

# Ethnographic Research Findings on the Everyday Challenges of People with PNH in Three Countries

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## CONCLUSIONS

- The ethnographic research of the lived experience of people with Paroxysmal Nocturnal Haemoglobinuria (PNH) conducted in Autumn 2021 shows that diagnosis is often challenging, and when given is experienced as a disruption of normality often associated with a negative impact on ability to work, intimacy and parenthood, and hobbies and other activities.
- Treatment with C5 inhibitors is found to be practically and emotionally burdensome in itself.
- The study adds to the emerging evidence showing a significant symptom burden and reduced QoL despite C5 inhibitor treatment – and there's a greater breadth of experienced symptoms than commonly recognised.
- The analysis shows that the support networks of people with PNH are patchy and in need of strengthening to ensure people with PNH – and their ecologies – get the answers and resources they need to live well with PNH.

FIGURE 1: Participant characteristics

People with PNH (n=27)	n (%)
<b>Age (years)</b>	
18-35	8 (30%)
36-55	13 (48%)
56+	6 (22%)
<b>Gender</b>	
Female	17 (63%)
Male	10 (37%)
<b>Treatment</b>	
Eculizumab	19 (UK: 1, France: 11, Spain: 7) (71%)
Ravulizumab	8 (UK: 6, France: 0, Spain: 2) (29%)
<b>Site of administration</b>	
Hospital	16 (UK: 0, France: 8, Spain: 8) (59%)
Home	11 (UK: 7, France: 3, Spain: 1) (41%)

