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Health-related quality of life and preference for treatment modalities of patients with paroxysmal nocturnal hemoglobinuria (PNH): results of a real-world study

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KEY FINDINGS & CONCLUSIONS

- In this real-world study, patients with PNH, including those treated with Ci, experienced various symptoms such as tiredness, shortness of breath, lack of focus, headaches, and blood in urine.
- Tiredness was the most common and bothersome symptom. The FACIT-Fatigue mean score was below the general population score in both overall and Ci-treated patients.
- Findings suggest unmet need remain for treatment which may lead to better patient outcomes and HRQoL.
- Patients preferred convenient treatments like oral medication. In general administration of treatment at home was preferred to hospital setting.

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INTRODUCTION

- Paroxysmal nocturnal hemoglobinuria (PNH) is a rare, chronic, complement-mediated blood disorder caused by an acquired mutation of the PIG-A gene in the hematopoietic stem cells.[1]
- PNH is characterized by hemolysis, anemia, thrombosis, and other debilitating symptoms such as fatigue and shortness of breath.[1]
- Treatment options for PNH include complement-inhibitor (Ci) infusions such as intravenous complement 5 inhibitors (C5i) or subcutaneous complement 3 inhibitors (C3i), oral monotherapy factor B inhibitor, and oral factor D inhibitor as an add-on to C5i.[2-4]
- The objective of this non-interventional study was to assess symptomatic burden, health-related quality of life and preference for treatment modalities among PNH patients in real-world setting.

METHODS

- Data were drawn from the Adelphi PNH Disease Specific Programme™ Wave II, a cross-sectional survey of physicians and their patients with PNH, conducted in France, Germany, Italy, and Spain (December 2023 to May 2024).[5-8]
- DSP methodology has been validated and proved consistent over time.[5-8]
- Hematologists completed surveys for up to their next 10 consecutively consulting patients diagnosed with PNH. Of those, patients willing to participate were invited to voluntarily complete a patient-reported survey.
- Physicians reported on prescribed treatment at time of survey.
- Patients reported data on demographics, symptoms including their severity and most bothersome, FACIT Fatigue, and treatment modality preference.
- The FACIT-Fatigue questionnaire score ranges from 0 - 52, where higher score indicate less fatigue, with general population norm mean [standard deviation; SD] of 43.5 [8.3], in Germany).[9-11]
- Preference for various treatment modalities (e.g., dosing regimen, administration, and setting) was evaluated on a 5-point response scale ranging from very low to very high preference.
- Patient-reported data were analyzed descriptively with no imputation for missing values.
- Where feasible, results are reported for all respondents (overall) and for Ci-treated.

RESULTS

- A total of 94 patients (median [interquartile range; IQR]) age: 44.0 (34.0 - 57.2) years, 53% male) completed the survey.
- PNH subtype among all patients was 77% classical, 12% subclinical, and 12% PNH with concurrent bone marrow failure (BMF).
- Of patients with reported data on treatment duration, they were on any treatment for a median (IQR): 14 (6.1 - 23.6) months.
- At time of survey, 72% of patients were treated with Ci **(Table 1)**. The median duration (IQR) of Ci among these patients was 13.8 (5.8 - 23.2) months.

Table 1. Complement inhibitor treatments prescribed at time of survey

	n (%)
Prescribed any Ci	68 (72%)
Prescribed C5i	63 (67%)
Eculizumab	45 (71.4%)
Ravulizumab	18 (28.6%)
Prescribed C3i	5 (5%)
Pegcetacoplan	5 (100%)

CI: complement inhibitors; C5i: Complement 5 inhibitors; C3i: Complement 5 inhibitors; n: Number of patients prescribed with complement inhibitors treatment

