Post-authorization safety study of iptacopan in adult patients with paroxysmal nocturnal hemoglobinuria (PNH) using data from the non-interventional IPIG PNH Registry

First published: 17/03/2025

Last updated: 04/07/2025

Study Ongoing

Administrative details

EU PAS number

EUPAS1000000457

Study ID

1000000457

DARWIN EU® study

No

Study countries

- ☐ Canada
- ☐ China
- ☐ France

- [ ] Germany
- [ ] Italy
- [ ] Japan
- [ ] Spain
- [ ] Switzerland
- [ ] United Kingdom
- [ ] United States

Study description

This post-authorization safety study (PASS) is an observational single-arm descriptive cohort study based on the secondary use of data collected on iptacopan-treated patients with paroxysmal nocturnal hemoglobinuria (PNH) through the IPIG PNH registry.

The primary objective of the study is to describe the risk of infections due to encapsulated bacteria in patients with PNH treated with iptacopan.

The secondary objectives are to describe the risk of other safety outcomes in patients with PNH treated with iptacopan, and to describe the course of pregnancies exposed to iptacopan and pregnancy outcomes.

In addition, the study aims to describe in an observational setting the proportion of iptacopan-treated PNH patients not compliant with mandatory and recommended vaccinations against encapsulated bacteria.

Study status

Ongoing

Research institutions and networks

Institutions

Novartis Pharmaceuticals

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Novartis Clinical Disclosure Officer

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 01/02/2022

Actual: 01/02/2022

Study start date

Planned: 20/08/2024

Actual: 31/03/2025

Date of final study report
Planned: 31/03/2030

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Novartis Pharma AG

Study protocol

02.01.0201 Protocol_Redacted.pdf(3.23 MB)

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

CLNP023C12003

Methodological aspects

Study type

Study topic:

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Safety study (incl. comparative)

Data collection methods:

Secondary use of data

Study design:

A multinational non-interventional descriptive single-arm cohort study based on secondary analysis of the data collected within the IPIG PNH Registry on iptacopan-treated patients.

Main study objective:

The primary objective of the study is to describe the risk of infections caused by encapsulated bacteria in patients with PNH treated with iptacopan in routine clinical practice.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Name of medicine, other

Fabhalta

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

IPTACOPAN

Anatomical Therapeutic Chemical (ATC) code

(L04AJ08) iptacopan

iptacopan

Medical condition to be studied

Paroxysmal nocturnal haemoglobinuria

Population studied

Short description of the study population

Adult patients with paroxysmal nocturnal haemoglobinuria treated with iptacopan.

Age groups

In utero

Adult and elderly population (≥18 years)

Adults (18 to < 65 years)

Adults (18 to < 46 years)

Adults (46 to < 65 years)

Elderly (≥ 65 years)

Estimated number of subjects

200

Study design details

Comparators

None

Outcomes

- Infections caused by encapsulated bacteria (Neisseria meningitidis, Streptococcus pneumoniae and Haemophilus influenzae)
- Serious infections caused by encapsulated bacteria
- All serious infections
- Proportion of patients with vaccinations against encapsulated bacteria at the start of iptacopan treatment and at each subsequent visit
- Breakthrough hemolysis
- Serious hemolysis following discontinuation of iptacopan (within 14 days from discontinuation)
- Major adverse vascular events including thrombotic events
- Malignancies
- Hyperlipidemia
- Thrombocytopenia
- Serious adverse events
- All-cause mortality
- Pregnancy-related outcomes

Data analysis plan

The analysis in this study will be focused on the while-on-treatment and the hypothetical risk estimands. The treatment policy estimand strategy will not be implemented in this study because subjects discontinuing iptacopan are expected to start another PNH therapy.

Risk will be evaluated in terms of frequency (counts and percentages), cumulative incidence (event probability as a function of time), incidence rates (number of patients with events per 100 patient-years) and occurrence rates

(number of episodes per 100 patient-years). Incidence and occurrence rates will be calculated by year of treatment and for the entire duration of the study.

Subgroup and sensitivity analyses will be performed as described in the study Statistical Analysis Plan.

Documents

Study, other information

05.01.0301 Feasibility Documentation - Registry Based Study Feasibility Assessment _ 11-Jan-2024_Redacted.pdf(537.65 KB)

05.01.0301 Feasibility Documentation - Registry Evaluation and Quality Standards Tool (REQueST) _ 10-Sep-2023_Redacted.pdf(533.11 KB)

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 source(s)

The International PNH Interest Group PNH Registry / The IPIG PNH Registry

Data sources (types)

Disease registry

Use of a Common Data Model (CDM)

CDM mapping

Yes

CDM Mappings

CDM name

CDISC SDTM

CDM website

https://www.cdisc.org/standards/foundational/sdtm

Data quality specifications

Check conformance

Unknown

Check completeness

Unknown

Check stability

Unknown

Check logical consistency

Unknown

Data characterisation

Data characterisation conducted

Yes