

Unmet Medical Need in Patients with Paroxysmal Nocturnal Hemoglobinuria Treated with Complement 5 Inhibitors: Results from a Cross-sectional Survey in Canada

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CONCLUSIONS

- These findings suggest C5i-treated PNH patients with Hb ≥10 g/dL to 12 g/dL, still experience a high burden and unmet need.
- Patients report symptoms of anemia, fatigue (impacting personal, social and work life). Some of these patients are transfusion dependent, and few require hospitalization to manage their PNH.
- Restricting access based on Hb threshold ≤10g/dL or ≤10.5g/dL may hinder switching of patients who are inadequately controlled on C5i to alternative treatments (e.g., proximal inhibitors) that may offer a more comprehensive control and may help to address the remaining clinical and humanistic burden in PNH.

Poster presented at the International Society for Pharmacoeconomics and Outcomes Research, Barcelona, Spain, 17-20 November 2024.

INTRODUCTION

- Paroxysmal nocturnal hemoglobinuria (PNH) is a rare blood disorder characterized by complement mediated hemolysis.¹ Patients often experience anemia, fatigue and require blood transfusions.²
- Several treatments are approved in PNH, including terminal or proximal complement therapies such as complement 5 inhibitors (C5i) or complement 3 inhibitors (C3i), respectively.
- In Canada, based on provincial reimbursement criteria (public coverage), accessing C5i or switching from C5i to C3i is restricted to PNH patients with hemoglobin (Hb) ≤10g/dL³ or ≤10.5g/dL.⁴
- Limited data is available on the burden of PNH patients treated with C5i and having Hb ≥10 g/dL to <12 g/dL in Canada.

AIM

- This analysis aims to describe the burden of disease among C5i-treated PNH patients with Hb ≥10 g/dL to <12 g/dL in Canada.

METHODS

- Data were collected from Adelphi PNH Disease Specific Programme™, a cross-sectional survey of physicians and their patients living with PNH in Canada (January–November 2022).⁵⁻⁸
- Hematologists and hematologist-oncologists managing PNH completed patient record forms for up to the next 10 consulting adult patients with PNH, including sociodemographic, clinical characteristics, blood transfusions, hospitalizations, and impact of PNH on quality of life.
- Physicians reported data using existing patient clinical records, their own judgment as well as diagnostic/interpretation skills, and information at consultation.
- Data from patients treated with C5i with Hb ≥10 g/dL to <12 g/dL were analyzed using descriptive statistics and missing data were not imputed.

