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Clinical Profile of Patients With Paroxysmal Nocturnal Hemoglobinuria (PNH) Not Treated With Complement Inhibitors: Results From A Real-World Study

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KEY FINDINGS & CONCLUSIONS

- In this real-world study, most Ci-naïve patients received supportive treatments as part of disease management.
- Though supportive treatments help in alleviating symptoms to an extent, at the time of survey majority of patients still had suboptimal Hb <12 g/dL and low HRQoL.
- Targeted treatments may help improve clinical and patient outcomes, for those prescribed supportive therapy only.

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INTRODUCTION

- Paroxysmal nocturnal hemoglobinuria (PNH) is a rare clonal, not malignant, hematological disease characterized by intravascular hemolysis, thrombophilia and bone marrow failure.[1]
- Common clinical manifestations of PNH, such as anemia and fatigue have a negative impact on patients' lives.[1]
- Patients are managed by complement inhibitors (Ci) that target component 5 (C5),[2,3] component 3 (C3),[4] factor B[5] or factor D in combination with C5 inhibitors,[6] and/or supportive treatments.
- Use of Ci can regulate complement activation and prevent hemolysis, thereby reducing the risk of major adverse vascular events, improving hemoglobin (Hb) levels (optimal target of ≥12 g/dL) and enhancing health-related quality of life (HRQoL).[7]

OBJECTIVE

- The objective of this real-world study was to investigate the clinical characteristics and patient-reported outcomes (PROs) of PNH patients not treated with Ci (Ci-naïve).

METHODS

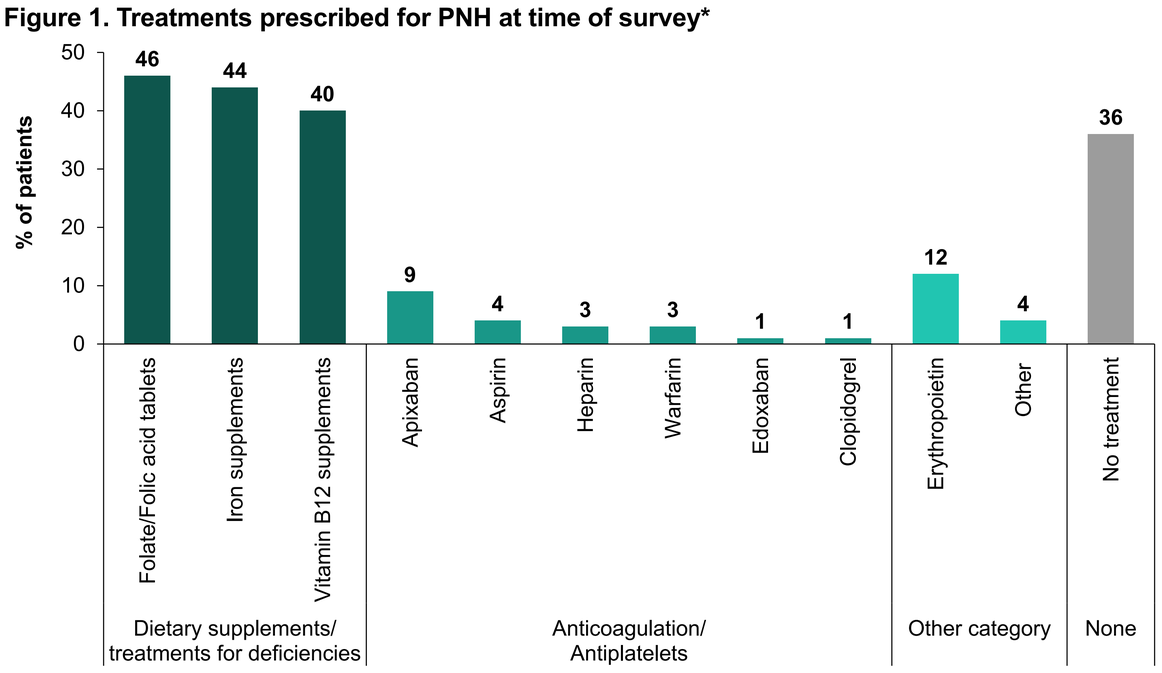
- Data were drawn from Adelphi PNH II Disease Specific Programme ™ (DSP), a cross-sectional survey of physicians and their patients (December 2023-May 2024) across France, Germany, Italy and Spain. DSP methodology has been validated and proved consistent over time.[8-11]
- Hematologists and hematologist-oncologists who managed and made treatment decisions for adult patients with PNH completed a questionnaire for up to their next 10 consecutively consulting patients using information from the consultation, clinical history, and their own clinical judgement.
- Physicians reported data on patients' demographics, clinical parameters, symptoms, and treatments. The same patients, were asked to voluntarily complete a patient survey on demographics, symptoms PROs etc.
- PRO was collected with FACIT-Fatigue questionnaire (scored 0 – 52; higher score indicating less fatigue, general population mean [standard deviation; SD]: 43.5 [8.3], in Germany), EQ-5D 5L and EQ-5D VAS scores.
- Data were collected at diagnosis and survey, analysed using descriptive statistics with no imputation for missing values. For outcomes assessed at diagnosis and survey, results were reported only for patients with data available at both timepoints. Data were presented as median and interquartile range (IQR), mean and standard deviation (SD) or frequency distributions.

