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: 26/09/2025

Last updated: 03/02/2026

Study Ongoing

Administrative details

EU PAS number

EUPAS1000000679

Study ID

1000000679

DARWIN EU® study

No

Study countries

Korea, Republic of

Study description

This study will collect information from people with Progressive Familial Intrahepatic Cholestasis (PFIC) as they use odevixibat in their daily lives. Odevixibat is a medicine that helps people with PFIC, a type of rare disease that makes their liver not work well and causes itching and yellow skin. 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. Obevixibat was approved by the Ministry of Food and Drug Safety (MFDS) in South Korea on 23 August 2024.

This study will collect information to see how well and how safe odevixibat is in the long run for participants in South Korea.

Study status

Ongoing

Research institutions and networks

Institutions

Ipsen Pharma

First published: 01/02/2024

Last updated: 01/02/2024

Institution

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

Actual: 25/09/2024

Study start date

Planned: 30/09/2025

Actual: 30/10/2025

Date of final study report

Planned: 31/01/2032

Sources of funding

- Pharmaceutical company and other private sector

Study protocol

clin60240030 16.1.1 protocol v1.0 2024FEB02 final -signed_Redacted.pdf

(581.04 KB)

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

NCT07185919

Link to ClinicalTrials.gov

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Study topic, other:

Observational study

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 study will collect information from people with Progressive Familial Intrahepatic Cholestasis (PFIC) as they use odevixibat in their daily lives.

Main study objective:

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

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

The registry population will comprise participants with PFIC (all types) enrolled into the Ipsen odevixibat PFIC registry. Participants with PFIC who have been prescribed odevixibat by their treating physician will be eligible

---

Age groups

- **In utero**
- **Paediatric Population (< 18 years)**
  - Neonate
    - Preterm newborn infants (0 – 27 days)
    - Term newborn infants (0 – 27 days)
  - 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

10

Study design details

Outcomes

Primary Outcome Measure:

1.Percentage of participants experiencing Adverse Events (AEs)

An Adverse event (AE) is any untoward medical occurrence, temporally associated with the use of study intervention, whether or not related to the study intervention.

[Time Frame: From first ICF signature and up to end of data collection (approximately 7 years of data collection)]

Secondary Outcome Measures:

2. Event-free survival (EFS)

EFS is defined as time from the start of odevixibat treatment to the first occurrence of surgical biliary diversion, liver transplant, or death

[Time Frame: From first ICF signature and up to end of data collection (approximately 7 years of data collection)]

3. Surgical biliary diversion-free survival

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

[Time Frame: From first ICF signature and up to end of data collection (approximately 7 years of data collection)]

4. Liver transplant-free survival

Liver transplant-free survival is defined as time from the start of odevixibat treatment to the first occurrence of liver transplant or death
[Time Frame: From first ICF signature and up to end of data collection (approximately 7 years of data collection)]
5. Overall survival (OS)
OS is defined as the time from the start of odevixibat treatment to death
[Time Frame: From first ICF signature and up to end of data collection (approximately 7 years of data collection)]

Data analysis plan
No formal sample size calculations have been performed for this registry. Enrolment for this registry will be based on the number of participants prescribed odevixibat and their willingness to participate in the registry, but the goal will be to enrol a minimum of 10 participants with PFIC (all types) in South Korea

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