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





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

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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

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	Infections caused by encapsulated bacteria (Neisseria meningitidis,
St	reptococcus pneumoniae and Haemophilus influenzae)
	Serious infections caused by encapsulated bacteria
	All serious infections
	Proportion of patients with vaccinations against encapsulated bacteria at the
st	art of iptacopan treatment and at each subsequent visit
	Breakthrough hemolysis
	Serious hemolysis following discontinuation of iptacopan (within 14 days
fro	om 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)

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
UHKHUWH

Check logical consistency

Unknown

Data characterisation

Data characterisation conducted

Yes