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Poster# **MDS-1026**

Real-world healthcare resource utilization among patients with paroxysmal nocturnal hemoglobinuria in the United States

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KEY FINDINGS & CONCLUSIONS

- This study provides first insights into all-cause and paroxysmal nocturnal hemoglobinuria (PNH)–specific healthcare resource utilization (HCRU) among patients with PNH in the US real-world setting
- More complement inhibitor (CI)-untreated patients had all-cause inpatient hospitalizations and longer median length of stay compared to CI-treated patients
- Very few patients receiving iptacopan experienced emergency room (ER) or inpatient hospitalizations relative to those receiving other CI therapies
- Inpatient hospitalizations and length of stay are typically key cost drivers; therefore, lower rates may indicate a reduced healthcare burden

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BACKGROUND

- Paroxysmal nocturnal hemoglobinuria (PNH) is a rare, life-threatening, hematologic disorder characterized by the premature destruction of red blood cells[1]
- Although new therapies have been approved in recent years that can provide complete control of hemolysis,[2] some patients may still require acute medical care due to PNH symptoms
- The impact of the expanded treatment landscape on real-world healthcare resource utilization (HCRU) is not well characterized

OBJECTIVE

- This study describes real-world HCRU among patients with PNH in the United States

METHODS

- This retrospective, observational cohort study used US claims data from the Komodo Healthcare Map™
- The study included adults who had ≥2 claims associated with a PNH diagnosis between January 1, 2018, and June 14, 2024 (**Figure 1**)
  - CI-treated cohort – patients who have ≥2 consecutive claims for any of the following treatments during the identification period (June 14, 2023, to June 14, 2024): eculizumab, ravulizumab, pegcetacoplan, iptacopan, danicopan, or crovalimab
  - CI-untreated cohort – patients who have ≤1 claim for any of the above treatments during the identification period. Rescue treatment for hemolysis during the identification period was permitted in the CI-untreated cohort
- The index date was the date of the first claim for the most recent PNH treatment of interest (index therapy) received within the identification period for the CI-treated cohort and the date of the earliest claim associated with a PNH diagnosis within the identification period for the CI-untreated cohort. Patients were classified into subgroups based on their index therapy (**Figure 2**). Patients were required to have continuous enrollment for ≥90 days prior to index
- All-cause and PNH-specific HCRU (inpatient hospitalizations, emergency room [ER] visits, and outpatient visits) was measured over a 12-month follow-up period post-index or until data cutoff (February 6, 2025), whichever occurred first. Only patients with ≥6 months of follow-up data were included

Figure 1. Study Design

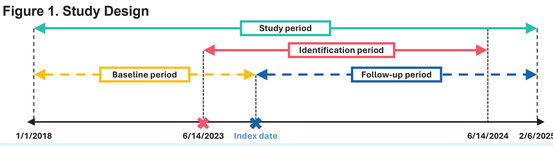
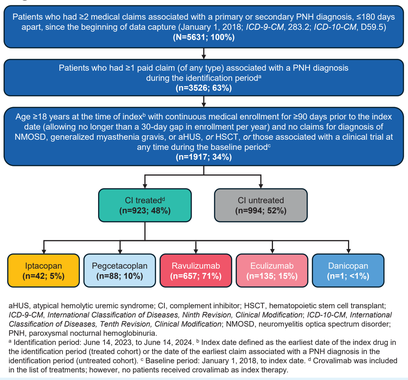


Figure 2. Patient Attrition



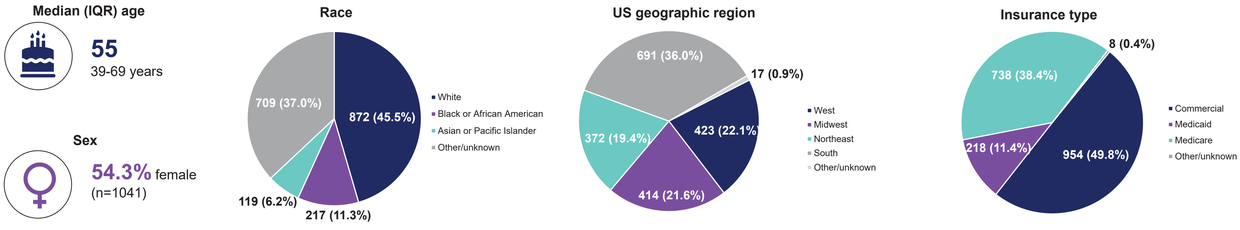
aHUS, atypical hemolytic uremic syndrome; CI, complement inhibitor; HSCT, hematopoietic stem cell transplant; *ICD-9-CM, International Classification of Diseases, Ninth Revision, Clinical Modification*; *ICD-10-CM, International Classification of Diseases, Tenth Revision, Clinical Modification*; NMOSD, neuromyelitis optica spectrum disorder; PNH, paroxysmal nocturnal hemoglobinuria.
[a] Identification period: June 14, 2023, to June 14, 2024. [b] Index date defined as the earliest date of the index drug in the identification period (treated cohort) or the date of the earliest claim associated with a PNH diagnosis in the identification period (untreated cohort). [c] Baseline period: January 1, 2018, to index date. [d] Crovalimab was included in the list of treatments; however, no patients received crovalimab as index therapy.

