

Collaborative Initiative With the National PNH Service: Survey Results From a Pegcetacoplan Patient Support Programme

Louise M. Arnold,^{1*} Richard J. Kelly,¹ Austin Kulasekararaj,² Joanna Large,² Catherine Barnfield,¹ Roochi Trikha,² Jill Stephenson,³ Sheena Patel,⁴ Morag Griffin¹

¹St James’s Hospital, Leeds, UK; ²King’s College Hospital NHS, London, UK; ³HealthNet Homecare (UK) Ltd; ⁴Sobi Ltd, Great Abington, UK
*louisearnold@nhs.net

CONCLUSIONS

- This collaborative service is the first patient support programme in the United Kingdom to offer self-administered treatment for paroxysmal nocturnal haemoglobinuria.
- The patient satisfaction survey reported strong positive patient feedback, as well as confidence in self-administration of pegcetacoplan.
- Overall, response rates of this small patient group are consistent with results of similar broader patient surveys, with responses reflecting dedication to managing a rare disease.^{1,2}

INTRODUCTION

- Paroxysmal nocturnal haemoglobinuria (PNH) is a rare disease characterised by complement-mediated haemolysis, anaemia, thrombosis and concurrent bone marrow failure, which could lead to serious morbidity and, if untreated, mortality.³
- Due to advancements in therapies,³ patients with a diagnosis of PNH have near-normal life expectancy, enabling treatment focus to shift onto autonomy over management.

The UK PNH National Service

- The UK PNH National Service offers centralised expertise for patients, using a multi-disciplinary team approach to manage new diagnoses, monitoring complexities and practical considerations of treatment.⁴
- Pegcetacoplan is reimbursed as monotherapy for PNH in adults who have anaemia after at least 3 months of treatment with a C5 complement inhibitor.⁵
- Pegcetacoplan is a self-administered subcutaneous infusion, prescribed twice weekly or every 3 days, with comprehensive training provided to patients to ensure proper use.⁶

Pegcetacoplan homecare patient support programme

- The pegcetacoplan homecare patient support programme (PSP) was launched in 2021 as a collaborative initiative between the PNH National Service, a homecare provider (HealthNet Homecare [UK] Ltd), and industry (Sobi Ltd) to provide tailored support to patients with PNH who receive self-administered treatment with pegcetacoplan, via self-administration training and an ongoing nurse support contact. Sobi support does not extend to service delivery and patient anonymity is maintained.
- Patient satisfaction surveys are commonly used to measure value of healthcare initiatives and identify areas for development.¹

AIM

- Here, we present results of a patient satisfaction survey to assess the patient experience and success of an established PSP for patients receiving pegcetacoplan and to identify further patient-identified support requirements.

METHODS

- In June 2024, in line with the National Homecare Medicines Committee (NHMC) guidance, a survey was sent to all patients in the homecare PSP.
- Questions included user satisfaction on aspects of the service, educational support and practical considerations of treatment.
- A tailored assessment tool, Patient Activation Measure (PAM), was integrated into the service to assess levels of confidence with administration and identify areas for individualised patient support.

References
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Disclosures
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Abbreviations
NHMC: National Homecare Medicines Committee; PAM: Patient Activation Measure; PNH: paroxysmal nocturnal haemoglobinuria; PSP: patient support programme; UK: United Kingdom.

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RESULTS

Of the 33 patients who received the survey, 24% (n=8) patients responded.



100%
rated their PSP experience as very good/good (Figure 1).



86%
felt satisfied with training provided (Figure 1).



100%
patients who were aware of the PAM assessment reported it as being 'effective and useful'.

Reflecting on treatment administration:



88%
of responders preferred self-administration (Figure 2).



86%
felt confident with the infusion process, all acknowledging the service and support provided for building this confidence (Figure 3).

Practical considerations:



100%
had no concerns with storing medication or ancillaries.



33%
had experience travelling with medicine or ancillaries and of these, no problems were reported (Figure 4).