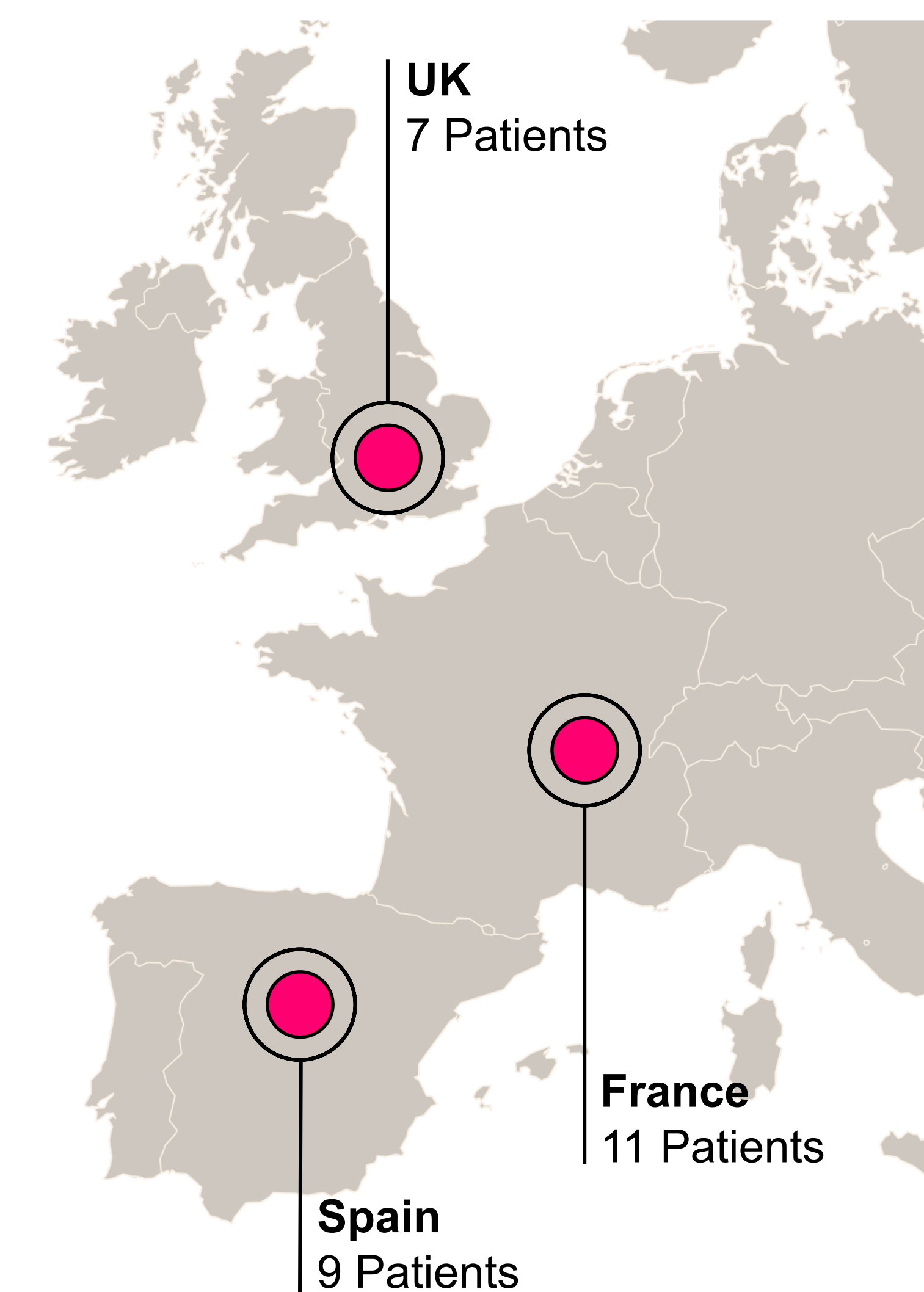
