Poster# **IR026**

Nicholas Kuypers | nicholas.kuypers@novartis.com

Preliminary Symptom and Treatment Data From an App-Based Study on Paroxysmal Nocturnal Hemoglobinuria

David Dingli, MD, PhD,[1] Connie Zhang, MPH,[2] Samantha McStocker, BS,[2] Amanda Healey, MPH,[2] Nicholas Kuypers, PhD,[3] Hiba Anwar, MPH,[2] Jincy Paulose, MD,[3] Ver Bilano, PhD,[4] Puja Seth,[5] Soyon Lee, PharmD, MPH[3]

[1]Division of Hematology, Mayo Clinic, Rochester, MN, USA; [2]Folia Health, Boston, MA, USA; [3]Novartis Pharmaceuticals Corporation, East Hanover, NJ, USA; [4]Novartis Pharmaceuticals UK, Ltd., London, UK; [5]Long Grove, IL, USA

KEY FINDINGS & CONCLUSIONS

- The range of home-reported outcomes (HROs) selected to track among this initial sample of patients with paroxysmal nocturnal hemoglobinuria (PNH) reveals diverse disease experiences
- This study seeks to establish a novel real-world data source to explore PNH patient priorities, symptom variability, treatment patterns, and health-related quality of life (HRQoL)
- Future analyses will be stratified by treatment use and conducted on the full participant sample to explore symptom burden, treatment satisfaction, and impact on HRQoL

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BACKGROUND

- Paroxysmal nocturnal hemoglobinuria (PNH) is a rare hematologic disorder characterized by the premature destruction of red blood cells[1]
- Understanding its real-world impact on health-related quality of life (HRQoL), symptom burden, and treatment utilization is crucial as new treatments with different mechanisms of action (eg, proximal complement inhibition) and modes of administration (eg, oral) become available
- The Folia Health app has a dual purpose in capturing home-reported outcomes (HROs) to better understand the day-to-day experience of individuals with PNH and enabling participants to use the same data they track for the study to monitor their symptoms, set treatment reminders, and generate customized reports to share with their case team

OBJECTIVES

- The study's primary objective is to assess PNH symptom burden using self-reported HROs
- The study's secondary objective is to evaluate HRQoL impact and treatment utilization patterns
- This analysis summarizes HROs and PNH treatments selected by >50% of participants among an initial sample of study participants

METHODS

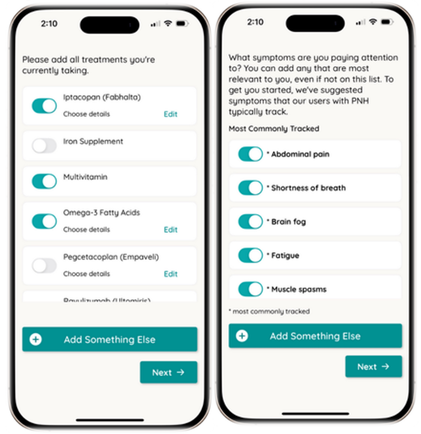
Study Description

- This app-based study assesses patient-reported burden, treatment patterns, and the impact of PNH on HRQoL through self-tracked HROs, including changes from personal baseline
- Prior to study initiation, interviews with patients with PNH and clinicians yielded PNH-specific symptoms, treatments, and measurements to suggest in the app to participants to track over the study period. Participants could also track additional symptoms, treatments, or measurements beyond what was suggested
- Key tracking features suggested by the interviewees include the ability to track how treatments affect quality of life as well as the ability to track flare events in common PNH symptoms (eg, fatigue). Tracked data are summarized in-app to support personal health management
- Patients are compensated for completing app-based study milestones

Study Enrollment and Data Collection

- This ongoing, 6-month prospective, observational study (NCT06411626), designed alongside patients with PNH and clinicians, will enroll ≥128 participants
- Participants are recruited through clinician-based referrals, patient advocacy organizations, and community-based online channels
- At enrollment, participants report baseline health (medical history, treatment history, and demographic information) and customize their Folia Health app profile by adding symptoms they are experiencing and treatments they are taking (eg, iptacopan, danicopan, ravulizumab, pegcetacoplan) (**Figure 1**)
- During the study, participants track symptoms, treatments, and HRQoL through HROs and complete monthly surveys on HRQoL, symptom exacerbation, and Functional Assessment of Chronic Illness Therapy – Fatigue Scale (FACIT-Fatigue)
  - Participants who switch treatments during the study period take an additional FACIT-Fatigue survey at the point of treatment switching

