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

First published: 10/08/2020
Last updated: 23/04/2024

Study | Ongoing

Administrative details

PURI
https://redirect.ema.europa.eu/resource/47815

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 5 years in which at least data on 247 person-years of exposure in patients with genetically confirmed FCS and at high risk for pancreatitis, in whom response to diet and triglyceride lowering therapy has been inadequate have been collected. 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 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 institution and networks

Institutions

United BioSource Corporation (UBC)

Switzerland
First published: 25/04/2013
Last updated
06/03/2024

Institution

ENCePP partner

Non-Pharmaceutical company

Contact details

Study institution contact
Medical Information Akcea

Study contact

Medical.info@sobi.com
Primary lead investigator
Janine Collins

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned:
14/08/2020
Actual:
27/11/2020

Study start date
Planned:
11/01/2021
Actual:
04/12/2020

Date of final study report
Planned:
30/09/2026

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Akcea Therapeutics

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 type:
Non-interventional study

Scope of the study:
Assessment of risk minimisation measure implementation or effectiveness
Effectiveness study (incl. comparative)

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 SmPC

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

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
247

Study design details

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 performed after the conclusion of the PASS phase which will be after 5 years in which at least data on 247 person-years of exposure have been collected in 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

Data sources

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