RESULTS

Patient Characteristics

- Overall, 1917 patients (994 CI untreated and 923 CI treated) were included
- Patient demographics were similar between cohorts; overall, the median age was 55 years, 54.3% were female, 45.5% were White, 11.3% were Black or African American, and 49.8% were covered by commercial insurance (**Figure 3**)
- The distribution of claims for the most recent CI therapy was as follows: ravulizumab (n=657 [71.2%]), eculizumab (n=135 [14.6%]), pegcetacoplan (n=88 [9.5%]), and iptacopan (n=42 [4.6%])
- The median age of the iptacopan cohort was 50 years, 64.3% of patients were female, and 50.0% were White

Figure 3. Baseline Demographics and Clinical Characteristics



CI-Treated vs CI-Untreated Cohorts

Healthcare Resource Utilization

- More CI-untreated patients had all-cause inpatient hospitalizations (12.6% vs 9.6%) and longer all-cause median length of stay per patient per month (0.53 vs 0.33 days) compared to CI-treated patients (**Figure 4; Table 1**)
- The proportion of patients with ≥1 PNH-specific inpatient hospitalization was comparable across CI-untreated and CI-treated cohorts (6.2% vs 6.8%, respectively); however, more CI-untreated patients had longer PNH-specific median length of stay per patient per month (0.42 vs 0.33 days) compared to CI-treated patients (**Figure 5; Table 2**)

Figure 4. All-Cause HCRU Among Patients With ≥6 Months Follow-Up

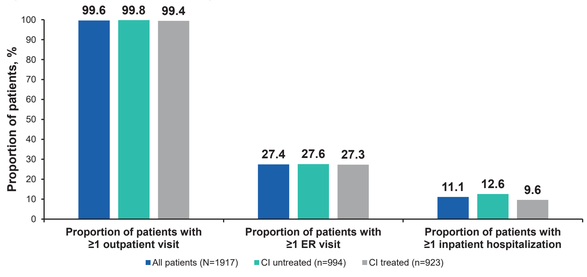


Table 1. All-Cause HCRU Among Patients With ≥6 Months Follow-Up

All-cause HCRU	All patients (N=1917)	CI untreated (n=994)	CI treated (n=923)
Patients with ≥6 months follow-up post-index date, n (%)	1815 (94.7)	921 (92.7)	894 (96.9)
Duration of follow-up, median (IQR), months	12.00 (12.00-12.00)	12.00 (12.00-12.00)	12.00 (12.00-12.00)
No. of outpatient visits, median (IQR), PPPM	1.58 (0.83-2.75)	1.50 (0.77-2.83)	1.58 (0.92-2.58)
No. of ER visits, median (IQR), PPPM	0.09 (0.08-0.19)	0.11 (0.08-0.21)	0.08 (0.08-0.17)
No. of hospitalizations, median (IQR), PPPM	0.08 (0.08-0.17)	0.08 (0.08-0.17)	0.08 (0.08-0.17)
Length of stay for all hospitalizations, median (IQR), days (PPPM)	0.42 (0.25-0.92)	0.53 (0.31-1.08)	0.33 (0.17-0.67)

CI, complement inhibitor; ER, emergency room; HCRU, healthcare resource utilization; PPPM, per patient per month.

Figure 5. PNH-Specific HCRU Among Patients With ≥6 Months Follow-Up

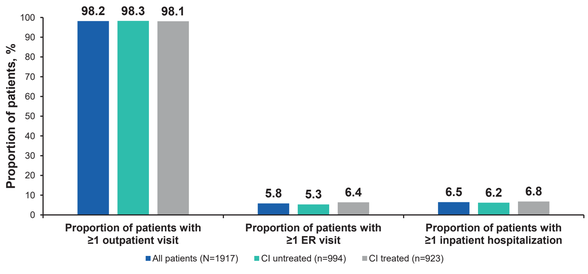


Table 2. PNH-Specific HCRU Among Patients With ≥6 Months Follow-Up

PNH-specific HCRU	All patients (N=1917)	CI untreated (n=994)	CI treated (n=923)
No. of outpatient visits, median (IQR), PPPM	0.64 (0.33-1.17)	0.33 (0.17-0.75)	0.83 (0.58-1.50)
No. of ER visits, median (IQR), PPPM	0.08 (0.08-0.13)	0.08 (0.08-0.11)	0.08 (0.08-0.17)
No. of PNH-related hospitalizations, median (IQR), PPPM	0.08 (0.08-0.12)	0.08 (0.08-0.08)	0.08 (0.08-0.17)
Length of stay for PNH-related hospitalizations, median (IQR), days (PPPM)	0.42 (0.25-0.73)	0.42 (0.25-0.88)	0.33 (0.25-0.67)

CI, complement inhibitor; ER, emergency room; HCRU, healthcare resource utilization; PNH, paroxysmal nocturnal hemoglobinuria; PPPM, per patient per month.

