WAYLIVRA® Post-Authorisation Safety Study (PASS) and Product Registry (WAY4001)

First published: 10/08/2020

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Study Ongoing

Administrative details

EU PAS number

EUPAS36702

Study ID

47815

DARWIN EU® study

No

Study countries

- [ ] Austria
- [ ] France
- [ ] Germany
- [ ] Greece

- [ ] Italy
- [ ] Netherlands
- [ ] Spain
- [ ] Sweden
- [ ] United Kingdom

Study description

The aim of this study (PASS phase and WAYLIVRA product registry phase) is to further characterise the safety and effectiveness of WAYLIVRA in patients with Familial Chylomicronaemia Syndrome (FCS) under real-world conditions. This study will be conducted in two phases. The first phase of the study is the PASS phase and will be concluded after a study term of 7 years (or earlier) if data collected amounts to 247 person-years of exposure. The exposure population includes patients with genetically confirmed FCS and at high risk for pancreatitis, in whom response to diet and triglyceride lowering therapy has been inadequate. Following the PASS phase, the study will continue as the WAYLIVRA Product Registry, or the Registry phase, which will be conducted throughout the commercial life of the drug, to obtain long-term data on safety and efficacy of WAYLIVRA. In both phases of the study, real world data will be collected on FCS patients who are prescribed WAYLIVRA. This study is designed as a non-interventional observational study. All patients will receive care according to normal clinical practice and clinical care will not be mandated by the protocol. As such, the decision to prescribe WAYLIVRA is separate from the decision to include the patient in the study and patients are not required to undergo any additional diagnostic or monitoring procedures.

Study status

Ongoing

Research institutions and networks

Institutions

Akcea Therapeutics, Inc.

Contact details

Study institution contact

Akcea Therapeutics, Inc. globalregulatoryaffairs@ionis.com

Study contact

globalregulatoryaffairs@ionis.com

Primary lead investigator

Akcea Therapeutics, Inc.

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 27/08/2020

Actual: 27/11/2020

Study start date

Planned: 04/12/2020

Actual: 04/12/2020

Date of final study report

Planned: 31/12/2028

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Akcea Therapeutics, Inc.

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)

Regulatory procedure number

EMEA/H/C/4538

Other study registration identification numbers and links

WAY4001

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Effectiveness study (incl. comparative)

Study design:

The first phase of the study is the PASS phase and will be concluded after a study term of 7 years (or earlier) if data collected amounts to 247 person-years. Following the PASS phase, the study will continue as the WAYLIVRA Product Registry.

Main study objective:

To evaluate the safety of WAYLIVRA on severe thrombocytopenia and bleeding in FCS patients according to the dose recommendation and dose algorithm in the Summary of Product Characteristics.

Study Design

Non-interventional study design

Cohort

Other

Non-interventional study design, other

Cohort Driven, Non-interventional observational study

Study drug and medical condition

Name of medicine

WAYLIVRA

Anatomical Therapeutic Chemical (ATC) code

(C10AX18) volanesorsen

volanesorsen

Additional medical condition(s)

Familial Chylomicronaemia Syndrome (FCS)

Population studied

Age groups

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

200

Study design details

Setting

This study will be conducted in Europe at centers that manage patients with FCS, as well as by physicians specializing in lipid disorders (e.g., endocrinologists, cardiologists).

Eligible patients from each participating site will be invited to participate in the study in order to minimize selection bias. For eligible patients who do not enroll, reason for non-enrolment will be collected.

Outcomes

Rate of Adverse Event Reporting of Severe Thrombocytopenia and Severe Bleeding

Rate and severity of adverse events with a focus on immunological events, hepatoxicity, renal toxicity and severe injection site reactions; Adherence rate to platelet monitoring and association of serious bleeding events; Dose and dose reduction rates; Pregnancy outcomes; Summarization of triglyceride reduction, pancreatitis prevention, and reduction in abdominal pain frequency and severity.

Data analysis plan

A PASS final analysis will be conducted after the collection of 247person/years of exposure to WAYLIVRA. The exposure population includes patients with genetically confirmed FCS and at high risk for pancreatitis, in whom response to diet and triglyceride lowering therapy has been inadequate, at which point a study report will be written. Complete analytical specifications for the study report, including tables and listings, will be included in the SAP, which will be prepared separately.

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

Data sources (types), other

both retrospective and prospective cases will be collected

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