# 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.

### FIGURE 1: Participant characteristics

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

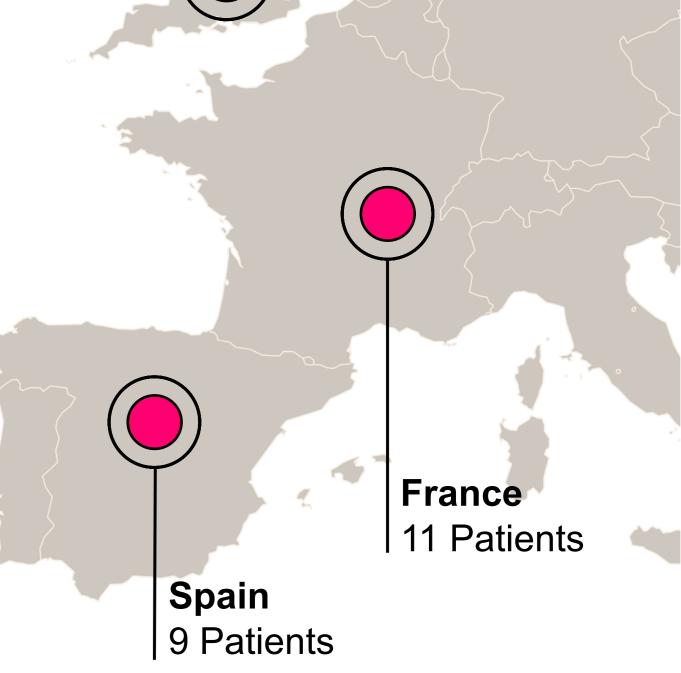


- Treatment with C5 inhibitors is found to be practically and emotionally burdensome in itself.
- The study adds to the emerging evidence showing a significant symptom burden and reduced QoL despite C5 inhibitor treatment – and there's a greater breadth of experienced symptoms than commonly recognised.
- 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.

# INTRODUCTION

- With the introduction of C5 inhibition as a treatment option in PNH, life expectancy has improved and is nearnormal<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>.

11 (UK: 7, France: 3, Spain: 1) (41%)



### FIGURE 2: Themes from ethnographic research

(verbatim quotes are reflective of the typical experience among participants)

## **Disruption of normality**

Home

Reaching a diagnosis remains a long, winding, and frustrating journey for many. Diagnosis brings an element of clarity and, for some, relief, but the predominant experience is a disruption of normality as people know it.

# 

## **Burden of treatment**

While C5 inhibitor treatment has improved outcomes and HRQoL in PNH, it is also found to be practically and emotionally burdensome and requiring both personal and professional sacrifices.

 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.

(18/27) had a challenging path to diagnosis<sup>a</sup>

A big part of the disruption is that symptoms and diagnosis typically occur when people are settling into or making initial strides on life trajectories. PNH impacted participants' ability to work, intimacy & parenthood, and hobbies & other activities.



For nearly half of the<br/>participants (13/27), PNH(10/27) explicity<br/>mentioned struggling with<br/>intimacy due to PNH.(7/27) gave up<br/>hobbies or activities<br/>entirely due to PNH.work.

<sup>a</sup> A challenging path to diagnosis includes at least one of the following: duration of diagnosis journey > 1 year, seeing multiple HCPs, getting the wrong tests, being hospitalised, being misdiagnosed, having to get help from HCPs in their personal network.

*"Me and [co-patient] Carmen talk about* 

CLINIC

everything on the day of treatment. We laugh and we arrive at the hospital feeling relaxed."

Hospital treatment, e.g., offers the benefits of socialising with other people with PNH and conversing with HCPs.

(13/16) of participants

receiving their treatment

least one of the burdens:

times, the emotional toll

of the hospital setting.

long travel, long wait

in hospital experienced at

81%

## 50%

(14/27) held a positive view of home treatment – e.g., because of fewer logistical hassles –, including some who didn't receive home treatment at the time of the study.

HOME

"I have to worry if the nurse will come on time, and whether we can finish treatment on time, so that I won't be late to take my daughters to their sports."

Another drawback of home treatment is that it can 'medicalise' the home and evoke negative emotional reactions.

## **Breadth of symptoms**

## CLINICALLY WELL-DOCUMENTED SYMPTOMS

Common PNH symptoms such as fatigue and dyspnea burdened participants in daily life despite treatment.

**67%** (18/27) reported that they were moderately or highly affected by their symptoms.

**78%** (21/27) regularly experienced PNH-related fatigue.

## Support network with holes

Participants expressed having to piece together their own support network and the challenges thereof in a rare disease area.



*"I thought that my life was over. Then arrived God, the world specialist on PNH. He took me into his hospital and saved me."* Some develop a strong relationship with their haematologist. However, the

 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).

<b>Disruption of normality</b>	Burden of treatment
Breadth of symptoms	Support network with holes

"Our office is on the 3<sup>rd</sup> floor. For other people, it's just a few steps, but for me, it's the Eiffel tower."

For one of the participants, daily activities, like the flight up the stairs to his office, have become unavoidable challenges due to dyspnea that isn't solved by C5 inhibitor treatment.

### **EXPANDED SYMPTOMS**

There is a wider, and mostly clinically unrecognised, set of symptoms experienced by people with PNH.

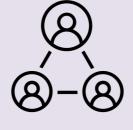
**20+** additional physical, cognitive, and psychological symptoms were associated with PNH by participants.

**48%** (13/27) mentioned that they struggled to get HCPs to recognise and address their expanded symptoms.

"You tell [the clinic] or whatever doctor's office and all they'll say is, 'Well, that's not classic PNH.' [...] It feels as if a patient who lives through it each day doesn't know what they're on about." HCPs predominant experience is nonetheless that HCPs only tend to focus on the medical aspects of PNH.



"I make efforts because I understand that my partner wants a normal life. We try to find common ground, but it's not always easy." Many receive strong support from family or friends, but participants also shared that it can be difficult for others to relate to an "invisible" condition and their symptoms.



FAMILY 8

FRIENDS

*"When members shared stories about life with PNH, it was great to realise that others were going through the same experiences."* 

PATIENT Patient organisations were an integral source of support and information across all three countries. However, they're not used by everyone – 67%

(18/27) were actively involved<sup>b</sup> in their country's respective patient organisation.



(18/27) expressed needing support beyond what they were currently receiving. Some manage to build robust networks, but there are also those with a patchy network, leaving them to carry worries and questions to which there might be answers.

<sup>b</sup> Active involvement in patient organisations meant that participants attended events, connected with other people living with PNH and/or regularly posted in or were ware of news in the organisations' social media groups.

#### References

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#### **Disclosures**

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