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

METHODS

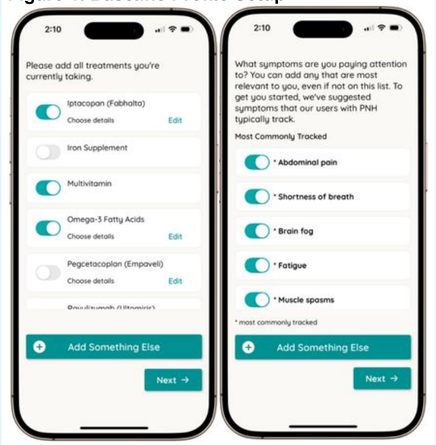
Study Description

- 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
- Interviews with patients with PNH and clinicians yielded PNH-specific symptoms, treatments, and measurements to suggest to participants to track over the study period. Participants can also track additional symptoms, treatments, or measurements beyond what is 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

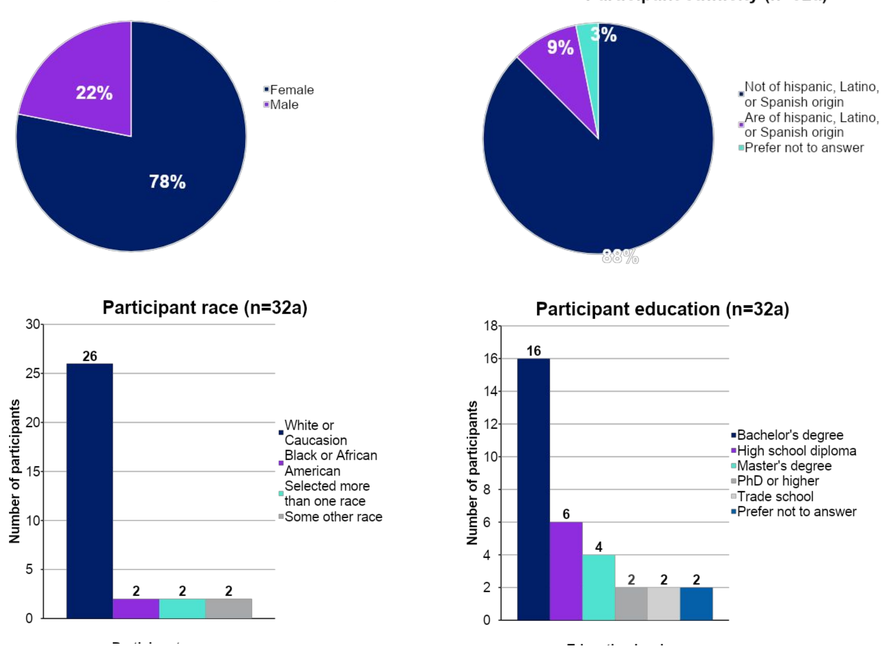


RESULTS

Patient Characteristics

- Enrollment began on June 11, 2024. As of April 22, 2025, 42 participants enrolled, of whom 35 (83%) submitted confirmation of diagnosis and were included in this preliminary analysis of baseline data, including profile setup data. 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=35), participants selected to add a total of 73 distinct symptoms and measurements and 55 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 (94.3%), brain fog (85.7%), and dark-colored urine (82.9%) (**Table 1**)
  - PNH treatments selected to add to profiles included iptacopan (51.4%), ravulizumab (28.6%), eculizumab (14.3%), and pegcetacoplan (8.6%) (**Table 2**)
- 31 of 35 participants also completed a baseline FACIT-Fatigue survey; mean (SD) FACIT-Fatigue score was 33.8 (12.0), and median (IQR) was 35.5 (19.5), indicating a moderate level of fatigue

Table 1. Top 10 Symptoms/Measurements Added to the In-App Profile to Track (n=35)

Rank	Symptom or measurement[a]	No. (%) of participants who added (n=35)
1	Fatigue	33 (94.3%)
2	Brain fog	30 (85.7%)
3	Dark-colored urine	29 (82.9%)
4	Abdominal pain	26 (74.3%)
5	Easy bleeding	26 (74.3%)
6	Bruising or discoloration	25 (71.4%)
7	Muscle cramping	24 (68.6%)
8	Shortness of breath	24 (68.6%)
9	Muscle spasms	20 (57.1%)
10	Hemoglobin	19 (54.3%)

[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.

Table 2. Top 10 Treatments Added to the In-App Profile to Track (n=35)

Rank	Treatment	No. (%) of participants who added (n=35)
1	Iptacopan (Fabhalta)	18 (51.4%)
2	Folic acid	15 (42.9%)
3	Vitamin D	15 (42.9%)
4	Multivitamin	14 (40.0%)
5	Ravulizumab (Ultomiris)	10 (28.6%)
6	Vitamin B12	9 (25.7%)
7	Calcium supplement	6 (17.1%)
8	Eculizumab (Soliris)	5 (14.3%)
9	Iron supplement	4 (11.4%)
10	Pegcetacoplan (Empaveli)	3 (8.6%)

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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