Figure 1. Baseline Profile Setup

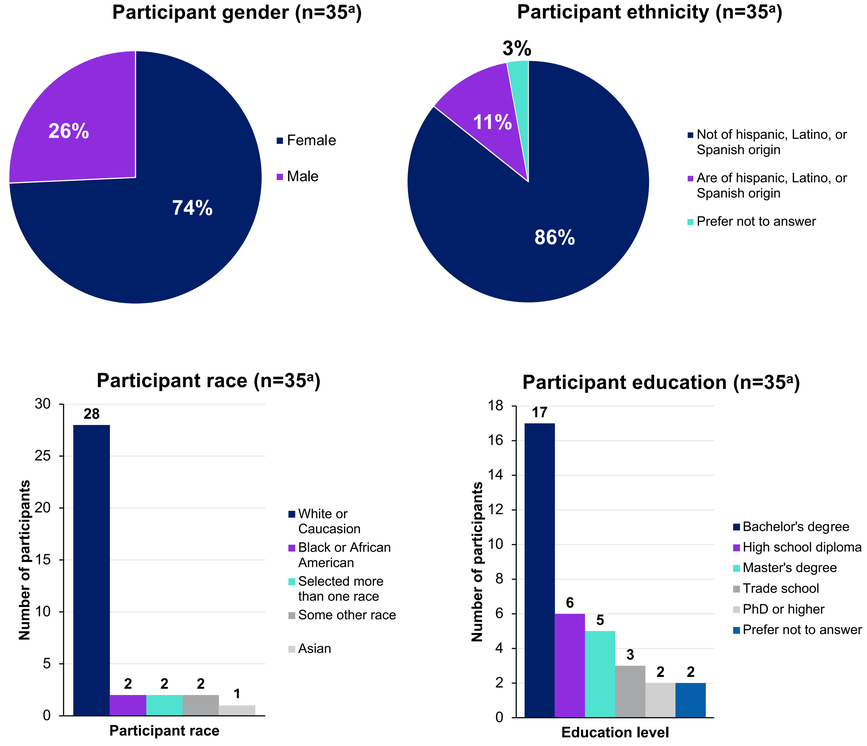


RESULTS

Patient Characteristics

- Enrollment began on June 11, 2024. As of September 2025, a preliminary analysis was performed on symptoms and treatments added by an initial subset of 38 participants to their in-app profile. Baseline demographic data can be found in **Figure 2**.

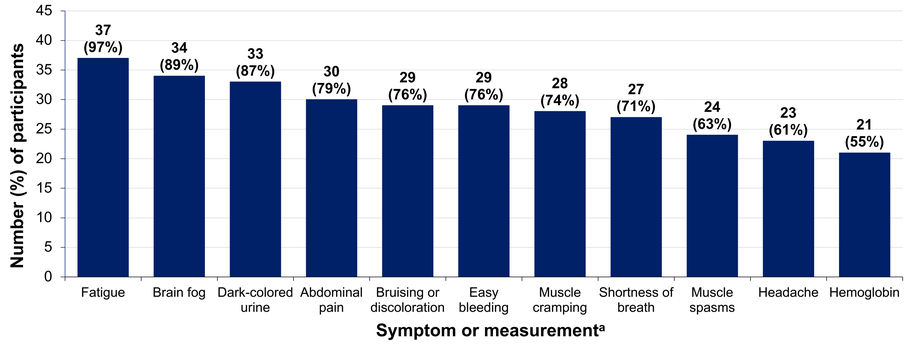
Figure 2. Patient Demographic Characteristics



[a] Three participants did not respond to baseline demographic questions.

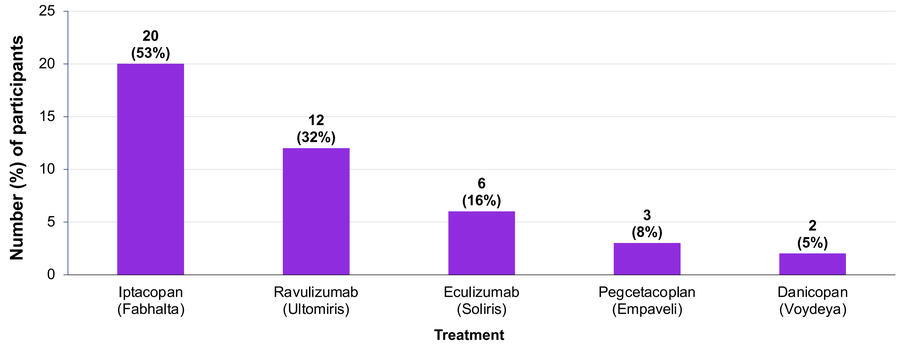
- Across the participant sample (n=38), participants[4] selected to add a total of 80 distinct symptoms and measurements and 57 distinct treatments, including supplements and over-the-counter medications, to their in-app profile to track throughout the study period
  - The most common symptoms selected included fatigue (97%), brain fog (89%), and dark-colored urine (87%) (**Figure 3**)
  - PNH treatments selected to add to profiles included iptacopan (53%), ravulizumab (32%), eculizumab (16%), pegcetacoplan (8%), and danicopan (5%) (**Figure 4**)
- 34 of 38 participants also completed a baseline FACIT-Fatigue survey; mean (SD) FACIT-Fatigue score was 33.26 (12.15), and median (IQR) was 34.75 (19.75), indicating a moderate level of fatigue

Figure 3. Symptoms/Measurements Added to In-App Profile (at baseline or during study period) by >50% of participants (n=38)



[a] Participants also report baseline severity of symptoms added to the profile at the start of the study. Baseline severities are not reported in this table.

Figure 4. PNH Treatments Added to In-App Profile (at baseline or during study period) (n=38)



Reference

1. Bektas M, et al. *J Manag Care Spec Pharm*. 2020;26(suppl 12-b):S3-S8.

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Disclosures

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