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Burden Of Transfusion In Patients With Paroxysmal Nocturnal Hemoglobinuria (PNH): Results From A Real-World Study In European Countries

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KEY FINDINGS & CONCLUSIONS

- Majority of transfusion-dependent patients in this study were treated with Ci, yet had suboptimal hemoglobin levels, and a high use of medical resources.
- These patients also experienced persistent symptoms including anemia, fatigue and dyspnea
- The high healthcare resource use, presence of symptoms and decreased quality of life, warrant the need for treatments with higher efficacy and reduced transfusion need.

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INTRODUCTION AND OBJECTIVE

- 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 dyspnea.
- Management of PNH involves complement inhibitors (Ci) and supportive treatments including blood transfusions, iron supplements, folic acid, etc. The burden of transfusion on PNH patients in the real-world is not well-characterized.[1]
- **The objective of this study was to investigate the real-world experience of PNH patients who are transfusion-dependent.**

METHODS

- Data were collected from the Adelphi PNH II Disease Specific Programme™ (Dec 2023-May 2024), a cross-sectional survey in France, Germany, Italy, Spain, and the United Kingdom. DSP methodology has been validated and proved consistent over time. [2-5]
- 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 patient's demographics, clinical parameters, symptoms, healthcare resource use (HCRU).
- Patients reported outcomes included FACIT-Fatigue (score 0-52, higher score indicating less fatigue)[6-8], EQ-5D-VAS (score 0-100)[9], EQ-5D-5L (utility score 0-1; with Germany tariff).[10]
- Data for transfusion dependent patients (defined as having ≥1 blood transfusions in ≥12 months prior to survey and diagnosed for ≥12 months) were analyzed using descriptive statistics, mean (standard deviation, SD), median (interquartile range, IQR); missing data were not imputed.

RESULTS

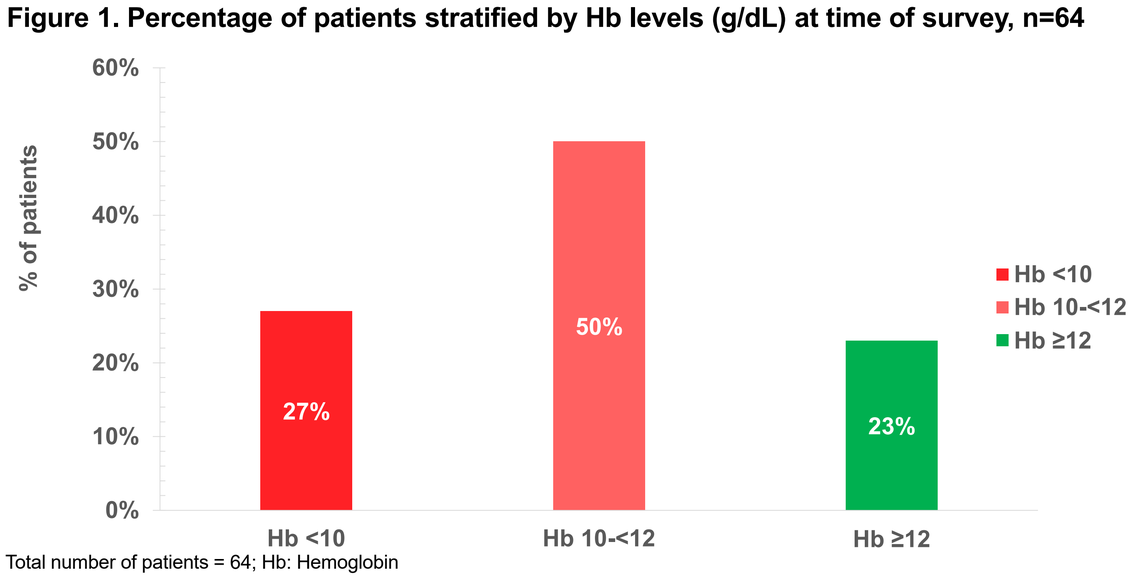
- Out of 240 patients with PNH, 64 patients were transfusion-dependent; their median (IQR) age was 50.5 (38.0–64.0) years, 56% were male, and 53% were employed **(Table 1)**.
- PNH subtype was 80% classical, 17% PNH with concurrent bone marrow failure, and 3% subclinical. Overall, 53% patients had at least one comorbidity at time of survey; aplastic anemia (16%) and depression (14%) being the most common **(Table 1)**.
- A majority (83%) of patients were receiving Ci **(Table 1)**, with a median (IQR) duration of 1.0 (0.7-2.4) years**.**
- In 0–6 months, prior to survey, 47% of patients had ≥1 transfusion (mean [SD]: 1.1 [1.9]); in 6–12 months: 91% had ≥1 transfusion (mean [SD]: 2.2 [2.0]).
- Mean (SD) number of blood units administered during most recent transfusion was 2.0 (0.7).
- At the time of survey, only 23% of patients had Hb ≥12 g/dL (**Figure 1**) and majority had suboptimal Hb level; the median (IQR) Hb was 11.0 (9.9-11.9) g/dL **(Table 1)**.

