# Work Productivity and Activity Impairment (WPAI), and Hemoglobin Levels During OPERA: a Real-World Study of Pegcetacoplan Treatment in US Adults With Paroxysmal Nocturnal Hemoglobinuria (PNH)

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## OBJECTIVE

This real-world study aims to report trends in hemoglobin levels and WPAI for patients in OPERA, an observational real-world study on pegcetacoplan treatment for US adults with PNH, post-approval.

## CONCLUSIONS

- Overall, patients with PNH treated with pegcetacoplan showed a positive trend in hemoglobin level over an 8-month period
- Pegcetacoplan improved work productivity and reduced activity impairment in patients with PNH over 6 months of treatment

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**Disclosures**: Fishman, J. reports current employment and current equity holder in publicly-traded company for Apellis Pharmaceuticals. Min, J. reports current employment with Apellis Pharmaceuticals. Arnett L. and Shenoy A. report current employment with Boston Strategic Partners Inc, which received funding from Apellis Pharmaceuticals, Inc. for this study.

**References:** 1. Parker C, et al. *Blood.* 2005;106(12):3699–709. 2. Kawamoto Y, et al. *BMC Anesthiology.* 2019;2(2):205-255. 3. Schrezenmeier H, et al. *Haematologica.* 2014;99(5):922-929. 4. Dingli D, et al. *Ann Hematol.* 2022; 101(2): 251–263 5. Hillmen P et al. *N Engl J Med* 2021;384:1028-37 6. de Castro C et al. *Am J Hematol* 2020;95:1334-43; 7. Wong RSM et al. *Ann Hematol* 2022;101:1971-98: 8. Peffault de Latour R et al. *Lancet Haematol* 2022;9:e648-59; 9. Wong RS et al. *Blood* 2021;138(Suppl 1):606

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## CONTEXT

- Paroxysmal nocturnal hemoglobinuria (PNH) is a rare, acquired, life-threatening disease (0.5 – 1.5 cases per million) characterized by chronic complement-mediated hemolysis causing anemia and fatigue[1,2,3]
- Anemia-related PNH symptoms may negatively impact patient work productivity and impair daily activities[4]
- Pegcetacoplan (PEG) is the first approved C3 inhibitor that demonstrated superiority over eculizumab in improving hemoglobin levels in adults with PNH (phase 3 PEGASUS trial)[5]
- Despite showing efficacy in clinical trials, there is limited information on the use of PEG in a real-world setting[6-9]

## METHODS

- OPERA, an ongoing, nationally representative, centrally recruited observational study (institutional review board approved) for patients with PNH, who were prescribed pegcetacoplan by a licensed medical professional. Data were collected from routine medical encounters without implementing any specific interventions
- Baseline hemoglobin levels were reported by healthcare providers at the site and subsequent hemoglobin levels were self-reported by patients during monthly calls, whenever available
- WPAI questionnaires were completed online on a quarterly basis, starting from the baseline assessment. Analysis was conducted only with patients who had a baseline assessment and at least one follow-up assessment. WPAI outcomes measured work time missed (absenteeism), impairment experienced while working (presenteeism), overall productivity loss, and activity impairment. Higher values on these measures indicated greater impairment
- Hemoglobin analysis was conducted only on patients who did not receive transfusions (intercurrent event) during pegcetacoplan treatment and had reported both a baseline hemoglobin level and at least one follow-up hemoglobin value
- Due to the rarity of the disease, the study anticipated a small sample size

## RESULTS

- Over 15 months, 50 US patients (≥18 years) with PNH enrolled in the OPERA study

**Table 1. Baseline Population & Characteristics (N = 50)**

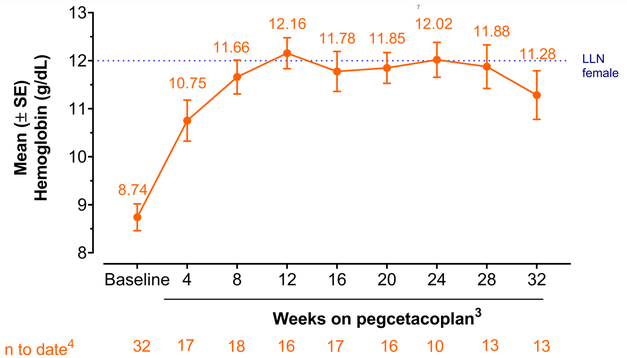
| | |
|---|---|
| **Age**, years, mean (SD) | 46.0 (17.5) |
| **Gender**, n (%) | |
| Female | 24 (48.0%) |
| Male | 26 (52.0%) |
| **Prior C5 inhibitor treatment**, n (%) | |
| ECU | 11 (22.0%) |
| RAV | 27 (54.0%) |
| Both | 9 (18.0%) |
| None | 3 (6.0%) |