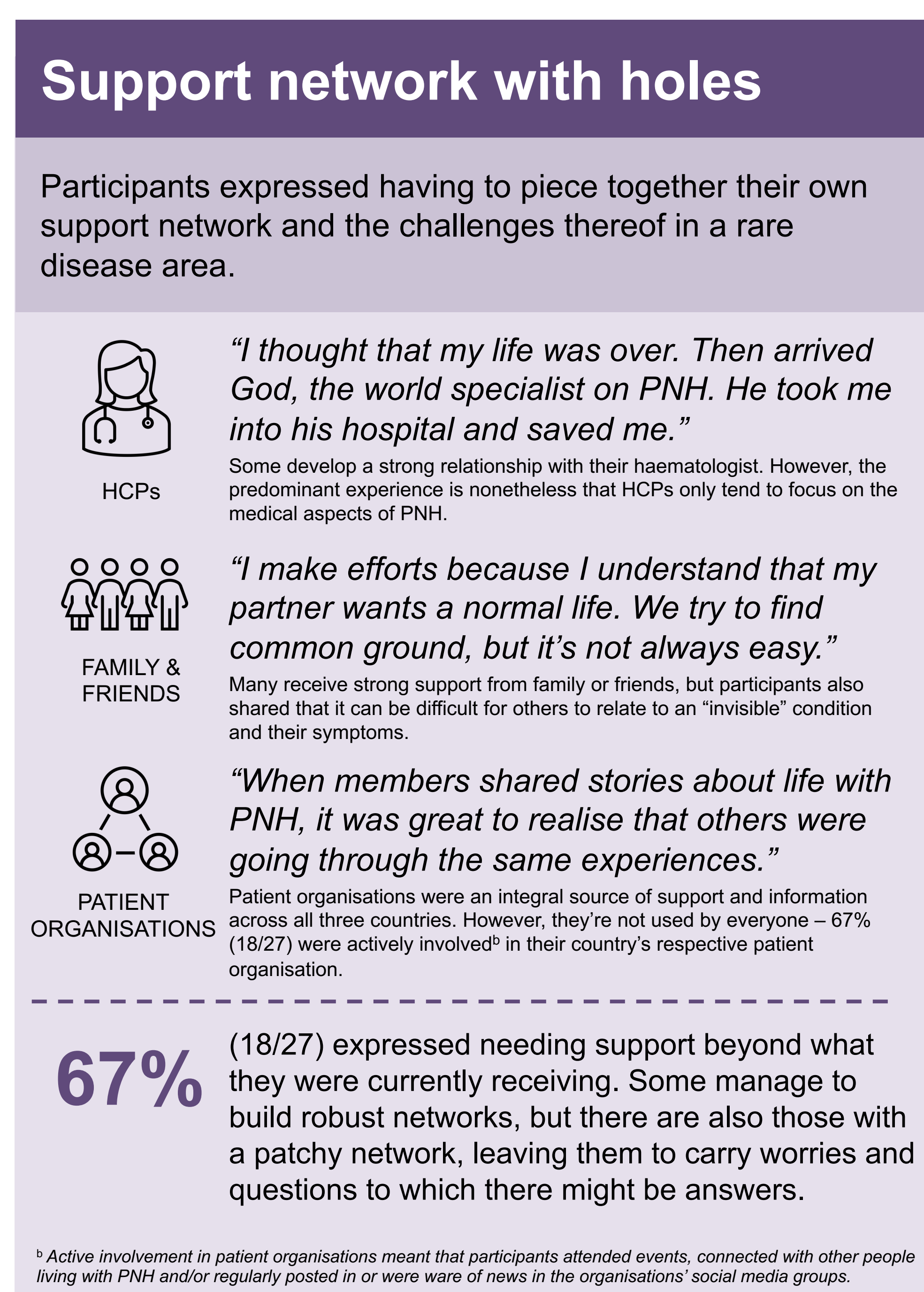
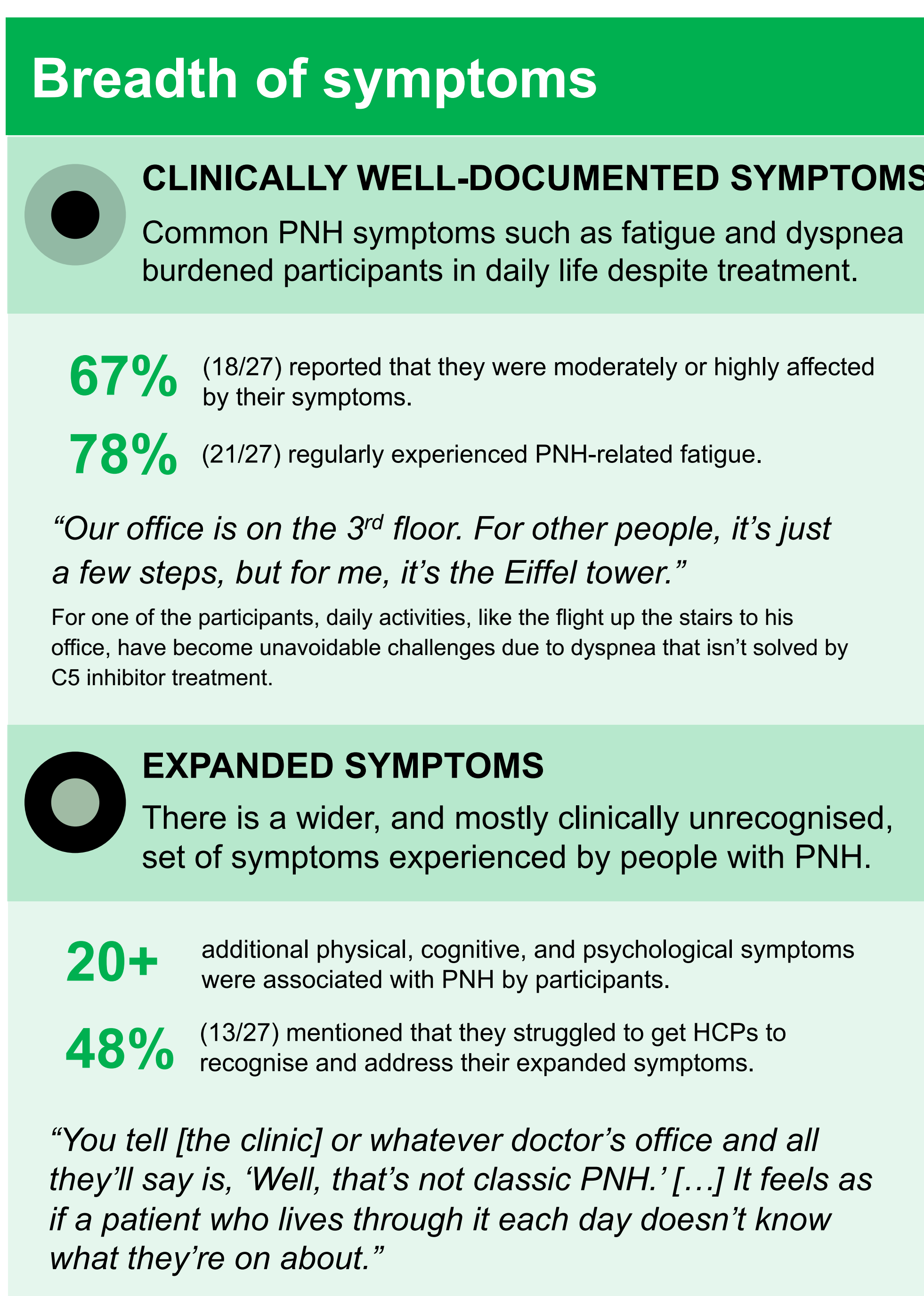
