Prospective Registry-Based Study Evaluating the Effectiveness and Safety of Odevixibat in Participants With Alagille Syndrome (ALGS)

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Study Ongoing

Administrative details

PURI

https://redirect.ema.europa.eu/resource/1000000441

EU PAS number

EUPAS1000000441

Study ID

1000000441

DARWIN EU® study

No

Study countries

United States

Study description

This study will collect information from patients with Alagille syndrome (ALGS) as they use odevixibat (Bylvay) in their daily lives. Odevixibat is a medicine that helps patients with ALGS, a rare disease that harms their liver and causes itching.

The main aim of this study is to observe the long-term, everyday effectiveness and safety of the drug odevixibat in patients with Alagille Syndrome (ALGS) who are receiving ongoing treatment.

Study status

Ongoing

Contact details

Study institution contact

Ipsen Clinical Study Enquiries

Study contact

clinical.trials@ipsen.com

Primary lead investigator

Ipsen Medical Director

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 07/05/2024

Actual: 07/05/2024

Study start date
Planned: 17/03/2025

Actual: 22/04/2025

Date of interim report, if expected
Planned: 30/11/2025

Date of final study report
Planned: 30/05/2030

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Ipsen Pharma

Study protocol

CLIN-60240-033_16.1.1 Protocol v4.0_Redacted.pdf(2.03 MB)

Regulatory

Was the study required by a regulatory body?

Yes

Is the study required by a Risk Management Plan (RMP)?

Not applicable

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Effectiveness study (incl. comparative)

Safety study (incl. comparative)

Data collection methods:

Combined primary data collection and secondary use of data

Study design:

This study will collect information from patients with Alagille syndrome (ALGS) as they use odevixibat (Bylvay) in their daily lives.

Main study objective:

To evaluate the incidence of biliary diversion surgery, liver transplantation, all-cause mortality in participants with ALGS chronically treated with odevixibat.

Study Design

Clinical trial regulatory scope

Clinical trial not part of marketing authorisation application or subject to marketing authorisation approval

Post-authorisation interventional clinical trial

Pre-authorisation clinical trial

Non-interventional study design

Case-only

Study drug and medical condition

Name of medicine

BYLVAY

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

ODEVIXIBAT

Anatomical Therapeutic Chemical (ATC) code

(A05AX05) odevixibat

odevixibat

Medical condition to be studied

Alagille syndrome

Population studied

Short description of the study population

Patients diagnosed with ALGS who start treatment with odevixibat (Bylvay) will be enrolled into the registry. Patients who started odevixibat treatment before the implementation of the registry study may also be enrolled.

Age groups

All

Estimated number of subjects

30

Study design details

Outcomes

Primary Outcome Measure:

1. Percentage of participants with Alagille syndrome (ALGS) who are chronically treated with odevixibat and undergo biliary diversion surgery or liver transplantation.

[Time Frame: From first dose to end of study (approximately 5 years data collection)]

2. Surgical biliary diversion-free survival.

Defined as time from the start of odevixibat treatment to the first occurrence of surgical biliary diversion or death.

[Time Frame: From first dose to end of study (approximately 5 years data collection)]

3. Liver transplant-free survival,

Defined as time from the start of odevixibat treatment to the first occurrence of liver transplant or death.

[Time Frame: From first dose to end of study (approximately 5 years data

collection)]

4. Overall survival

Defined as time from the start of odevixibat treatment to death.

[Time Frame: From first dose to end of study (approximately 5 years data collection)]

Secondary Outcome Measure:

5. Change from baseline in Body Mass Index (BMI)

[Time Frame: From first dose to end of study (approximately 5 years data collection)]

6. Percentage of participants with Adverse events (AEs) associated with fat-soluble vitamin (FSV) deficiencies and their possible sequelae.

[Time Frame: From signing of the ICF to the last dose of odevixibat + 180 days]

7. Percentage of participants with suspected hepatotoxic Adverse events (AEs) requiring interruption of odevixibat treatment

[Time Frame: From signing of the ICF to the last dose of odevixibat + 180 days]

8. Percentage of participants with bleeding AEs

[Time Frame: From signing of the ICF to the last dose of odevixibat + 180 days]

9. Percentage of participants with AEs

[Time Frame: From signing of the ICF to the last dose of odevixibat + 180 days]

Data analysis plan

No formal sample size calculations have been performed for this registry-based study. Enrolment will be based on the number of participants prescribed odevixibat and their willingness to participate in the study, but the goal will be to enroll approximately 30 to 45 participants with ALGS.

Data management

Data sources

Data sources (types)

Drug registry

Non-interventional study

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