The PNH Registry

Your opportunity to enhance global understanding of PNH
What is PNH?

- Paroxysmal nocturnal hemoglobinuria (PNH) is a progressive, life-threatening disease of chronic hemolysis that causes thrombosis, end organ damage, and impaired quality of life.
- Even in the absence of symptoms, hemolysis is ongoing, and the consequences can be unpredictable, sudden, and potentially fatal.

![Survival of patients with PNH receiving historical supportive care](image)

- Thrombosis and renal failure are leading causes of death.
- PNH may be diagnosed at any age, with median age in the early 30s; diagnosis is typically delayed from 1 to more than 10 years.
- Identify patients with PNH early within the high-risk groups.

- Hemolysis with any of the following:
  - Coombs-negative hemolytic anemia
  - Hemoglobinuria or hemosiderinuria
  - Renal dysfunction

- Aplastic anemia

- Myelodysplastic syndrome
  - Any subtype with hemolysis
  - Hypoplastic
  - Refractory cytopenia

- Cytopenia
  - Any subtype with hemolysis
  - Refractory iron deficiency anemia
  - Nonresponsive to therapy
  - With thrombosis

- Unexplained thrombosis (venous or arterial)
  - Evidence of hemolysis
  - Unusual sites
  - Any cytopenia
  - Nonresponsive to anticoagulant
What is the PNH Registry?

The PNH Registry is an international, observational, noninterventional study collecting data on patients with PNH, regardless of clone size or treatment approach.

The PNH Registry:

- Provides an invaluable opportunity to increase understanding of the natural history of PNH and the safety and efficacy of treatments
- Offers the international community greater insight into a rare disease with potentially devastating consequences

“The breadth and diversity of the [PNH] Registry provides an excellent basis for investigating research questions that may not be answerable from a single institution or country.”

How is the PNH Registry governed?

- The PNH Registry is overseen by an independent, collaborative executive committee of physicians who are highly experienced in managing patients with PNH and who provide scientific advice and advocacy
- Individual physicians are key to data integrity and success of the PNH Registry
- All patient data are de-identified for confidentiality
What are the objectives of the PNH Registry?

- Collect and publish data to characterize the progression of PNH, clinical and patient-reported outcomes, and morbidities and mortality of patients with PNH
- Expand knowledge of PNH natural history and demographics
- Analyze and assess the safety and efficacy of approaches to PNH management
- Raise PNH awareness in the medical community and patient population
- Establish a robust, international database collected in a real-world setting

How extensive is the PNH Registry?

The PNH Registry includes more than 4000* patients worldwide.

Countries include*:

- Argentina
- Australia
- Austria
- Belgium
- Canada
- Chile
- Colombia
- Czech Republic
- Denmark
- Finland
- France
- Germany
- Greece
- Hong Kong
- Ireland
- Israel
- Japan
- Luxembourg
- Malaysia
- Mexico
- Netherlands
- New Zealand
- Norway
- Portugal
- Russia
- Saudi Arabia
- Singapore
- Slovenia
- South Korea
- Spain
- Sweden
- Switzerland
- Taiwan
- Thailand
- Turkey
- United Kingdom
- United States

*As of December 2015.
Who can contribute?

• All physicians managing patients with PNH, regardless of treatment approach

Who is eligible to be enrolled?

• All patients who have been newly or previously diagnosed with PNH or have evidence of positive PNH cells

How are data collected?

The PNH Registry adheres to a uniform process of data collection.

• Data are easily entered by means of a secure Web portal at enrollment and approximately every 6 months thereafter

• Data can be collected during routine visits or entered from patient medical records

• Data entry includes: demographics, medical history, PNH diagnosis, flow cytometry results, symptomatology, safety events of interest, PNH progression, clinical outcomes, quality of life, and pregnancy

• The PNH Registry data are analyzed by a collaborative global scientific board chaired by Peter Hillmen, MD, FRCP, FRCPath, PhD, in England, and are managed by an independent group with expertise in observational research
Data generated by the PNH Registry advance understanding of the disease

**HAEMATOLOGICA 2014:**
“Baseline Characteristics and Disease Burden in Patients in the International Paroxysmal Nocturnal Hemoglobinuria Registry”
—Schrezenmeier et al.

**ASH 2011:**
“Clinical Characteristics of Classic Paroxysmal Nocturnal Hemoglobinuria (PNH) in Pediatric Patients: A Comparison With Classic PNH in Adults. An International PNH Registry Study”
—Urbano-Ispizua et al.

**EHA 2011:**
“Pediatric Diagnosis of Paroxysmal Nocturnal Hemoglobinuria in the International PNH Registry”
—Urbano-Ispizua et al.

**ASH 2010:**
“Use of Blood Transfusions in Paroxysmal Nocturnal Hemoglobinuria Patients With and Without Aplastic Anemia Enrolled in the Global PNH Registry”
—Schrezenmeier et al.

**ASH 2010:**
—Muus et al.

**EHA 2010:**
“Evaluation of Paroxysmal Nocturnal Hemoglobinuria Disease Burden in Patients Enrolled in the International PNH Registry”
—Urbano-Ispizua et al.

**ASH 2009:**
“A Global Registry of Patients With Paroxysmal Nocturnal Hemoglobinuria”
—Brodsky et al.
What are the key benefits of PNH Registry participation?

- Enhance understanding of PNH
- Capture the long-term outcomes of patients in order to better guide and assess treatments
- Expand a robust, international database on PNH
- Contribute to scientific exchange and publications
- Help to define practice patterns
- Promote evidence-based medicine
- Share data and treatment experiences through participation in periodic Registry meetings

Contact your local representative of the PNH Registry to learn more

www.PNHRegistry.com
Enroll your patients in the PNH Registry today

Unite with a global community of physicians in contributing to the largest, most comprehensive registry of patients with PNH.

Contribute to treatment objectives, practice patterns, and best practices.

Support scientific collaboration in the PNH community.

To learn more, contact the PNH Registry at www.PNHRegistry.com.

The PNH Registry is sponsored by Alexion Pharmaceuticals.

References: