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

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Data source

Human

Disease registry

## Administrative details

### Administrative details

**Data source ID**

1000000281

**Data holder**

International PNH Interest Group (IPIG)

**Data source type**

Disease registry

**Main financial support**

Funding from industry or contract research

**Care setting**

Hospital outpatient care

**Data source qualification**

If the data source has successfully undergone a formal qualification process (e.g., from the EMA, ISO or other certifications), this should be described.

No

# Contact details

## IPIG Registry Coordinator registry@pnhinterestgroup.org

**Main**

registry@pnhinterestgroup.org

# Data source regions and languages

**Data source countries**

Argentina

Australia

Austria

Belgium

Canada

China

Denmark

Finland

France

Germany

Greece

Italy

Japan

Korea, Republic of

Netherlands

Norway

Spain

Sweden

Switzerland

Taiwan

Türkiye

United Kingdom

United States

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**Data source languages**

English

## Data source establishment

**Data source time span**

**First collection:** 10/05/2024

The date when data started to be collected or extracted.

# Studies

## List of studies that have been conducted using the data source

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

Characterization of Participants treated with Ultomiris and Long term safety outcomes: an IPIG registry based study

A Post-Authorization Safety Study (Pass) to Characterize Safety Events and Special Conditions, Such as Pregnancy and Infant Outcomes, in Paroxysmal

# Data elements collected

## The data source contains the following information

**Disease information**

Does the data source collect information with a focus on a specific disease? This might be a patient registry or other similar initiatives.

Yes

**Disease details**

Paroxysmal nocturnal haemoglobinuria

**Rare diseases**

Are rare diseases captured? In the European Union a rare disease is one that affects no more than 5 people in 10,000.

Yes

**Pregnancy and/or neonates**

Does the data source collect information on pregnant women and/or neonatal subpopulation (under 28 days of age)?

Yes

**Hospital admission and/or discharge**

Yes

**ICU admission**

Is information on intensive care unit admission available?

Yes

### Cause of death

Captured

### Prescriptions of medicines

Not Captured

### Dispensing of medicines

Not Captured

### Advanced therapy medicinal products (ATMP)

Is information on advanced therapy medicinal products included? A medicinal product for human use that is either a gene therapy medicinal product, a somatic cell therapy product or a tissue engineered products as defined in Regulation (EC) No 1394/2007 [Reg (EC) No 1394/2007 Art 1(1)].

No

### Contraception

Is information on the use of any type of contraception (oral, injectable, devices etc.) available?

No

### Indication for use

Does the data source capture information on the therapeutic indication for the use of medicinal products?

Not Captured

### Medical devices

Is information on medicinal devices (e.g., pens, syringes, inhalers) available?

No

## Administration of vaccines

Yes

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## Procedures

Does the data source capture information on procedures (e.g., diagnostic tests, therapeutic, surgical interventions)?

Captured

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## Healthcare provider

Is information on the person providing healthcare (e.g., physician, pharmacist, specialist) available? The healthcare provider refers to individual health professionals or a health facility organisation licensed to provide health care diagnosis and treatment services including medication, surgery and medical devices.

No

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## Clinical measurements

Is information on clinical measurements (e.g., BMI, blood pressure, height) available?

Yes

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## Genetic data

Are data related to genotyping, genome sequencing available?

Not Captured

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## Biomarker data

Does the data source capture biomarker information? The term “biomarker” refers to a broad subcategory of medical signs ( objective indications of medical state observed from outside the patient), which can be measured accurately and reproducibly. For example, haematological assays, infectious disease markers or metabolomic biomarkers.

Not Captured

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## Patient-reported outcomes

Is information on patient-reported outcomes (e.g., quality of life) available?

Yes

### Patient-generated data

Is patient-generated information (e.g., from wearable devices) available?

No

### Units of healthcare utilisation

Are units of healthcare utilisation (e.g., number of visits to GP per year, number of hospital days) available or can they be derived? Units of healthcare utilisation refer to the quantification of the use of services for the purpose of preventing or curing health problems.

Yes

### Unique identifier for persons

Are patients uniquely identified in the data source?

Yes

### Diagnostic codes

Captured

### Diagnosis / medical event vocabulary

MedDRA

### Medicinal product information

Captured

### Medicinal product information collected

Active ingredient(s)

Brand name

Dosage regime

Dose

Route of administration

**Quality of life measurements**

Captured

**Quality of life measurements vocabulary**

EQ5D

other

**Quality of life measurements, other**

FACIT-Fatigue, EORTC-Q30

**Lifestyle factors**

Not Captured

**Sociodemographic information**

Captured

**Sociodemographic information collected**

Age

Country of origin

Ethnicity

Gender

Other

**Sociodemographic information other**

Employment status

# Quantitative descriptors

## Population Qualitative Data

**Population age groups**

All

## Population

**Population size**

450

**Active population size**

450

# Data flows and management

## Access and validation

**Biospecimen access**

Are biospecimens available in the data source (e.g., tissue samples)?

No

**Access to subject details**

Can individual patients/practitioners/practices included in the data source be contacted?

No

**Description of data collection**

A standard set of data at enrollment and follow-up is entered into the electronic data capture (EDC) system of the registry by investigators at sites under a unique patient code.

Patients complete patient reported outcome questionnaires electronically using their own device and unique login.

## Event triggering registration

**Event triggering registration of a person in the data source**

Other

**Event triggering registration of a person in the data source, other**

Consent of patient

**Event triggering de-registration of a person in the data source**

Death

Loss to follow up

Other

**Event triggering de-registration of a person in the data source, other**

Withdrawal of consent

## Data source linkage

**Linkage**

Is the data source described created by the linkage of other data sources (prelinked data source) and/or can the data source be linked to other data source on an ad-hoc basis?

No

## Data management specifications that apply for the data source

### Possibility of data validation

Can validity of the data in the data source be verified (e.g., access to original medical charts)?

Yes

### Data source preservation

Are records preserved in the data source indefinitely?

Yes

### Approval for publication

Is an approval needed for publishing the results of a study using the data source?

Yes

## Common Data Model (CDM) mapping

### CDM mapping

Has the data source been converted (ETL-ed) to a common data model?

No