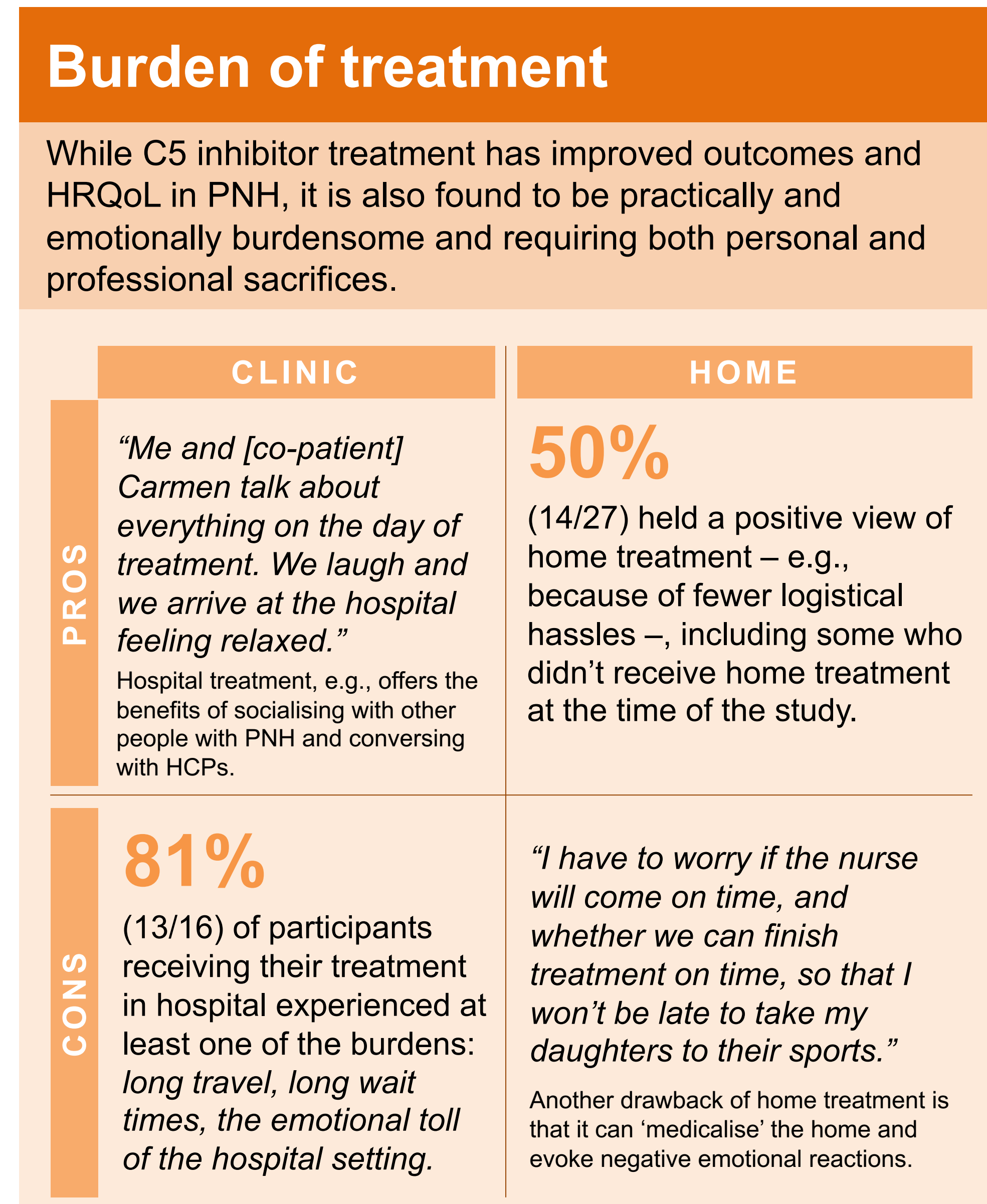
