# 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**

Novartis Clinical Disclosure Officer

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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

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**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

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**Study type:**

Non-interventional study

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**Scope of the study:**

Assessment of risk minimisation measure implementation or effectiveness

Safety study (incl. comparative)

**Data collection methods:**

Secondary use of data

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**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

**Medicinal product name, 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

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**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

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**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