A Prospective, Observational Cohort Study to Assess Long-Term Safety in Patients Prescribed Epidyolex® with a Focus on Drug-induced Liver Injury (DILI)

First published: 29/06/2022

Last updated: 07/11/2024

Study Ongoing

Administrative details

EU PAS number

EUPAS47466

Study ID

50740

DARWIN EU® study

No

Study countries

Germany

Italy

Spain

United Kingdom

Study description

This is a multicentre, prospective, non-interventional observational cohort study of patients who are planned to receive, or already receiving Epidyolex under real-world conditions of clinical care. PASS objectives include: • To assess DILI and its risk factors of Epidyolex in patients prescribed Epidyolex in standard clinical practice. • To assess the cognitive development/behaviour adverse drug reaction profile of Epidyolex in patients prescribed Epidyolex in standard clinical practice. • To further characterise the overall safety profile of Epidyolex patients in standard clinical practice.

Study status

Ongoing

Research institutions and networks

Institutions

IQVIA

United Kingdom

First published: 12/11/2021

Last updated: 22/04/2024

Institution **Non-Pharmaceutical company** **ENCePP partner**

Contact details

Study institution contact

Vicki Osborne Vicki.osborne@jazzpharma.com

Study contact

Vicki.osborne@jazzpharma.com

Primary lead investigator

Vicki Osborne

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 31/12/2021

Actual: 17/01/2022

Study start date

Planned: 17/03/2023

Actual: 26/05/2023

Date of final study report

Planned: 31/08/2026

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

GW Pharmaceuticals, part of Jazz Pharmaceuticals

Regulatory

Was the study required by a regulatory body?

No

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 DILI and its risk factors of Epidyolex in patients prescribed Epidyolex in standard clinical practice.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medicinal product name

EPIDYOLEX

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

CANNABIDIOL

Anatomical Therapeutic Chemical (ATC) code

(N03AX24) cannabidiol

cannabidiol

Medical condition to be studied

Lennox-Gastaut syndrome

Severe myoclonic epilepsy of infancy

Tuberous sclerosis complex

Population studied

Age groups

- 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)

Estimated number of subjects

230

Study design details

Outcomes

To assess frequency, severity, and outcome of DILI and its risk factors as determined and assessed by standard serum liver tests (Serum ALT, AST, ALP, and total bilirubin levels with fractionation if routinely done) • Incidence of Hy’s Law cases, Number and nature of SAEs and related AEs specific to suicidality (class effect), aggression, euphoria, and impact on cognitive development. • Number and nature of other SAEs • Number and nature of AEs • Changes in weight from baseline • Change in CGIC from baseline.

Data analysis plan

The statistical analyses will be descriptive in nature. Each variable will be summarized descriptively by age group (paediatric versus adults), overall, by prior treatment (whether naïve to treatment or not), and by follow-up study year. Unless stated otherwise, descriptive statistics for continuous variables will include the number of non-missing values (n), mean, SD, median, minimum, maximum, interquartile range, and 95% CI where appropriate. Categorical variables will be summarized by the number and percentage of patients falling in each category. The primary analyses will include all patients from EU member countries who received at least 1 dose of Epidyolex. Supplementary analysis will be performed including EU member countries and the UK.

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)

Other

Data sources (types), other

Prospective patient-based data collection

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