Paroxysmal Nocturnal Hemoglobinuria (PNH) Registry

This study is currently recruiting participants. (see Contacts and Locations)

Verified September 2014 by Alexion Pharmaceuticals

Sponsor:
Alexion Pharmaceuticals

Collaborator:
ICON

Information provided by (Responsible Party):
Alexion Pharmaceuticals

ClinicalTrials.gov Identifier:
NCT01374360

First received: April 15, 2011
Last updated: September 9, 2014
Last verified: September 2014
History of Changes

Purpose

This study is a collection of data to evaluate safety and characterize progression of Paroxysmal Nocturnal Hemoglobinuria (PNH).

Condition
Paroxysmal Nocturnal Hemoglobinuria

Study Type: Observational
Study Design: Observational Model: Cohort
Time Perspective: Prospective

Official Title: Paroxysmal Nocturnal Hemoglobinuria (PNH) Registry

Resource links provided by NLM:

Genetics Home Reference related topics: paroxysmal nocturnal hemoglobinuria

Drug Information available for: Eculizumab

Genetic and Rare Diseases Information Center resources: Myelodysplastic Syndromes Paroxysmal Nocturnal Hemoglobinuria

U.S. FDA Resources

Further study details as provided by Alexion Pharmaceuticals:

Primary Outcome Measures:

- The PNH Registry will collect data to evaluate safety data specific to the use of Soliris. [Time Frame: Ongoing (up to 13 years)] [Designated as safety issue: Yes]

  Primary analyses will assess safety endpoints, including occurrence and time to first event for the following: meningococcal infections, infections with serious outcomes, formation of HAHA to Soliris, malignancy, thrombotic events, pulmonary hypertension, impaired renal function, serious hemolysis, pregnancies, infusion reactions, targeted adverse events, bone marrow transplant, and mortality.

Secondary Outcome Measures:

- PNH Registry will collect data to characterize the progression of PNH as well as clinical outcomes, mortality and morbidity in Soliris and non-Soliris treated patients. [Time Frame: Ongoing (up to 13 years)] [Designated as safety issue: No]

Secondary analyses will include descriptions of patient populations, PNH specific treatments, concomitant medications, progression of disease, PNH clone sites, clinical symptoms, and clinical outcomes.

Estimated Enrollment:	2000
Study Start Date:	January 2007
Estimated Study Completion Date:	December 2020
Estimated Primary Completion Date:	December 2020 (Final data collection date for primary outcome measure)

Groups/Cohorts
Receiving Soliris PNH patients of any age, including minors, that are receiving Soliris
Not receiving Soliris PNH patients of any age, including minors, that are not receiving Soliris

Detailed Description:

Collection of data to evaluate safety and characterize progression of Paroxysmal Nocturnal Hemoglobinuria (PNH).

Eligibility

Genders Eligible for Study:	Both
Accepts Healthy Volunteers:	No
Sampling Method:	Probability Sample

Study Population

PNH Patients

Criteria

Inclusion Criteria:

- Patients of any age, including minors, with a diagnosis of PNH or a detected PNH clone, including patients previously treated with Soliris and withdrawn from treatment. (Subjects under the age of eighteen years must have parent/legal guardian consent. Upon turning eighteen years of age, these subjects must be re-consented).
- Ability to comprehend and sign consent to have data entered in the PNH Registry.

Contacts and Locations

Choosing to participate in a study is an important personal decision. Talk with your doctor and family members or friends about deciding to join a study. To learn more about this study, you or your doctor may contact the study research staff using the Contacts provided below. For general information, see Learn About Clinical Studies.

Please refer to this study by its ClinicalTrials.gov identifier: NCT01374360

Contacts

Contact: Lynn Sanders 203-439-9609 sandersl@alxn.com

Locations

United States, Maryland

Johns Hopkins University Medical Center — **Recruiting**
Baltimore, Maryland, United States

United States, New York

Roswell Park Cancer Institute — **Recruiting**
Buffalo, New York, United States
Contact: Erin Kittelman 716-845-1516 Erin.Kittelman@RoswellPark.org

Sponsors and Collaborators

Alexion Pharmaceuticals

ICON

Investigators

Study Director: Stephen Squinto, PhD Alexion Pharmaceuticals

More Information

No publications provided

Responsible Party:	Alexion Pharmaceuticals
ClinicalTrials.gov Identifier:	NCT01374360 History of Changes
Other Study ID Numbers:	M07-001
Study First Received:	April 15, 2011
Last Updated:	September 9, 2014
Health Authority:	Argentina: Human Research Bioethics Committee Australia: Human Research Ethics Committee Australia: National Health and Medical Research Council Belgium: Ethics Committee Canada: Ethics Review Committee Finland: Ethics Committee France: Comité consultatif sur le traitement de l'information en matière de recherche dans le domaine de la santé France: Conseil National de l'Ordre des Médecins Germany: Paul-Ehrlich-Institut Netherlands: Independent Ethics Committee Norway: Ethics Committee Spain: Ethics Committee Sweden: Regional Ethical Review Board Switzerland: Swissmedic United Kingdom: Research Ethics Committee United States: Food and Drug Administration

Keywords provided by Alexion Pharmaceuticals:
PNH
Paroxysmal Nocturnal hemoglobinuria
Soliris
Eculizumab

Additional relevant MeSH terms:

Hemoglobinuria
Hemoglobinuria, Paroxysmal
Anemia
Anemia, Hemolytic
Bone Marrow Diseases
Hematologic Diseases
Myelodysplastic Syndromes
Proteinuria
Signs and Symptoms
Urination Disorders
Urologic Diseases
Urological Manifestations

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