PCR97

# Impact of Paroxysmal Nocturnal Hemoglobinuria on Employment and Activity: Results from a Real-World Study

**Panse J[1], Balp MM[2], Wiyani A[3], O'Neill CB[4], Mellor J[5], Earl L[5], Taylor Y[5], Simons A[5], Snellman J[2]**

[1]University Hospital RWTH Aachen, Aachen, NW, Germany; [2]Novartis Pharma AG, Basel, BS, Switzerland; [3]Novartis Pharmaceuticals UK Limited, London, UK; [4]Novartis Business Services, Dublin, County Dublin, Ireland; [5]Adelphi Real World, Bollington, Cheshire, UK

## Introduction and Objective

- Paroxysmal nocturnal hemoglobinuria (PNH) is a rare blood condition, caused by complement-mediated haemolysis with persistent anemia and fatigue[1,2].
- Currently approved first-line treatments include complement 5 inhibitors (C5i), eculizumab and ravulizumab[2].
- Limited data are available on the impact of PNH on work and activity among patients treated with C5i.
- The aim of this study was to assess the impact of PNH despite C5i treatment on adult patients' employment, ability to work, and overall level of activity impairment.

## Methods

- Data were drawn from the Adelphi PNH Disease Specific Programme™, a cross-sectional survey of PNH-treating physicians and their patients from Canada, France, Germany, Italy, Japan, Spain, and the United States (data collected January-July 2022).
- Patients filled self-completion forms comprising demographics, employment status, and the disease specific Work Productivity and Activity Impairment questionnaire (WPAI-PNH).
- WPAI-PNH asked about the effect of PNH on patients' ability to work and perform daily activities. It has a 7-day recall, four scores calculated % absenteeism, presenteeism, overall work impairment and activity impairment; higher scores indicate higher impairment.
- Physicians reported based on medical charts, patient's current treatment, dates of diagnosis and treatment initiation.
- Descriptive analyses were conducted on data from C5i-treated PNH patients.

## Results

- A total of 143 PNH patients treated with C5i (77.6%: eculizumab and 22.4%: ravulizumab) were included in the analysis. Demographics are in **Table 1**.

### Table 1. Patients' demographics at the time of survey

| | N | Overall |
|---|---|---|
| Age years*, mean (SD), median | 143 | 48.2 (14.7), 47.0 |
| Male patients*, n (%) | 143 | 74 (51.7%) |
| Time since diagnosis, years#, mean (SD), median | 142& | 2.5 (2.8), 1.7 |
| Duration since starting C5i, years#, mean (SD), median | 140& | 1.8 (2.1), 1.1 |
| Employment status – employed patients*, n (%) | 141& | 70 (49.7%) |

C5i: Complement 5 Inhibitor; N: Sample Size; SD: Standard Deviation
*Data obtained through patient self-completion forms; #Data obtained through physician survey based on medical charts; &Missing data excluded from overall base (N = 143)

- At the time of survey, 52 (36.9%) patients were employed full time and 18 (12.8%) part-time, and 11 (7.8%) were students (**Table 2**).
- Patients reported that due to their PNH, they missed their work/or studies for a mean (SD) of 2.6 (3.9) days per week in the last three months.

### Table 2. Employment status of C5i-treated PNH patients at the time of survey

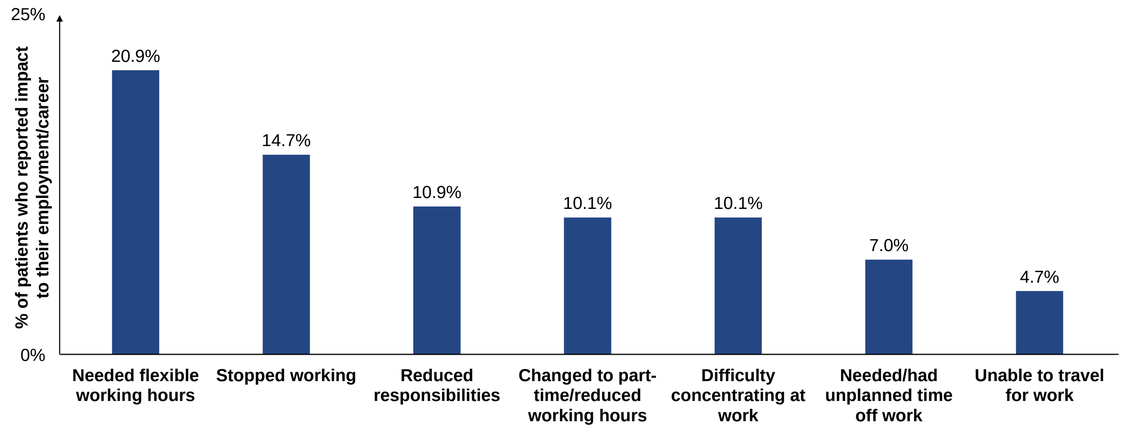
| Category reported, n (%) | N = 141* |
|---|---|
| Working full time | 52 (36.9%) |
| Homemaker | 22 (15.6%) |
| Retired | 20 (14.2%) |
| Working part time | 18 (12.8%) |
| On long term sick leave | 12 (8.5%) |
| Student | 11 (7.8%) |
| Unemployed | 6 (4.3%) |