RESULTS

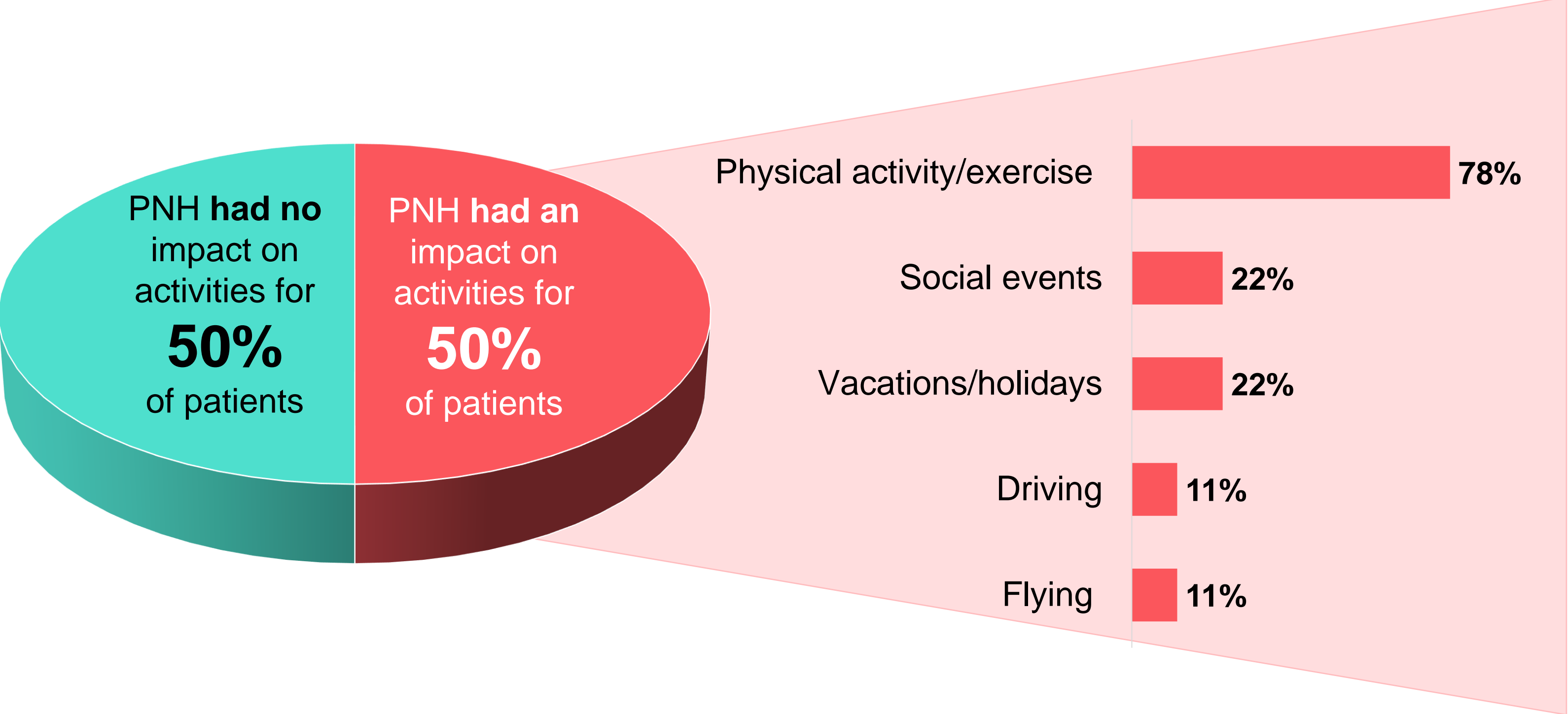
- A total of four physicians provided data on 31 patients; among them 27 patients (87%) were treated with C5i. From these, 19 (70%) C5i-treated patients had Hb ≥10 to <12 g/dL (79% eculizumab; 21% ravulizumab), for a mean (standard deviation; SD) duration of 2.5 (3.3) years (**Table 1**).
- The mean (SD) patient age was 51.9 (15.7) years, 68% were male and mean (SD) Hb was 10.5 (0.5) g/dL at time of survey.
- Various symptoms were recorded for these patients, including anemia (90%), fatigue (74%), and weakness (58%).
- Physicians reported:
 - Around two-thirds of patients had a ≥1 blood transfusion in the past 12 months. Mean (SD) number of transfusions was 1.4 (1.4) and a mean (SD) of 2.2 (0.6) units of blood were given at the most recent transfusion.
 - Over 1 in 10 patients had ≥1 PNH-related hospitalization in the past 12 months due to severe fatigue or infection.
- For half of these patients, PNH imposed a reduction or avoidance of activities, social events, vacations/holidays, etc. (**Figure 1**).
- Many aspects of life were impacted at various degrees by PNH such as employment/education, ability to travel/go on vacation, emotional/psychological health and social life (**Figure 2**).

Table 1. Physician-reported sociodemographic and clinical characteristics*

	N=19
Age, mean (SD)	51.9 (15.7)
Sex, male, n (%)	13 (68)
Employment status, n (%)	
Working full time	8 (42)
Working part time	6 (32)
Retired	4 (21)
Unemployed	1 (5)
Time since diagnosis, years, mean (SD)	2.8 (3.6)
Time since C5i treatment, years, mean (SD)	2.5 (3.3)
C5i prescribed, n (%)	
Eculizumab	15 (79)
Ravulizumab	4 (21)
Most recent hemoglobin level (g/dL), mean (SD)	10.5 (0.5)
Transfusions in the (past 12 months)	n=16
Patients receiving ≥ 1 transfusion, n (%)	10 (63)
Unit of blood received (n = 11), mean (SD)	2.2 (0.6)
Hospitalization (in the past 12 months)	n=16
≥ 1 hospitalization, n (%)	2 (13)

