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

First published: 18/02/2014

Last updated: 20/10/2026

Study | Ongoing

Administrative details

EU PAS number

EUPAS5326

Study ID

47071

DARWIN EU® study

No

Study countries

- 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.

• 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

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

ITA- AOU Paolo Giaccone di Palermo

ITA- AOU policlinico di Bari

ITA- Fondazione Toscana Gabriele Monasterio (FTGM)

ITA- AOU San Luigi Gonzaga

ITA- AOU Federico II (for 2 sites)

ITA- Sapienza Università di Roma

ITA- Azienda Ospedaliera G. Brotzu

ITA- AOU Policlinico S. Orsola-Malpighi

ITA- Università degli Studi Genova

ITA- AOUI Verona

ITA- Ospedale Bassini

ITA- AOU Mater Domini Catanzaro

ITA- AOU di Ferrara

ITA - Azienda Ospedaliera di Padova

FRA- CHU de Lille

FRA- CHU de Strasbourg

FRA- CHU - l'Assistance Publique - Hôpitaux de Marseille

FRA- HP - HU La Pitié-Salpêtrière – C.-Foix

FRA- Hopital Louis Pradel;

GRC- University General Hospital of Heraklion

GRC- Metropolitan Hospital

GRC- National and Kapodistrian University of Athens

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Inbar Aviezer

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

Actual: 02/03/2026

Date of final study report
Planned: 15/10/2026

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 topic:

Disease /health condition

Human medicinal product

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)

Data collection methods:

Combined primary data collection and secondary use of data

Study design:

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

Main study objective:

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.
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), Pregnancy and outcomes of the pregnancy.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medicinal product name

LOJUXTA

Medicinal product name, 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 10057080

Population studied

Short description of the study population

patients treated with lomitapide

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

Women of childbearing potential using contraception

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) and outcome of the pregnancy. 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

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

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)

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