Table 1. Physician-reported sociodemographic and clinical characteristics

Physician-reported sociodemographic and clinical characteristics	
N = 64	
Age, median (IQR)	50.5 (38.0-64.0)
Sex, male, n (%)	36 (56)
Employed (full-time or part-time), n (%)	34 (53)
Concomitant conditions at time of survey (Top 5), n (%)	
Aplastic anemia	10 (16%)
Anxiety	7 (11%)
Depression	9 (14%)
Diabetes without chronic complications	6 (9%)
Renal disease	5 (8%)
Not diagnosed with any concomitant conditions	30 (47%)
Treatment	
Ci therapy, n (%)	**53 (83%)**
C5i therapy, n (%)	38 (72%)
Eculizumab n (%)	26 (49%)
Ravulizumab n (%)	12 (23%)
C3i therapy (pegcetacoplan), n (%)	15 (28%)
Only on supportive therapy, n (%)	**9 (14%)**
None, n (%)	**2 (3%)**
Number of blood transfusions	
0–6 months, mean (SD)	1.1 (1.9)
6–12 months, mean (SD)	2.2 (2.0)
Laboratory parameters	
Hemoglobin (g/dL)* **At the time of survey,** Median (IQR)	11.0 (9.9-11.9)
Hemoglobin (g/dL), n# **Before most recent transfusion,** Median (IQR)	8.0 (7.0-9.0)

n = Number of patients, IQR: Interquartile range, SD: standard deviation, * most recent, # n=60 due to missing data.
C5i: Complement 5 inhibitors; C3i: Complement 3 inhibitors; g/dL: Grams per deciliter; U/L: Units Per Liter; SD: Standard deviation; IQR: Interquartile Range;

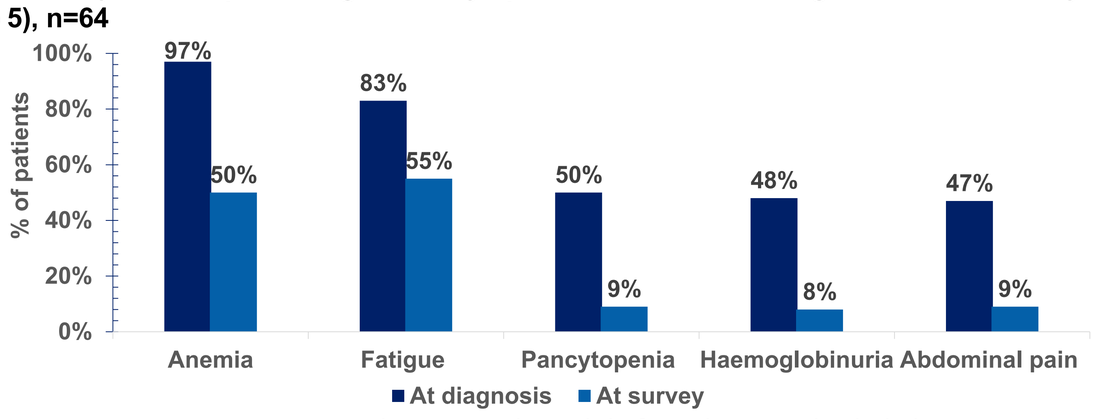
Figure 1. Percentage of patients stratified by Hb levels (g/dL) at time of survey, n=64



Total number of patients = 64; Hb: Hemoglobin

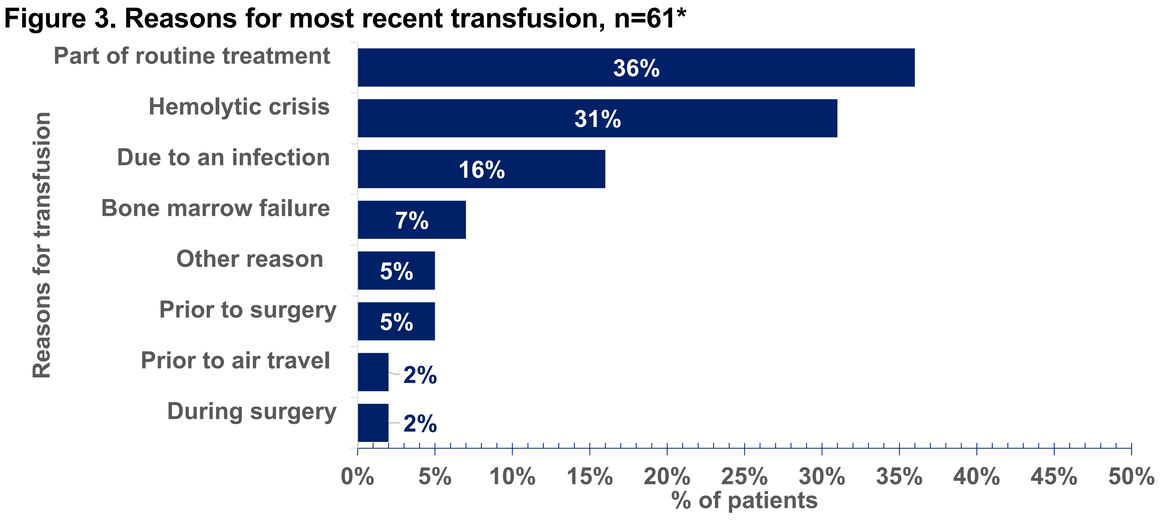
- Physicians reported various signs and symptoms experienced by patients at diagnosis and time of survey, with most common being anemia (97%,50%) and fatigue (83%, 55%) **(Figure 2).**