RESULTS

Physician-reported clinical profile of Ci-naïve PNH patients:

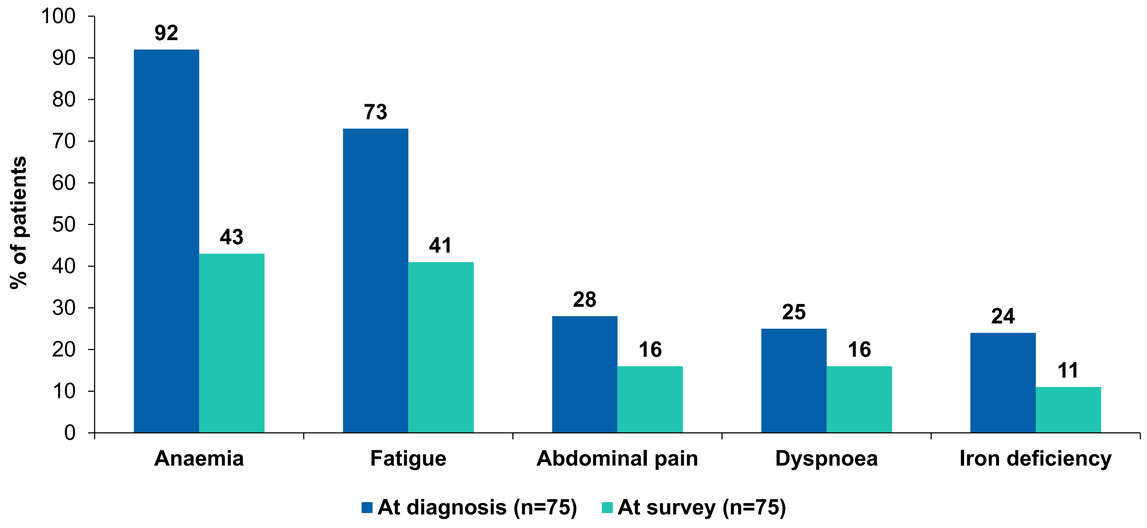
- Thirty-one physicians provided data on 78 Ci-naive patients. Median (IQR) age at survey was 46.0 (36.8-60.0), and 60% were male. Time since diagnosis, and time on supportive treatments was 1.2 (0.5-2.0), and 1.1 (0.7-1.9) years, respectively.
- PNH subtype among all patients (n=77) was 39% subclinical, 38% classical, and 23% PNH in the context of another bone marrow failure syndrome.
- Overall (n=78), 54% of patients had at least one comorbidity at survey.
  - PNH-related comorbidities: myelodysplastic syndrome (14%), aplastic anemia (10%)
  - Non-PNH related comorbidities: diabetes (8%), anxiety (6%), congestive heart failure (5%) etc
- Commonly prescribed supportive therapies at survey (total patients, n=78) were dietary supplements (56%), anticoagulants (21%), while 36% received no treatments **(Figure 1).** Clinical symptoms reported by physicians were presented in **Figure 2.**
- Among the cohort (n=77), 35% had ≥1 blood transfusions within 12-month prior to survey, with a mean (SD) of 2.4 (2.4) transfusions. Of these (n=77), 17% had ≥ 1 hospitalization in the past 12 months, with a mean (SD) of 1.4 (0.9).