Individual Drug Cohorts

Healthcare Resource Utilization

- Among CI-treated patients, patients receiving iptacopan generally had fewer all-cause ER visits and inpatient hospitalizations (19.5% and 4.9%, respectively) compared to pegcetacoplan (36.0% and 17.4%), ravulizumab (24.5% and 9.5%), and eculizumab (37.6% and 6.4%) (**Figure 6; Table 3**)
- The proportion of patients with PNH-specific ER visits and inpatient hospitalizations was 2.4% and 2.4%, respectively, for iptacopan, 17.4% and 11.6% for pegcetacoplan, 4.8% and 6.7% for ravulizumab, and 7.2% and 5.6% for eculizumab (**Figure 7; Table 4**)

Figure 6. All-Cause HCRU Among Patients With ≥6 Months Follow-Up by Individual Drug

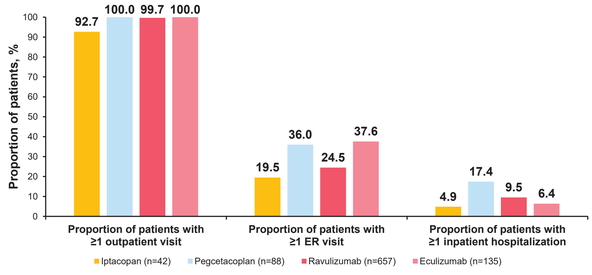


Table 3. All-Cause HCRU Among Patients With ≥6 Months Follow-Up by Individual Drug[a]

All-cause HCRU	Iptacopan (n=42)	Pegcetacoplan (n=88)	Ravulizumab (n=657)	Eculizumab (n=135)
Patients with ≥6 months follow-up post-index date, n (%)	41 (97.6)	86 (97.7)	641 (98.6)	125 (92.6)
Duration of follow-up, median (IQR), months	10.55 (9.95-11.40)	12.00 (12.00-12.00)	12.00 (12.00-12.00)	12.00 (12.00-12.00)
No. of outpatient visits, median (IQR), PPPM	1.39 (0.69-2.64)	1.58 (0.83-2.50)	1.33 (0.83-2.33)	2.58 (2.17-3.67)
No. of ER visits, median (IQR), PPPM	0.10 (0.10-0.13)	0.11 (0.08-0.20)	0.08 (0.08-0.17)	0.08 (0.08-0.25)
No. of hospitalizations, median (IQR), PPPM	NR	0.08 (0.08-0.16)	0.08 (0.08-0.17)	0.08 (0.08-0.09)
Length of stay for all hospitalizations, median (IQR), days (PPPM)	NR	0.33 (0.10-0.88)	0.33 (0.25-0.67)	0.25 (0.23-0.38)

ER, emergency room; HCRU, healthcare resource utilization; NR, not reported; PPPM, per patient per month.
[a] Data in cells with a patient count <5 are not reported.

Figure 7. PNH-Specific HCRU Among Patients With ≥6 Months Follow-Up by Individual Drug

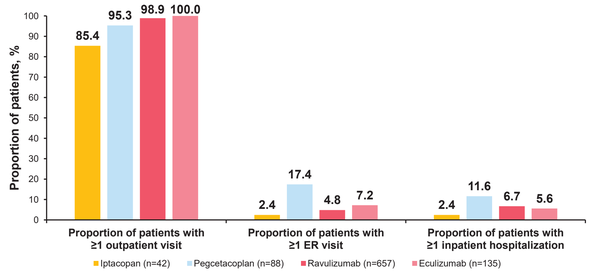


Table 4. PNH-Specific HCRU Among Patients With ≥6 Months Follow-Up by Individual Drug[a]

PNH-specific HCRU	Iptacopan (n=42)	Pegcetacoplan (n=88)	Ravulizumab (n=657)	Eculizumab (n=135)
No. of outpatient visits, median (IQR), PPPM	0.79 (0.42-1.10)	0.75 (0.42-1.17)	0.76 (0.58-1.25)	2.17 (1.50-2.50)
No. of ER visits, median (IQR), PPPM	NR	0.08 (0.08-0.11)	0.08 (0.08-0.15)	0.11 (0.08-0.17)
No. of PNH-related hospitalizations, median (IQR), PPPM	NR	0.08 (0.08-0.08)	0.08 (0.08-0.17)	0.08 (0.08-0.10)
Length of stay for PNH-related hospitalizations, median (IQR), days (PPPM)	NR	0.33 (0.10-0.60)	0.42 (0.25-0.67)	0.25 (0.21-0.42)

ER, emergency room; HCRU, healthcare resource utilization; PNH, paroxysmal nocturnal hemoglobinuria; PPPM, per patient per month.
[a] Data in cells with a patient count <5 are not reported.

Study Limitations

- Study cohorts were derived using the treatment claims data available in the Komodo Healthcare Map database. Misclassification of patients may occur due to missing claims data

References

1. Bektas M, et al. *J Manag Care Spec Pharm*. 2020;26(suppl 12-b):S3-S8.
2. Shi JJ, et al. *Ther Adv Hematol*. 2024;15:20406207241307500.

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