Early Hemoglobin and Transfusion Trends from OPERA: a Real-World Study of Pegcetacoplan Treatment in US Adults with Paroxysmal Nocturnal Hemoglobinuria

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OBJECTIVES

To analyze preliminary trends in hemoglobin levels and transfusion rates for patients in OPERA, a descriptive, observational, exploratory outcomes study, presenting real-world data on pegcetacoplan treatment for US adults with PNH post-approval.

CONCLUSIONS

- Thus far, this real-world study of US adults with PNH receiving pegcetacoplan indicates a positive trend in hemoglobin levels after treatment, with the possibility of normalizing hemoglobin levels.
- Few OPERA patients have reported receiving transfusions since initiating pegcetacoplan treatment.
- These data suggest that US patients with PNH on pegcetacoplan treatment in a real-world setting experience similar improvements in hemoglobin levels, trending toward normalization, and freedom from transfusion dependence as reported in previous clinical trials.[5-9]

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BACKGROUND

- Paroxysmal nocturnal hemoglobinuria (PNH) is an ultra-rare (1-1.5 per million), acquired, life-threatening disease characterized by complement-mediated hemolysis and thrombosis[1]
- Chronic hemolysis-driven anemia can result in persistent fatigue, requiring patients to depend on frequent blood transfusions[2]
- Pegcetacoplan is the first approved C3 inhibitor for US adults with PNH (FDA May, 2021; US brand name EMPAVELI®), and for EU adults with PNH who remain anemic after at least 3 months of treatment with a C5 inhibitor (EMA Dec, 2021; ASPAVELI®)[3,4]
- Although clinical trials have assessed the efficacy of pegcetacoplan, there is limited information on the use of pegcetacoplan in a real-world setting[5-9]

METHODS

- OPERA is a nationally representative and centrally recruited, opt-in study, where patients were electronically consented then enrolled to participate as approved by an institutional review board
- OPERA collected information from routine care and did not direct any medical interventions
- Given disease rarity, a small sample size was expected

OPERA Study Design

Inclusion	• US adults ≥18 years of age, diagnosed with PNH • Prescribed pegcetacoplan by a licensed medical professional
Exclusion	• Individuals who do not have the ability to answer web-based questionnaires by themselves • Individuals who do not speak English • Prescribed pegcetacoplan for an off-label indication (i.e. any diagnosis other than PNH)
Timepoints	Baseline, monthly over 1 year*
Dosing	OPERA patients received pegcetacoplan in 1080 mg doses twice weekly or every 3 days
OPERA Data Collection	
Pharmacy/ Site Provided	Baseline hemoglobin (healthcare provider verified)
Patient Reported (subject to availability)	**Baseline online survey**: • Prior PNH treatment • Prior reliance on transfusions • Count of transfusions within 12 months prior to enrollment **Monthly phone calls**: • Most recent hemoglobin levels if available from their regular primary care visits • Transfusion incidence during pegcetacoplan treatment
Hemoglobin Analysis	
Inclusion	Patients who reported both a baseline and ≥1 follow-up hemoglobin value
Exclusion	Patients who reported a transfusion during the treatment period

*Study is still active, therefore not all patients have completed 12 months of follow-up

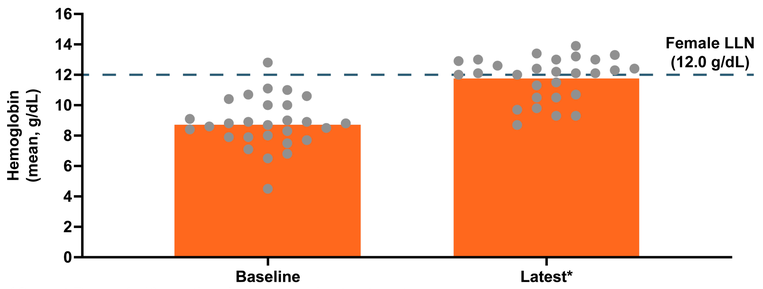
RESULTS

OPERA Demographics (N=44)

Age, years, mean (SD)	44.2 (16.7)
Gender, n (%)	
Female	23 (52.3%)
Male	21 (47.7%)
Prior C5 inhibitor treatment, n (%)	
ECU	10 (22.7%)
RAV	22 (50.0%)
Both	9 (20.5%)
None	3 (6.8%)

- Over 12 months, OPERA enrolled 44 patients in the study
- At baseline, 93.2% of patients were previously treated with a C5 inhibitor

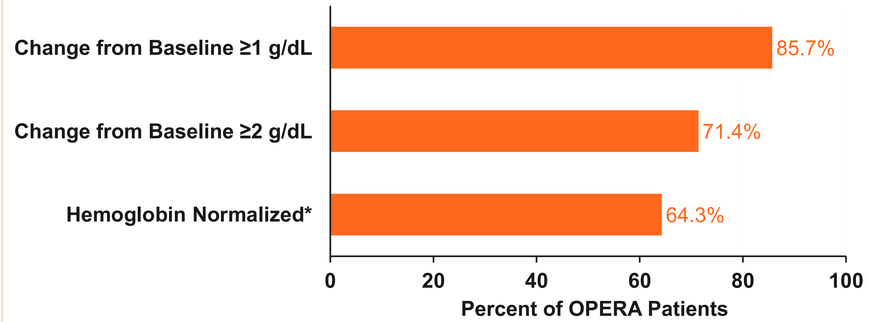
Mean Hemoglobin Level for OPERA Patients (N=28)



Current Range 1–10 months of pegcetacoplan treatment
Normal range for hemoglobin levels: male, 13.6–18.0 g/dL; female, 12.0–16.0 g/dL; N=28

- Of 28 patients who qualified for the hemoglobin analysis, the median (IQR) latest follow-up timepoint was 4.0 (2.3) months on pegcetacoplan treatment
- The mean hemoglobin level increased from baseline to near normal levels after initiating pegcetacoplan treatment for the latest reported hemoglobin value

Hemoglobin Level Improvement Observed in OPERA (N=28)



Hemoglobin normalization defined as hemoglobin levels ≥12.0 g/dL

The majority of OPERA patients saw improvements in their hemoglobin levels, with over two thirds achieving normal levels after initiating pegcetacoplan treatment.

Transfusion Treatment* (N=44)

Patients relying on transfusions in the 12 months prior to enrollment, n (%)	24 (54.5%)
Patients requiring ≥4 transfusions in the 12 months prior to enrollment, n (%)	10 (22.7%)
Patients reporting a transfusion after initiating pegcetacoplan treatment thus far, n (%)	8 (18.2%)

Self-reported/subject to availability

Few OPERA patients have reported transfusions after initiating pegcetacoplan treatment.

Abbreviations: EMA, European Medicines Agency; EU, The European Union; FDA, Food and Drug Administration; g/dL, grams per deciliter; IQR, interquartile range; LLN, lower limit of normal; mg, milligrams; PNH, paroxysmal nocturnal hemoglobinuria; SD, standard deviation; US, United States