

ELEVATE, a Global Observational Longitudinal Prospective Registry of Patients With Acute Hepatic Porphyria (AHP)

First published: 23/09/2021

Last updated: 10/09/2024

Study

Ongoing

Administrative details

PURI

<https://redirect.ema.europa.eu/resource/43202>

EU PAS number

EUPAS43201

Study ID

43202

DARWIN EU® study

No

Study countries

☐ Belgium

- ☐ France
 - ☐ Germany
 - ☐ Italy
 - ☐ Netherlands
 - ☐ Sweden
 - ☐ Switzerland
 - ☐ Taiwan
 - ☐ United Kingdom
 - ☐ United States
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Study description

This global patient registry is being conducted to characterize the natural history and real-world clinical management of patients with AHP, and to further characterize the real-world safety and effectiveness of givosiran and other approved AHP therapies.

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

Multiple centres: 31 centres are involved in the study

Contact details

Study institution contact

Karien Verhulst

Study contact

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Primary lead investigator

Sophie Zhang

Primary lead investigator

Study timelines

Date when funding contract was signed

Actual: 19/11/2020

Study start date

Actual: 26/04/2021

Data analysis start date

Planned: 30/06/2027

Date of final study report

Planned: 31/10/2027

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Alnylam

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)

Other study registration identification numbers and links

ALN-AS1-006,CT.gov NCT04883905

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Disease epidemiology

Effectiveness study (incl. comparative)

Main study objective:

To characterize the long-term real-world safety of givosiran in patients with all types of AHP, including patients with hepatic and/or renal impairment, adolescents (≥ 12 to < 18 years of age), elderly patients (> 65 years of age), and pregnant or lactating women.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

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

GIVOSIRAN

Medical condition to be studied

Porphyria non-acute

Porphyria acute

Additional medical condition(s)

Acute hepatic porphyria (AHP)

Population studied

Short description of the study population

Study will enroll AHP patients who will be managed and treated per routine clinical practice. Patients will be enrolled regardless of treatment status. The target is to enroll 150 givosiran treated patients.

Age groups

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

Pregnant women

Renal impaired

Estimated number of subjects

300

Study design details

Outcomes

The primary outcomes of interest include the incidence of hepatic events, renal events and pancreatitis. Additional information will be collected on mortality, hospitalization and events leading to treatment discontinuation or dose adjustment.

- The annualized rate of porphyria attacks
 - The 12-Item Short Form Health Survey Version 2 (SF-12 V2) (Standard Version).
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Data analysis plan

Summary statistics will be used to characterize the study population and subgroups of interest (when appropriate). Analysis will be primarily descriptive. There are no protocol-specified hypotheses. Incidence rates of safety outcomes of interest will be provided during givosiran treatment as well as for patients during non-exposed periods. Survival analysis techniques such as Kaplan-Meier curves will be used to summarize time-to-event outcomes. To assess potential risk factors for time to event, Cox proportional hazards models may also be explored.

Data management

Data sources

Data sources (types)

[Disease registry](#)

[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