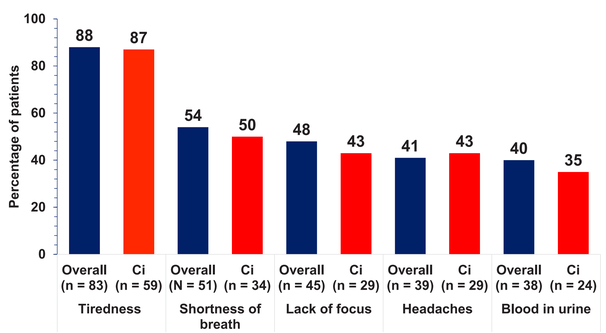
FACIT-Fatigue results

- The mean (SD) FACIT-Fatigue score for all patients (n=92) was 36.6 (9.1) and for Ci-treated (n=66) was 36.8 (8.7)

Patient-reported symptoms

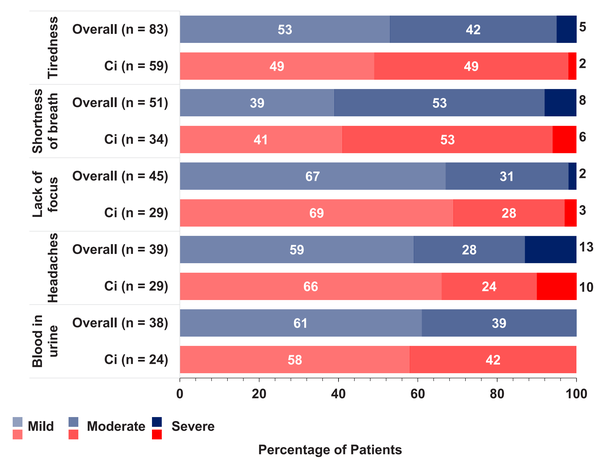
- Most common symptoms reported by overall and Ci-treated patients were tiredness (88% and 87%), shortness of breath (54% and 50%), lack of focus/brain fog (48% and 43%), headache (41% and 43%), and blood in urine/dark urine (40% and 35%). **(Figure 1).**
- The percentage of patients (overall and Ci-treated) reporting symptom severity as moderate-severe were for tiredness (47% and 51%), shortness of breath (61% and 59%), lack of focus/brain fog (33% and 31%), headaches (41% and 34%), and blood in urine/dark urine (39% and 42%) **(Figure 2).**
- A majority of patients (59% of overall (48/81) and 63% of Ci-treated group (38/60)) reported tiredness as the most bothersome symptom.

Figure 1. Patient reported symptoms* at the time of survey



*Top 5 symptoms reported at the time of survey

Figure 2. Percentage of patients reporting symptom severity

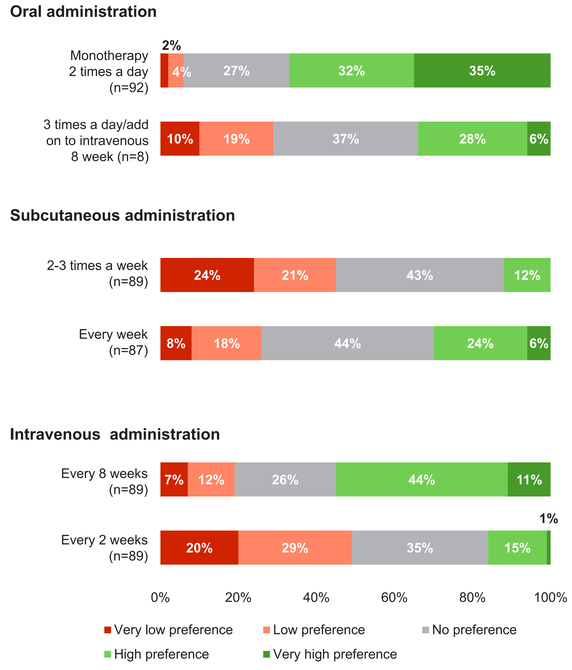


n = number of patients experiencing symptoms

Patient preference for PNH medication

- A majority of patients (67%) had a high/very high preference for oral monotherapy (**Figure 3**).
- For treatments with parenteral administrations, 55% had a high/very high preference for intravenous administration every 8 weeks.
- Administration setting at home was preferred by respondents (63% with high/very high preference).

Figure 3. Patient preference for PNH medication (frequency and route of administration)



n =number of respondents of each question

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Disclosures

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