Figure 2. Physician-reported signs and symptoms at the time of diagnosis and at survey (Top 5), n=64



- The most common symptoms patients experienced before they received their most recent transfusion was fatigue (61%) and dyspnea (36%)
- Common reasons for recent transfusion were 'routine-transfusions' (36%), 'hemolytic crisis' (31%), and 'infections' (16%) (**Figure 3**).

Figure 3. Reasons for most recent transfusion, n=61*

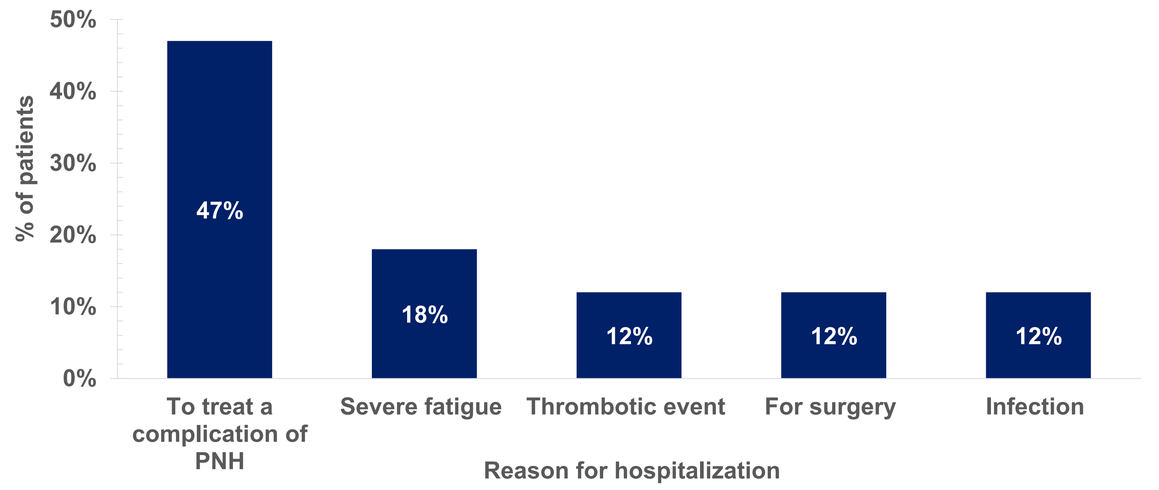


*Total number of patients = 64; Here we present data for 61 patients, reason for transfusion was not reported for 3 patients

Humanistic burden and healthcare resources utilization

- Within 12 months prior to survey (n=61), 28% patients had ≥1 hospitalization, of those (n=17) 35% were admitted via the emergency room. Additionally, 88% required an overnight stay for a mean (SD) 4.6 (1.8) nights. The most frequent reasons for hospitalization were due to treatment of complication of PNH (47%) and severe fatigue (18%) (**Figure 4**).
- Patients (n=12, median [IQR] age: 49.5 [39.3-61.8] years) reported mean (SD) FACIT-Fatigue score of 35.8 (6.0), which is lower than the general population norm mean (SD) of 43.5 (8.3), in Germany).[8]
- Mean (SD) EQ-5D-VAS and EQ-5D utility score were 63.8 (14.8), and 0.78 (0.17), respectively.

Figure 4. Reason for most recent hospitalization, n=17



References

1. Brodsky RA. Blood 2014;124:2804–11.
2. Anderson P, et al. Curr Med Res Opin. 2008;24:3063–72.
3. Anderson P, et al. Curr Med Res Opin 2023;39:1707–15.
4. Babineaux SM, et al. BMJ Open. 2016;6:e010352.
5. Higgins V, et al. Diabetes Metab Syndr Obes. 2016;9:371–80.
6. https://www.facit.org/measures/facit-fatigue. Last updated: February: 7- May-2025.
7. Chandran V, et al. Annals of the rheumatic diseases. 2007 Jul 1;66(7):936-9.
8. Montan I, et al. Value in Health. 2018 Nov 1;21(11):1313-21.
9. The European Journal of Health Economics. 2019 Mar 15;20(2):205-16
10. https://euroqol.org/information-and-support/euroqol-instruments/eq-5d-5l/

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Disclosures

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