Figure 1. Treatments prescribed for PNH at time of survey*



*Treatment options were not mutually exclusive.

Figure 2. Physician reported clinical symptoms at diagnosis and survey



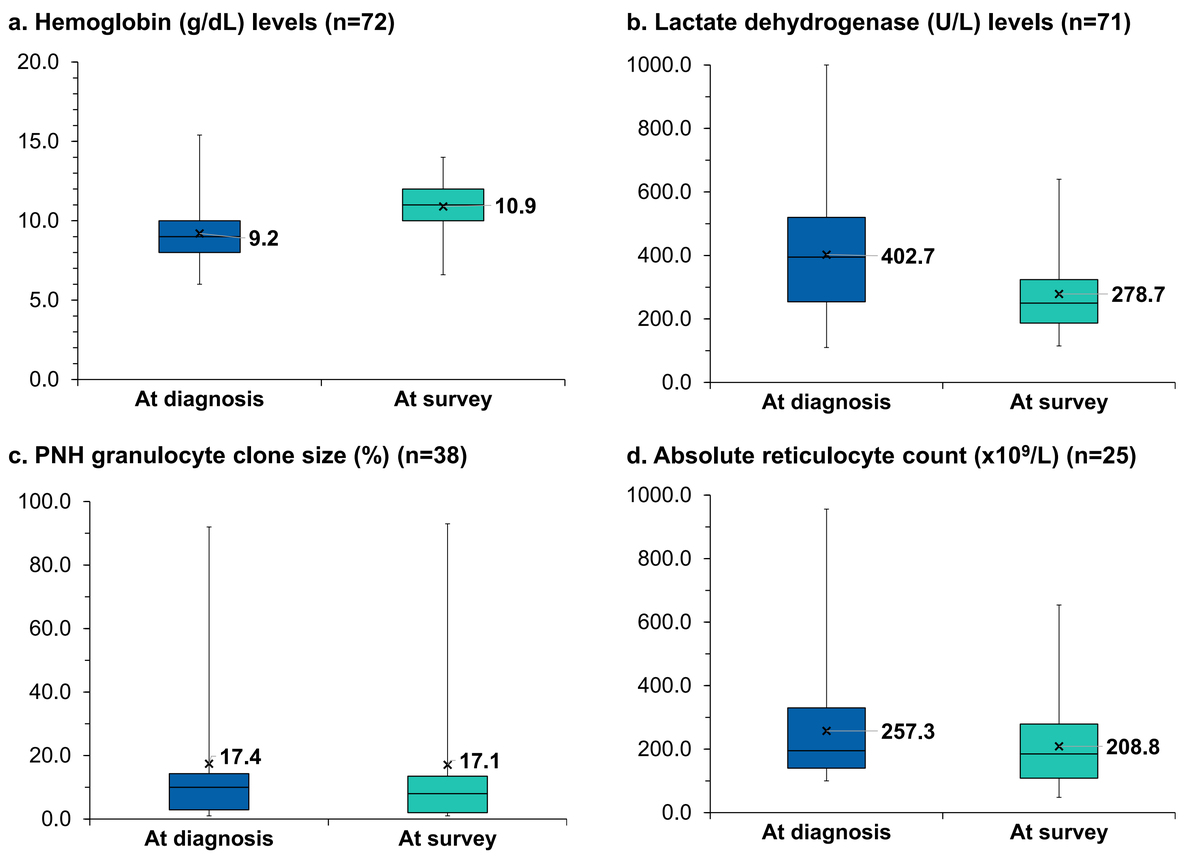
Physician perceived PNH disease severity

- At diagnosis (n=78), physicians perceived PNH patients to have mild (58%), moderate (36%) and severe (6%) disease; at survey they reported mild (83%), moderate (17%) and severe (0%).

Clinical parameters at diagnosis and at survey in Ci-naive cohort

- Overall (n=72), 93% and 7% of patients had Hb <12 g/dL and ≥12 g/dL at diagnosis respectively. While, at survey 72% and 28% of patients had Hb <12 g/dL and ≥12 g/dL. Patients' LDH level at survey were within the normal range (**Figure 3).**

Figure 3. Clinical values at diagnosis and at time of survey



Plot shows range from minimum and maximum values, box shows range from 25th to 75th percentile. Median denoted by horizontal line within the box. Mean denoted by X and is labelled on chart.

