

When surviving isn't enough for people with PNH

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The ongoing burden of PNH

With a global incidence of just **1.3 cases per million people**, many of us won't be aware of paroxysmal nocturnal haemoglobinuria (PNH) and the debilitating symptoms it causes.¹

But, for people living with PNH, **the burden is all too familiar.**



Despite receiving current treatments, many people with PNH **still experience debilitating symptoms.**²

These symptoms include **pain, shortness of breath and, commonly, fatigue** that limits daily activities.²

In fact, studies of people with PNH in Europe have revealed that, even when being treated with C5 inhibitors, **86% remain anaemic** and **85% still experience symptoms** that impair their daily lives.²

 Pain

 Shortness of breath

 Fatigue

85%
experience symptoms that impact daily life²

86%
remain anaemic²

“I can't ignore the fatigue after I've done an activity or after I get home from a walk. It's as if a plate breaks – it feels like my body cracks into pieces.”

– Quote from a person living with PNH



Transforming life for people with PNH

A challenge for the healthcare community is that many of these symptoms can't easily be picked up on graphs and charts. Yes, doctors can measure haemoglobin (Hb) levels and check for complications, but improvement isn't the same as normality – **and surviving isn't the same as thriving.**



To me, the measure of how well a person is responding to treatment is, in part, the extent to which **they're still having to modify their lifestyle to accommodate their condition.** If someone's taking time off work, missing social events, or feeling limited in their daily lives, I don't think we can say that their condition is truly under control.

It's important that we consider not just the physical impact of this condition, but **the emotional and financial impact it causes too** – especially in challenging economic times that add further pressure on people with PNH.

Blood tests, scans and questionnaires – they're all essential ways of charting progress, but they don't paint the full picture. Because many people with PNH are so used to putting up with their symptoms, they may not reveal the true toll of their condition.



Transforming life for people with PNH (continued)

To make real progress in improving the quality of life for people living with PNH, **it's crucial that we make it common practice to really question how individuals are feeling** and to recognise how symptoms are still affecting their lives.

Questionnaires used to assess quality of life help gauge how symptoms are affecting a person. If, through conversations and the use of these measures, it becomes clear that PNH is significantly limiting life, despite treatment, **I believe it's our duty to explore how we can provide further support.**



People with PNH shouldn't have to resign themselves to a life of less – doing less, expecting less, receiving less.

Instead, together with the wider healthcare community, it's my hope that we can empower them to ask for more.

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References

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2. Panse J et al. *Eur J Haematol.* 2022;109:351–363.

PP-17510 | February 2023