

Cohort

Other

Non-interventional study design, other

This registry study is a multi-centre, long-term, prospective, observational cohort study

Study drug and medical condition

Name of medicine

LOJUXTA

Name of medicine, other

Juxtapid

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

LOMITAPIDE

Anatomical Therapeutic Chemical (ATC) code

(C10AX12) lomitapide

lomitapide

Medical condition to be studied

Type IIa hyperlipidaemia

Additional medical condition(s)

MedDRA code for homozygous familial hypercholesterolaemia (HoFH) is 10057100

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

Hepatic impaired

Renal impaired

Estimated number of subjects

300

Study design details

Outcomes

The safety-related events of special interest include: Hepatic abnormalities, GI events, Tumours, Events associated with coagulopathy, Other safety events of interest include: MACE events, Death and cause of death, Pregnancy (both prospectively and retrospectively reported pregnancies will be collected). Effectiveness evaluations: Magnitude of reduction in serum LDL-C from baseline, Absolute and percent change from baseline in total cholesterol, non-HDL-C, apolipoprotein B (apo B), triglycerides (TG), lipoprotein a (Lp(a)), HDL-C, apolipoprotein AI (apo- AI) and very-low-density lipoprotein cholesterol (VLDL-C), Changes in concomitant medications or apheresis treatments.

Data analysis plan

The main objectives of the study are to evaluate the long-term safety and effectiveness of lomitapide under conditions of usual clinical practice, and to evaluate the effectiveness of risk minimisation interventions in mitigating the serious risks of lomitapide in countries where such efforts are in place.

Summary tabulations will be presented that will display the number of observations, mean, standard deviation, median, minimum and maximum for continuous variables and the number and percentage per category for categorical or ordered categorical data. In addition, two-sided 95% confidence intervals will be calculated for all outcomes.

Data management

Data sources

Data sources (types)

Disease registry

Other

Data sources (types), other

Prospective patient-based data collection, risk assessment, retrospective 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