**Table 2. WPAI Assessment**

| | Baseline | | While on PEG[a] | |
|---|---|---|---|---|
| | N | % | N | % |
| Employment (n=18) | 14 | 77.8% | 14 | 77.8% |
| Employed reporting hours missed from work in the past 7 days due to health (BL, n=13*, PEG, n=14) | 6 | 46.2% | 2 | 14.3% |
| Employed reporting affected productivity while at work (BL, n=13*, PEG, n=14) | 10 | 76.9% | 10 | 71.4% |
| Patients reporting activity impairment (n=18) | 17 | 94.4% | 14 | 77.8% |

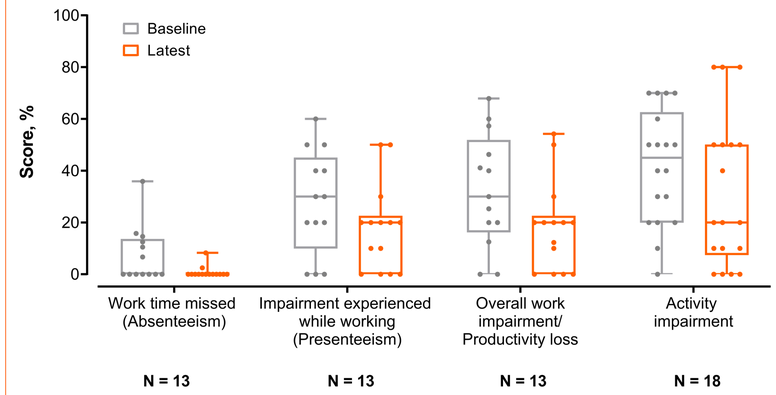
*one patient reported 0 working hours in the previous 7 days at baseline and could not be included in this calculation; [a]PEG treatment duration varied as study is ongoing, median (IQR) 6 (5.3) months on PEG

**Figure 1. Subgroup Analysis of Average Hemoglobin Levels Reported (among those without a Transfusion) per Study Month[1,2]**




Of 32 patients meeting hemoglobin analysis criteria, mean (SD) baseline hemoglobin level was 8.7 (1.6) g/dL and mean (SD) latest reported hemoglobin level increased to 11.9 (1.7) g/dL, after a median (IQR) follow-up period of 8 (5.0) months on PEG. Note: For 49 patients completing ≥1 monthly call, the incidence rate for transfusions per person year was 0.8 (95% CI 0.6–0.9)

**Figure 2. WPAI Analysis of Patients on Pegcetacoplan**




Note: For WPAI analysis lower score indicates a better outcome. For patients meeting WPAI analysis criteria, compared to baseline, mean (SD) absenteeism improved by 6.6% (10.6%), presenteeism by 8.5% (29.4%), overall work-productivity loss by 12.6% (30.6%), and activity impairment by 10.0% (32.2%) after a median (IQR) 6 (5.3) months on pegcetacoplan

Footnote: 1. Data represented depicts summary statistics of the population's reported hemoglobin 2. Hemoglobin is reported if/when this lab test is available, which varies (physician-directed) during routine care and monitoring 3. The figure only includes time points where data was available for 10 or more patients 4. n= patients who reported a baseline and ≥1 follow-up hemoglobin value and no transfusions while on PEG.

Abbreviations: AE(s), adverse event(s); BL, baseline; ECU, eculizumab; g/dL, grams per deciliter; IQR, interquartile range; LLN, Lower limit of normal; PEG, pegcetacoplan; PNH, paroxysmal nocturnal hemoglobinuria; RAV, Ravulizumab; SD, standard deviation; SE, standard error; US, United States; WPAI, Work Productivity and Activity Impairment