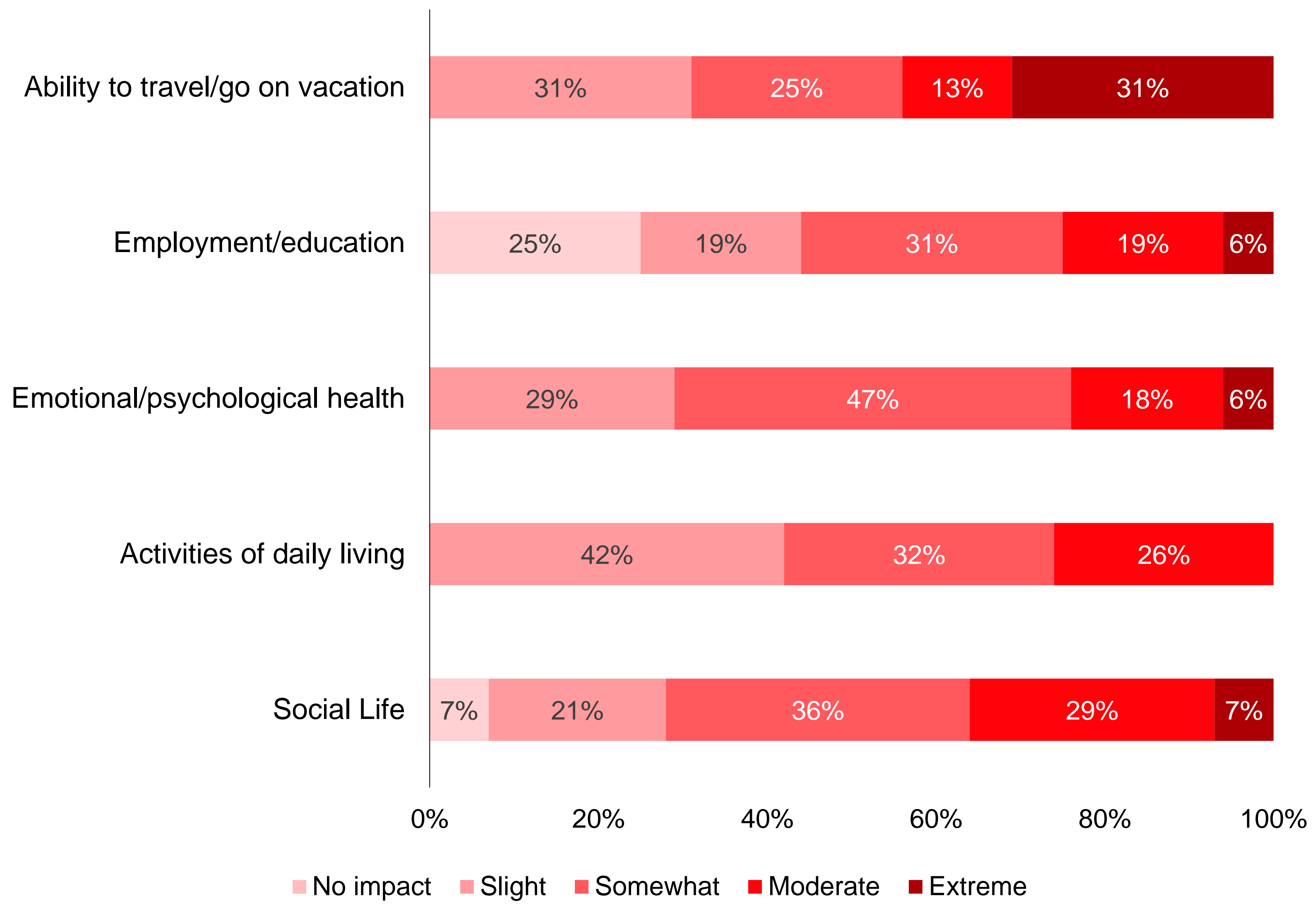
C5i: complement 5 inhibitors, SD: standard deviation.

Figure 1. Impact of PNH on patients’ activities (physician-reported data on reduced or avoided activities by patients)*



*N = 18, multiple activities could be selected

Figure 2. Levels of impact PNH had on aspects of patients’ life (physician-reported data)*



*N = 19

- Patients included in the DSP sample may not be fully representative of the overall population of Canadian PNH patients. In addition, the small sample size may limit the generalizability of results.

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Acknowledgements

The authors acknowledge **Jahnavi Yenamandra** (Novartis, Hyderabad) for preparing poster content and **Rupa De** for designing the poster layout. The final responsibility for the content lies with the authors.

Disclosures

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



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