Prospective Non-Interventional Study Evaluating the Long-term Safety of Odevixibat in Patients With Alagille Syndrome (ALGS)

First published: 08/05/2026

Last updated: 04/08/2026

Study Ongoing

Administrative details

EU PAS number

EUPAS1000000961

Study ID

1000000961

DARWIN EU® study

No

Study countries

European Union

Study description

This study will collect information from patients with Alagille Syndrome (ALGS) who are using odevixibat in their daily lives. Odevixibat is a medication that helps patients with ALGS, a rare disease that affects the liver and causes itching.

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

Study status

Ongoing

Contact details

Study institution contact

Ipsen Clinical Study Enquiries clinical.trials@ipsen.com

Study contact

clinical.trials@ipsen.com

Primary lead investigator

Ipsen Medical Director

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 21/03/2025

Actual: 21/03/2025

Study start date

Planned: 30/09/2026

Actual: 10/07/2026

Date of final study report
Planned: 31/10/2031

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Ipsen Pharma

Study protocol

CLIN-60240-034_16.1.1 Protocol v3.0_Redacted.pdf (7.4 MB)

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/006462

Other study registration identification numbers and links

CLIN-60240-034

https://clinicaltrials.gov/study/NCT07585097

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Safety study (incl. comparative)

Data collection methods:

Primary data collection

Study design:

This study will collect information from patients with ALGS who are using odevixibat in their daily lives. Odevixibat is a medication that helps patients with ALGS, a rare disease that affects the liver and causes itching.

Main study objective:

To evaluate the incidence of adverse events (AEs) and serious adverse events (SAEs) in participants with ALGS chronically treated with odevixibat.

Study Design

Non-interventional study design

Case-only

Study drug and medical condition

Medicinal product name

KAYFANDA

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

Participants with Alagille syndrome (ALGS) who have been prescribed odevixibat by their treating physician will be enrolled into the study. Participants who started odevixibat treatment before study implementation may also be enrolled. The study will be conducted in the EU/EEA.

Age groups

- **In utero**
- **Paediatric Population (< 18 years)**
  - Infants and toddlers (28 days – 23 months)
  - Children (2 to < 12 years)
  - Adolescents (12 to < 18 years)
- **Adult and elderly population (≥18 years)**
  - Adults (18 to < 65 years)
    - Adults (18 to < 46 years)
    - Adults (46 to < 65 years)
  - Elderly (≥ 65 years)
    - Adults (65 to < 75 years)
    - Adults (75 to < 85 years)
    - Adults (85 years and over)

Estimated number of subjects

30

Study design details

Outcomes

Primary outcomes are the percentage of participants experiencing adverse events (AEs) and serious adverse events (SAEs).

Secondary outcomes include diarrhoea-related events, fat-soluble vitamin

deficiencies and related manifestations, suspected hepatotoxicity and related manifestations, changes from baseline in ALT, AST, GGT, blood bilirubin and INR, hospitalisations and treatment discontinuations related to diarrhoea, hepatotoxicity or fat-soluble vitamin deficiency, pregnancy and maternal complications, adverse effects in the foetus/neonate/infant after exposure during pregnancy or lactation, biliary diversion surgery, liver transplantation, death, and switching from odevixibat to maralixibat.

Data analysis plan

No formal sample size calculation was performed. 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 enrol approximately 30 participants with ALGS in the EU/EEA based on current estimates.

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)

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