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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KEYWORDS
Paroxysmal nocturnal hemoglobinuria (PNH), Chronic hemolysis, Fatigue, Cognitive function

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*.

• Chronic hemolysis-driven anemia can result in persistent fatigue, negatively impacting patients’ quality-of-life (QOL)‡.

• Pegcetacoplan is the first approved C5 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®)‡. Although clinical trials have assessed the efficacy of pegcetacoplan, there is limited information on the use of pegcetacoplan in a real-world setting§.

• The Patient Reported Outcome Measure Information System (PROMIS) - Cognitive Abilities has been validated for use across other disease conditions¶, 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 Document.

• Given disease rarity, a small sample size was expected.

RESULTS

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

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<th>OPERA Demographics (N=44)</th>
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<td>Age, years, mean (SD)</td>
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<td>Gender, n (%)</td>
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<td>Prior C5 Inhibitor treatment, n (%)</td>
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| Improvement in Patient Reported Outcome Measures: Fatigue and Cognitive Function (n=8*) |

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<th>Time on pegcetacoplan</th>
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<td>FACT-Cog score (range: 0-113)</td>
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• Of 26 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 ≥112.0 g/dL).

• OPERA patients exhibited improvements in self-reported fatigue and cognitive function after initiating PEG treatment.

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§.

• OPERA patients exhibited improvements in self-reported fatigue and cognitive function after initiating PEG treatment.

Abbreviations: EMA, European Medicines Agency; ECU, eculizumab; EU, The European Union; FACIT, Functional Assessment of Chronic Illness Therapy; 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

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

**For full references, see supplemental material.