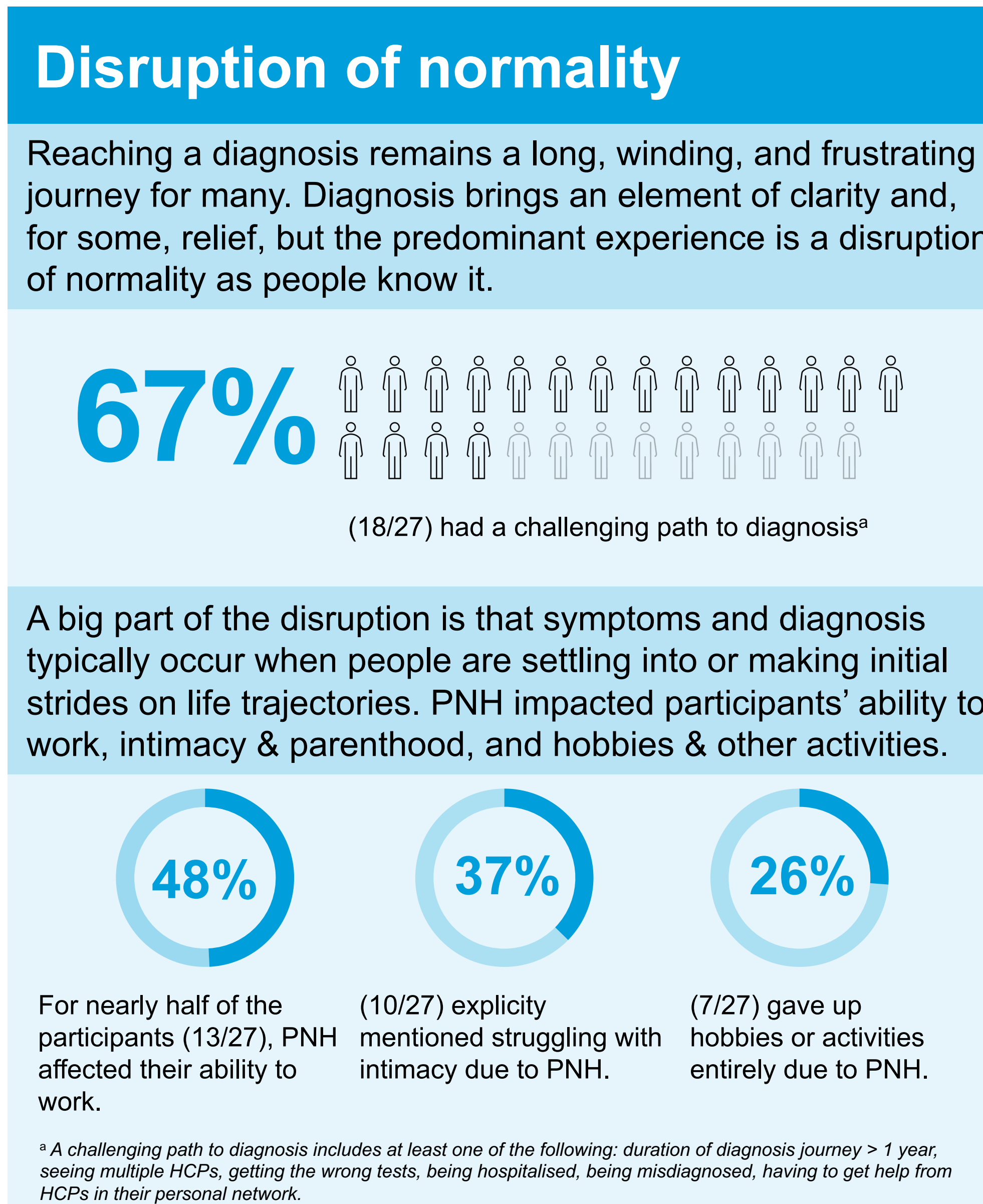


