Paroxysmal Nocturnal Hemoglobinuria (PNH) Registry (M07-001)

First published: 13/08/2020

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Study Ongoing

Administrative details

EU PAS number

EUPAS36476

Study ID

47935

DARWIN EU® study

No

Study countries

- [ ] Argentina
- [ ] Australia
- [ ] Belgium
- [ ] Canada
- [ ] Colombia
- [ ] France

- [ ] Germany
- [ ] Japan
- [ ] Korea, Republic of
- [ ] Netherlands
- [ ] Spain
- [ ] Sweden
- [ ] Switzerland
- [ ] Taiwan
- [ ] Türkiye
- [ ] United Kingdom
- [ ] United States

Study description

The PNH Registry is a global observational, non-interventional study following patients of any age with a diagnosis of PNH or with a detected PNH clone. The goals of the PNH Registry are to compile data on the progression of PNH and to optimize clinical decision making through enhanced understanding of PNH and its treatments.

Study status

Ongoing

Contact details

Study institution contact

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Study contact

Philippe.Gustovic@alexion.com

Primary lead investigator

Philippe Gustovic

Primary lead investigator

Study timelines

Date when funding contract was signed
Actual: 01/09/2007

Study start date
Actual: 14/04/2008

Date of final study report
Planned: 31/12/2025

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Alexion Pharmaceuticals Inc.

Study protocol

M07-001 Am 7_Final_18Jan2019 SIGNED_PG and PDV.pdf (541.8 KB)

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)

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:

The PNH Registry will collect and evaluate safety data specific to the use of Soliris or Ultomiris in patients with PNH. The PNH registry will collect data to characterize the progression of PNH as well as clinical outcomes, mortality and morbidity in all enrolled patients.

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Observational, non-interventional registry

Study drug and medical condition

Medical condition to be studied

Paroxysmal nocturnal haemoglobinuria

Population studied

Age groups

- Adolescents (12 to < 18 years)
- Children (2 to < 12 years)
- Infants and toddlers (28 days – 23 months)
- Preterm newborn infants (0 – 27 days)
- Term newborn infants (0 – 27 days)
- 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)

Estimated number of subjects

6000

Study design details

Data analysis plan

Primary analyses will assess safety endpoints, including occurrence and time to first event for pre-specified events. All SAEs will be collected for treated patients to characterize the long-term safety profile. Primary analysis will also describe treatment discontinuation, dose adjustments and clinical outcomes. Secondary analyses will describe patient population and health-related quality of life assessment. Analyses will be detailed in a Statistical Analysis Plan. Descriptive analyses will be reported for continuous variables, frequencies and percentages will be reported for categorical variables. The total number of events and total person-years during the period of interest will be determined. The predicted event rate will be calculated using Poisson regression with over-dispersion or generalized estimating equations with a log link, as is appropriate. Survival analysis will be used for time to event outcomes. Propensity scores may be used to reduce the bias.

Documents

Study publications

Lee JW, et al. Effectiveness of eculizumab in patients with paroxysmal nocturna…

Schrezenmeier H, et al. Baseline clinical characteristics and disease burden in…

Röth A, et al. Screening and diagnostic clinical algorithm for paroxysmal noctu…

Röth A, et al. Beneficial effects of eculizumab regardless of prior transfusion…

Höchsmann B, et al. Effect of Eculizumab Treatment in Patients with Paroxysmal …

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 sources (types)

Disease registry

Other

Data sources (types), other

Medical records

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