Home Reported Outcomes in PNH

Last Update: Jan 14, 2025
Home Reported Outcomes in PNH: A Mobile App-Based, Prospective, Observational Program to Evaluate Disease Burden and Treatment Patterns in Paroxysmal Nocturnal Hemoglobinuria in the US
ClinicalTrials.gov Identifier:
NCT06411626
Novartis Reference Number:CLNP023C1US01
See if you Pre-qualify
All compounds are either investigational or being studied for (a) new use(s). Efficacy and safety have not been established. There is no guarantee that they will become commercially available for the use(s) under investigation.

Study Description

The study aims to longitudinally capture the full spectrum of symptoms, treatment utilization, and overall Health-Related Quality of Life (HRQoL) experienced by PNH patients. By primarily utilizing home reported outcomes (HRO) data on symptom burden and treatment usage, supplemented with patient-reported outcome (PRO) measures, the study seeks to establish a new real-world data (RWD) source to understand symptom variability and HRQoL among PNH patients, including those receiving orally administered iptacopan. The study will be prospective and observational, conducted over an initial period of six months per individual from the point of study enrollment. Participants will utilize the Folia mobile app to enroll, consent, and complete all study activities. A hybrid recruitment method of clinic referrals and community referrals will be employed to identify participants, who will be asked to track routine treatment, symptoms, changes in treatment plans, and HRQoL using the Folia Health mobile app. Monthly survey check-ins will be conducted to capture additional data inputs, with the possibility of integrating electronic health record (EHR) and/or claims data.

Condition
Paroxysmal Nocturnal Hemoglobinuria
Overall Status
Recruiting
Number of Participants
128
Start Date
Jun 11, 2024
Completion Date
May 15, 2025
Gender
All
Age(s)
18 Years - 99 Years (Adult, Older Adult)

Interventions

Other

PNH-relevant therapies

This is an observational study. There is no treatment allocation. The decision to initiate PNH-relevant therapies (such as eculizumab, ravulizumab, pegcetacoplan, iptacopan, and others) will be based solely on clinical judgement.

Eligibility Criteria

Inclusion Criteria:

Study participants eligible for inclusion in this study must meet all of the following criteria:

* Aged 18 or older
* US-based with a proficient understanding of and ability to read the English language
* Any patient with a diagnosis of PNH, regardless of symptom or treatment history

Exclusion Criteria:

Study participants who do not fit all inclusion criteria listed above are unable to participate in this study. Outside of required inclusion criteria, there are no other exclusion criteria in order to meet the exploratory nature of the primary endpoint.

United States

Novartis Investigative Site

Recruiting

East Hanover,New Jersey,07936,United States

Worldwide Contacts

If the location of your choosing does not feature any contact detail, please reach out using the information below.

Novartis Pharmaceuticals

Phone: +41613241111
Email: novartis.email@novartis.com

Source URL: *https://prod1.novartis.com/clinicaltrials/study/nct06411626*

List of links present in page

1. https://clinicaltrials.gov/ct2/show/NCT06411626
2. #trial-eligibility
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