Registry to Document Treatment Effectiveness, Safety, Including Prospective Long-term Outcomes in Participants with Progressive Familial Intrahepatic Cholestasis (PFIC) who take Odevixibat (Bylvay)

First published: 11/05/2026

Last updated: 11/05/2026

Study Ongoing

Administrative details

EU PAS number

EUPAS1000000975

Study ID

1000000975

DARWIN EU® study

No

Study countries

China

Study description

This registry-based study will collect information from people with Progressive Familial Intrahepatic Cholestasis (PFIC) who take odevixibat (Bylvay) as part of routine clinical care in China.

PFIC is a rare genetic liver disease that affects bile secretion and can cause bile acids to build up in the liver, which may lead to symptoms such as severe itching (pruritus).

Odevixibat was first allowed to be used for PFIC in babies older than 6 months by the European Medicines Agency (EMA) on 16 July 2021 and by the United States Food and Drug Administration (FDA) on 20 July 2021 for itching in babies older than 3 months. Odevixibat is approved for the treatment of pruritus in PFIC and was approved in China on 01 December 2024 for patients 6 months of age and older with PFIC.

The main aim of this registry is to assess long-term real-world safety (based on adverse events) and to describe effectiveness outcomes.

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: 07/10/2025

Actual: 07/10/2025

Study start date

Planned: 16/04/2026

Actual: 16/04/2026

Date of final study report

Planned: 30/04/2031

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Ipsen Pharma

Study protocol

CLIN-60240-032_16.1.1 Protocol Amendment1 v2.0 2025JAN29_Redacted.pdf
(6.85 MB)

Regulatory

Was the study required by a regulatory body?

Yes

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

Not applicable

Other study registration identification numbers and links

CLIN-60240-032

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:

Primary data collection

Study design:

This will be a long-term, observational, prospective, and voluntary participation registry-based study designed to examine the real-world usage of odevixibat for the treatment of PFIC.

Main study objective:

The primary objective of this registry-based study is to evaluate the long-term safety of odevixibat based on adverse events (AEs).

Study Design

Non-interventional study design

Case-only

Study drug and medical condition

Medicinal product name

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

Progressive familial intrahepatic cholestasis

Population studied

Short description of the study population

Participants with Progressive Familial Intrahepatic Cholestasis (PFIC) (all types) who have been prescribed odevixibat by their treating physician will be eligible. Participants who started odevixibat treatment before the implementation of the registry may also be enrolled.

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

20

Study design details

Outcomes

Safety Endpoints:

• All AEs, according to incidence, severity grade, causality, outcome, action taken, and seriousness

7.3.1.2 Effectiveness Endpoints

• Event-free survival (EFS), defined as time from the start of odevixibat treatment to the first occurrence of surgical biliary diversion, liver transplant, or death

• Surgical biliary diversion-free survival, defined as time from the start of odevixibat treatment to the first occurrence of surgical biliary diversion or death

• Liver transplant-free survival defined as time from the start of odevixibat treatment to the first occurrence of liver transplant or death

• Overall survival, defined as the time from the start of odevixibat treatment to death

• Pruritus improvement, described at each patient visit using a (semi-) objective scoring scale to assess the level of pruritus from the start of the odevixibat treatment

• Change from baseline in serum bile acid, assessed by measuring serum bile acid levels at each patient visit

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Data analysis plan

No formal sample size calculations have been performed for this registry-based study.

Enrolment for this registry-based study will be based on the number of

participants prescribed odevixibat, but the goal will be to enrol a minimum of 20 participants with PFIC (all types) in China.

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