Early Hemoglobin and Quality-of-Life 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 QOL 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 after treatment, with the possibility of normalizing hemoglobin levels; displaying similar hemoglobin improvements as reported in previous clinical trials[5-9]
- OPERA patients exhibited improvements in self-reported fatigue and cognitive function after initiating PEG treatment

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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, negatively impacting patients' quality-of-life (QOL)[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]
- The Patient Reported Outcome Measure Information System (PROMIS) - Cognitive Abilities has been validated for use across other disease conditions[10], but has not been explored among patients with PNH

METHODS

- OPERA is a nationally representative and centrally recruited, exploratory 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
- The detailed study design, patient reported outcomes, and hemoglobin analysis are described in the OPERA Study Design Table
- 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)	**Quarterly online surveys (low score=negative outcome)**: • Functional Assessment of Chronic Illness Therapy (FACIT)-Fatigue (0–52 score) • Patient-Reported Outcomes Measurement Information System (PROMIS) scale for Cognitive Abilities (23.27–67.09 t-score) **Monthly phone calls**: • Most recent hemoglobin levels if available from their physician-directed routine clinical care
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

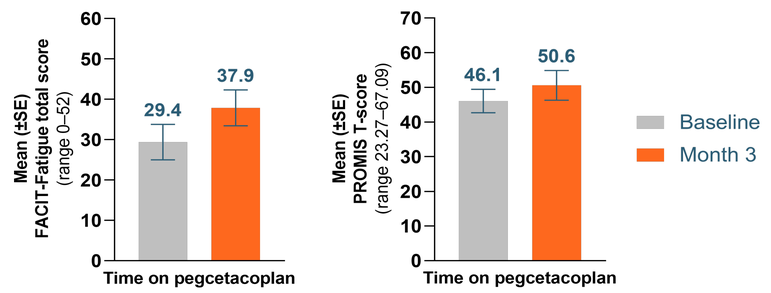
RESULTS

- Over 12 months, 44 patients enrolled in the OPERA study

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%)

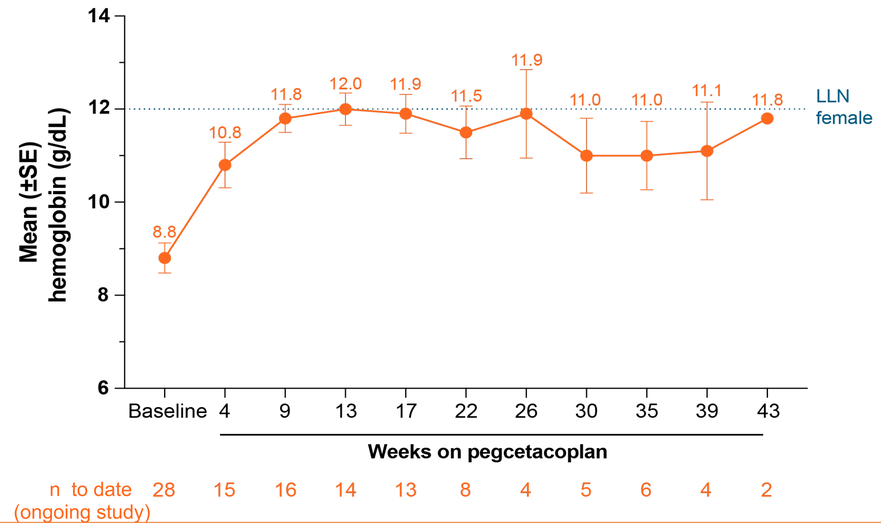
Improvement in Patient Reported Outcome Measures: Fatigue and Cognitive Function (n=8*)



*Reduced n due to ongoing study and analysis only including patients with both baseline & month 3 outcomes
Change from baseline considered clinically significant if ≥ 5 points (FACIT-Fatigue), if ≥ 8 points (PROMIS)[11,12]; PROMIS mean (SD) general population T-score= 50 (10)[10]

- Of 28 patients who qualified for hemoglobin analysis (monthly means depicted in Figure), median (IQR) latest follow-up timepoint was 4.0 (2.3) months on pegcetacoplan treatment
- Among them, 85.7% reported a latest mean Hb change from baseline by ≥1.0 g/dL, 71.4% by ≥2.0 g/dL, and 64.3% achieved Hb normalization (as measured by ≥12.0 g/dL).

Study Population Average Hemoglobin Levels Reported per Month of Pegcetacoplan Treatment Completed



Data represented depicts summary statistics of the population's reported hemoglobin
Hemoglobin is reported if/when this lab test is available, which varies (physician-directed) during routine care & monitoring
*Current Range 1–10 months of pegcetacoplan treatment; n= patients who reported a baseline and ≥1 follow-up hemoglobin value

Abbreviations: EMA, European Medicines Agency; ECU, eculizumab; EU, The European Union; FACIT-Fatigue, Functional Assessment of Chronic Illness Therapy-Fatigue; FDA, Food and Drug Administration; g/dL, grams per deciliter; IQR, interquartile range; LLN, lower limit of normal; mg, milligrams; PNH, paroxysmal nocturnal hemoglobinuria; PROMIS, Patient-Reported Outcomes Measurement Information System; RAV, ravulizumab; SD, standard deviation; US, United States