C5i: Complement 5 Inhibitor; N: Sample Size; PNH: Paroxysmal Nocturnal Hemoglobinuria
*Missing data excluded from overall base (N = 143)

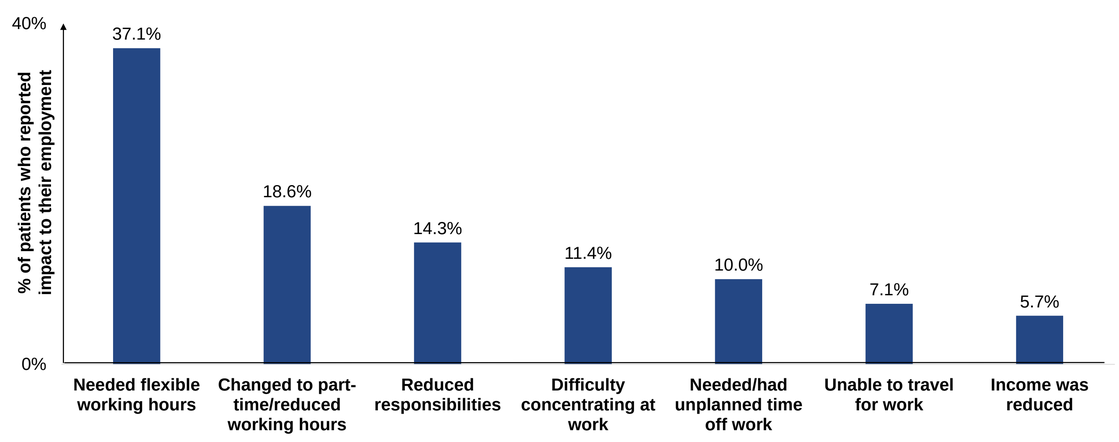
- A total of 74 out of 129 patients with data available (57.4%) and 45 out of 70 currently employed patients (64.3%) reported impact to their employment/career due to PNH such as requiring flexible hours, stopped working, reduced responsibilities, etc. (**Figure 1a & b**)

### Figure 1. Patient-reported impact to their employment/career due to PNH

**a) In all patients (n = 129)***



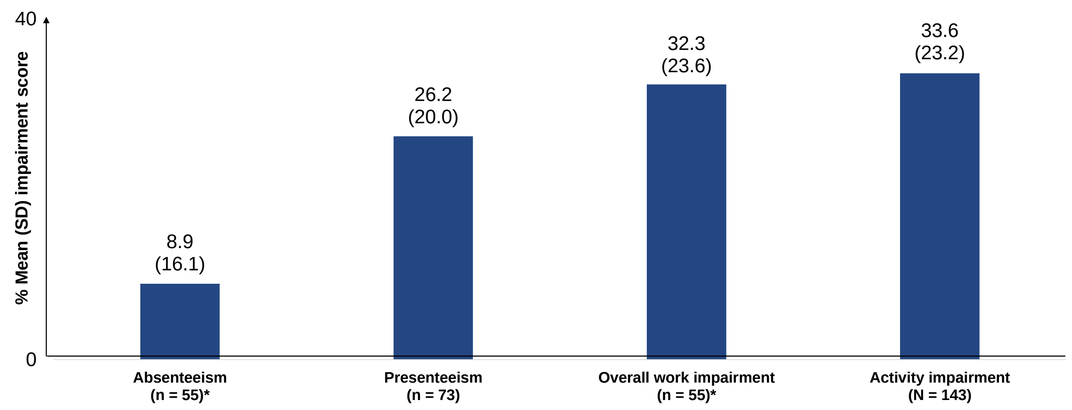

**b) In employed patients (n = 70)**




n: Sample Size; PNH: Paroxysmal Nocturnal Hemoglobinuria
*Missing data excluded from overall base (N = 143); 55 out of 129 patients with data available (42.6%) and 25 out of 70 currently employed patients (35.7%) reported no impact (or not applicable) to their employment/career due to PNH
Note: we considered top 7 most impactful factors to employment/career due to PNH as reported by patients

- Among employed patients, the mean (SD) % absenteeism was 8.9 (16.1), presenteeism was 26.2 (20.0), overall work impairment was 32.3 (23.6), and among all patients, activity impairment was 33.6 (23.2) (**Figure 2**).

### Figure 2. Work Productivity and Activity Impairment (WPAI) scores




N, n: Sample Size; SD: Standard Deviation
Higher score indicates higher impairment; *missing data excluded from base number

## Conclusions

- PNH patients treated with C5i reported that their disease had an impact on their ability to work and perform daily activities.
- Many patients reported impact to their employment/career due to their PNH such as flexible hours, reduced working hours or reduced responsibilities.
- Newer therapies could provide more comprehensive control of PNH, which may positively impact employment, productivity, and overall activity.

### References

1. Fattizzo B et al. *Journal of Blood Medicine*. 2022;13:327-35.
2. Risitano AM et al. *Front Immunol*. 2019;10:1157.

### Disclosures

Data collection for the DSP was undertaken by Adelphi Real World as part of an independent survey and data are owned by Adelphi. Novartis is one of multiple subscribers to the DSP and supported this analysis.

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