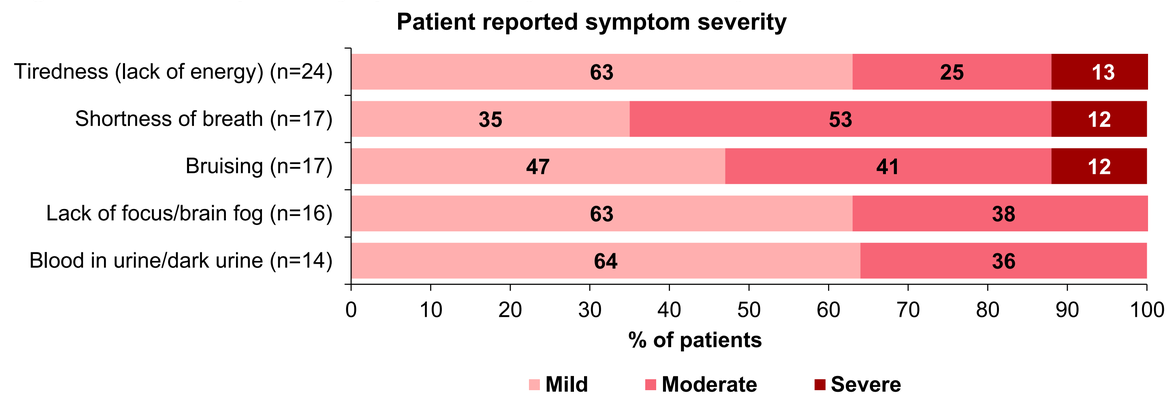
Patients diagnosed for ≥12 months (n=42)

- Of patients diagnosed for ≥12 months, commonly prescribed supportive therapies at survey were dietary supplements (64%), anticoagulants/anti-platelets (21%), while 26% received no treatments.
- Among these patients, 26% had ≥1 blood transfusions in the past 12-months, with a mean (SD) of 1.8 (1.5). Among these patients (n=42) 5% had ≥ 1 hospitalisation in the last 12 months with a mean (SD) of 1.5 (0.7).

Patients-reported symptoms and outcomes at survey

- Twenty-six patients (median [IQR]) age: 46.0 (39.0 - 59.5) years, 54% female) completed the patient survey.
- At lease one current symptom was present in 92% of patients (n=24 out of 26 patients experienced symptoms). Majority experienced tiredness (92%), shortness of breath (65%), and bruising (65%) etc. Patient reported symptom severity is shown in **Figure 4**.
- The mean (SD) FACIT-Fatigue score for all patients was 36.1 (10.1). The mean (SD) EQ-5D 5L and EQ-5D VAS scores were 0.84 (0.16) and 64.5 (18.5) respectively.

Figure 4. Patient reported symptom severity at time of survey*



*Data are being rounded up to 0 decimal point

References

1. Brodsky RA. *Blood* 2014;124:2804–11. **2.** Food and Drug Administration (FDA). Soliris (eculizumab): November 20, 2020. https://www.accessdata.fda.gov/drugsatfda_docs/label/2020/125166s434lbl.pdf. **3.** FDA. Ultomiris (ravulizumab): July 1, 2022. https://www.accessdata.fda.gov/drugsatfda_docs/label/2022/761108s021lbl.pdf. **4.** FDA. Empaveli (pegcetacoplan): February 8, 2023. www.accessdata.fda.gov/drugsatfda_docs/label/2023/215014s002lbl.pdf. **5.** FDA. Fabhalta (iptacopan): https://www.accessdata.fda.gov/drugsatfda_docs/label/2024/218276s001lbl.pdf#page=19. **6.** FDA. Voydeya (danicopan): https://www.accessdata.fda.gov/drugsatfda_docs/label/2024/218037s000lbl.pdf. **7.** Waheed A. *Blood Reviews* 2024; Mar 1;64:101158. **8.** Anderson P, et al. Curr Med Res Opin. 2008;24:3063–72. **9.** Anderson P, et al. Curr Med Res Opin. 2023;39:1707–15. **10.** Babineaux SM, et al. BMJ Open. 2016;6:e010352. **11.** Higgins V, et al. Diabetes Metab Syndr Obes. 2016;9:371–80

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Disclosures

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