Prospective Registry-Based Study of the Long-Term Safety of Odevixibat in Patients with Progressive Familial Intrahepatic Cholestasis (PFIC)

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Last updated: 06/05/2025

Study Ongoing

Administrative details

EU PAS number

EUPAS106243

Study ID

106458

DARWIN EU® study

No

Study countries

Austria

Belgium

Brazil

- Canada
- China
- France
- Hungary
- India
- Ireland
- Israel
- Italy
- Netherlands
- Poland
- Portugal
- Slovenia
- South Africa
- Spain
- Sweden
- Switzerland
- Türkiye
- United Arab Emirates
- United Kingdom
- United States

Study description

A registry-based safety study to examine the long-term, real-world safety profile of odevixibat in patients with PFIC compared to patients not receiving odevixibat. Data for this study will be obtained from the TreatFIC registry. The overall objectives of this registry-based safety study are to evaluate the long-term safety of odevixibat and to evaluate the impact of odevixibat on the occurrence of severe diarrhoea, the impact of odevixibat on the clinical manifestations of fat-soluble vitamin deficiency, the impact of odevixibat on the effectiveness of fat-soluble drugs, the impact of odevixibat on nutritional status

and the impact of odevixibat on hepatic function and signs of hepatotoxicity.

Study status

Ongoing

Research institutions and networks

Institutions

Ipsen Pharma

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Last updated: 01/02/2024

Institution

TreatFIC Registry

Contact details

Study institution contact

Medical Director clinical.trials@Ipsen.com

Study contact

clinical.trials@Ipsen.com

Primary lead investigator

Medical Director

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 01/12/2022

Actual: 01/12/2022

Study start date
Planned: 01/02/2023

Actual: 21/02/2023

Date of final study report
Planned: 31/12/2026

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Ipsen

Study protocol

A4250-019 - Protocol Version 24 Mar 2023_ Redacted_PDFA.pdf (503.79 KB)

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 3 (required)

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Main study objective:

To assess the long-term, real-world safety profile of odevixibat treatment in patients with PFIC compared to patients not receiving odevixibat (untreated control cohort).

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medicinal product name

BYLVAY

Medicinal product name, other

Odevixibat

Anatomical Therapeutic Chemical (ATC) code

(A05AX05) odevixibat

odevixibat

Medical condition to be studied

Progressive familial intrahepatic cholestasis

Population studied

Age groups

- Infants and toddlers (28 days – 23 months)
- Children (2 to < 12 years)
- Adolescents (12 to < 18 years)
- 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)

Special population of interest

Hepatic impaired

Estimated number of subjects

100

Study design details

Outcomes

Yes: Incidence of: diarrhoea, clinical manifestations of FSV deficiency, change in FSV levels, reports of ineffectiveness of previously effective fat-soluble drugs, new nutritional interventions, clinical manifestations of hepatotoxicity, hospitalizations or discontinuation of treatment due to diarrhoea, FSV deficiency or hepatotoxicity. Changes in ALT, AST bilirubin, INR or growth parameters.

Data analysis plan

Descriptive analysis will be conducted and presented by odevixibat cohort (Patients with PFIC who received odevixibat at any time before or during the study) and control cohort (Patients with PFIC who did not receive odevixibat). Demographic and baseline characteristics of all patients will be described by cohort using mean, standard deviation, median, minimum and maximum for continuous variables and count and percentages for discrete variables. For the safety endpoints, the number of events and incident rates will be calculated. AEs will also be analysed by incident users and prevalent users separately as a part of a subgroup analysis. For clinical laboratory variables, descriptive statistics for results and change from baseline at each follow-up visit (year) will be presented for each cohort.

Documents

Study report

A4250-019_Synopsis_Redacted_PDFA (1).pdf (2.12 MB)

Data management

Data sources

Data source(s), other

TreatFIC Registry, Netherlands

Data sources (types)

Disease registry

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