FIGURE 2: Themes from ethnographic research (verbatim quotes are reflective of the typical experience among participants)



## INTRODUCTION

- With the introduction of C5 inhibition as a treatment option in PNH, life expectancy has improved and is near-normal<sup>1</sup>. However, the burden of disease remains significant for many patients despite treatment<sup>2,3</sup>.
- Questionnaire-based studies have found that people with PNH still experience a variety of symptoms while on C5 inhibitor treatment; many remain anaemic; the burden of illness remains substantial; and health-related quality of life is impacted negatively<sup>2,3,4</sup>.
- In-depth knowledge about the everyday challenges of people with PNH on C5 inhibitors remains limited.

## OBJECTIVE(S)

- The objective of this observational, ethnographic study was to investigate the experience of living with PNH, including experiences related to participants' condition and treatment, their ways of managing the condition, and their challenges.

## METHODS

- People with PNH were recruited through patient organisations (PNH Support; HPNE; HPN-AM). Written informed consent was provided by all participants before study entry.
- Data were collected through 8-12 hours of participant observation, semi-structured interviews, written exercises, and conversations with friends and family. Participants also filled in a daily questionnaire for 5 days about their fatigue level and activities.
- Visits were conducted at treatment centres to interview healthcare professionals.
- Analysis was performed using an abductive grounded theory approach and themes were identified through qualitative coding, needs mapping, and clustering.

## RESULTS

- Data were analysed from 27 people with PNH (19 on eculizumab; 8 on ravulizumab). 7 HCPs were interviewed and 3 treatment centres visited (Figure 1).
- Four themes in relation to the everyday challenges of people with PNH surfaced from the study (Figure 2).



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## Disclosures

AMWL, MB-K, KK, PH, MR: Employed by ReD Associates, which carried out the study with funding from Sobi; MP, AN: PNH Support has received consultancy fees from Sobi; JS: Employed by Sobi.

## Acknowledgements

We thank the patients, their caregivers and their healthcare professionals who contributed to this study. We also thank the patient organisations PNH Support (informed the study design), HPNE, and HPN-AM for their contributions to the study. The study was carried out by ReD Associates with funding and input from Sobi. Sobi reviewed and provided feedback on the poster. The authors had full editorial control of the poster.