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.\(^1,2\)
- In this acquired hematopoietic stem cell disorder, clonal expansion of hematopoietic stem cells with complete or marked loss of the terminal complement inhibitors CD55 and CD59 renders blood cells susceptible to chronic complement-mediated hemolysis.\(^1,3\)
- In a patient population in which half the patients have <30% clone, 1 in 7 patients died within 5 years.\(^5\)
- 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.\(^4\)
- The following patient populations warrant testing as they are at higher risk for PNH:\(^7,8\):
  - Coombs-negative hemolytic anemia
  - Hemoglobinuria
  - Aplastic anemia
  - Refractory anemia-
    - myelodysplastic syndromes
  - Unexplained cytopenias
  - Unexplained thrombosis
    - (venous or arterial)

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 an uncommon disease with potentially devastating consequences.\(^1\)

“The PNH Registry is a robust, effective, and critical tool to collect data to support the further understanding of the pathophysiology, clinical manifestations, and long-term outcomes of patients living with PNH.”

—Robert A. Brodsky, MD
Director, Division of Hematology
Professor of Medicine and Oncology
Johns Hopkins University

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 PNH patients 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 1650* patients worldwide.

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

Countries include*:

- United States
- Canada
- Argentina
- Denmark
- The Netherlands
- UK
- Japan
- South Korea
- Belgium
- France
- Germany
- Spain
- Switzerland
- Luxembourg
- Russia
- Mexico
- Finland
- Sweden
- Austria
- Norway
- Australia
- New Zealand
- Taiwan

*As of August 2012.
Data generated by the PNH Registry advance understanding of the disease

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

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.

Contact your local representative of the PNH Registry to learn more

Phone: 1.800.913.4893
E-mail: pnhregistry@iconplc.com
Enroll your patients in the PNH Registry today

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

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

Support scientific collaboration in the PNH community.

To learn more, contact the PNH Registry at 1.800.913.4893 or pnhregistry@iconplc.com.

The PNH Registry is sponsored by Alexion Pharmaceuticals.