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

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## 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.<sup>1,2</sup>

## 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.<sup>3</sup>
- Due to advancements in therapies,<sup>3</sup> 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.<sup>5</sup>
- Pegcetacoplan is a self-administered subcutaneous infusion, prescribed twice weekly or every 3 days, with comprehensive training provided to patients to ensure proper use.<sup>6</sup>

## 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.<sup>1</sup>

## 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.

### RESULTS

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



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:



self-administration (Figure 2).



**Practical considerations:** 

medication or ancillaries.



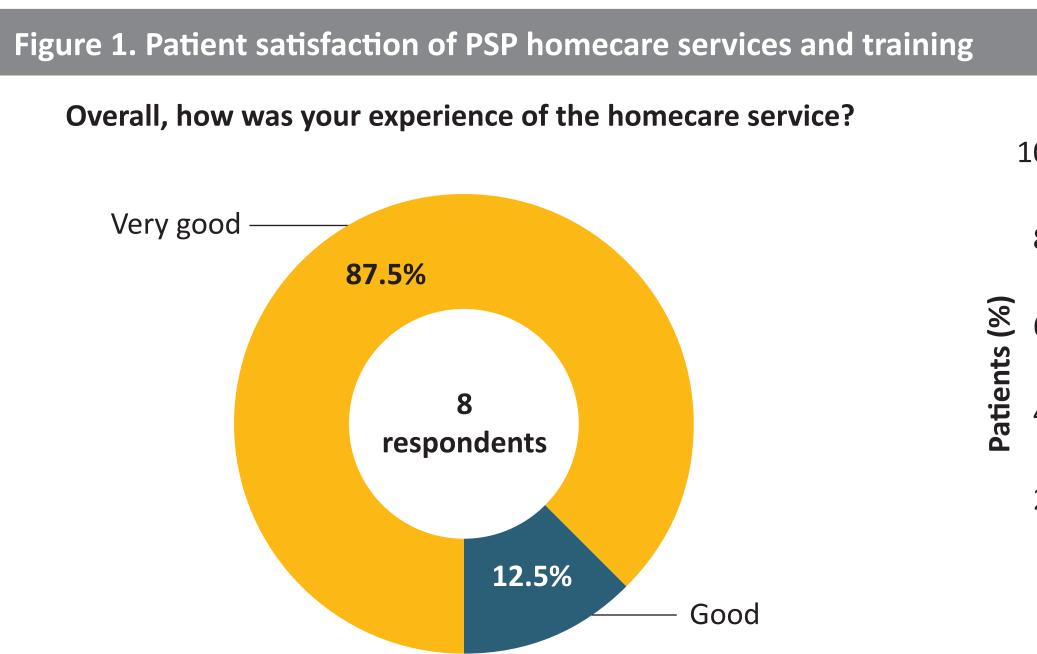
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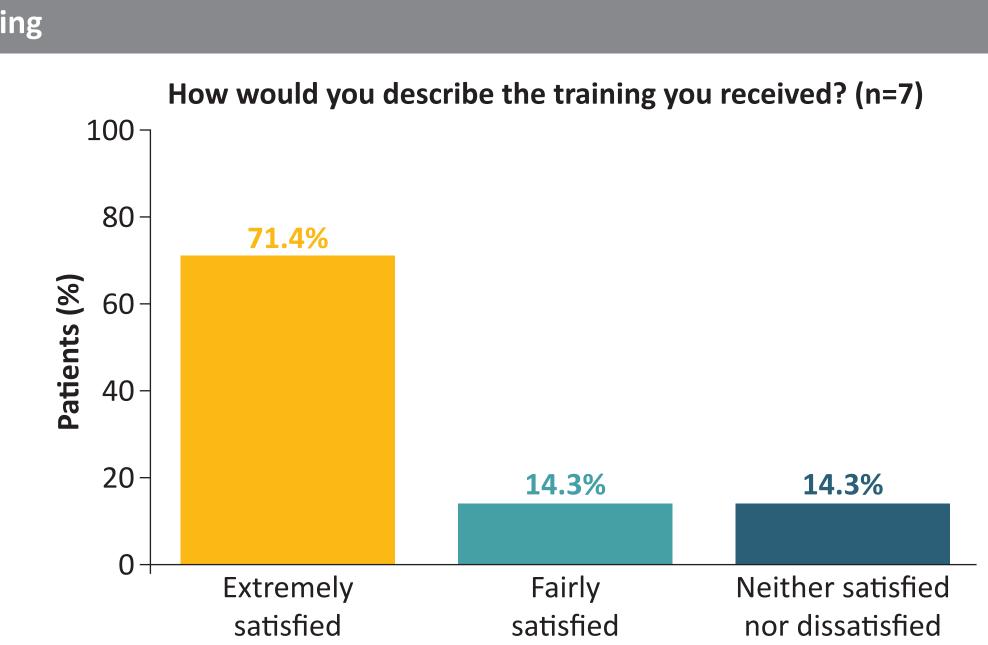
had experience travelling with medicine or ancillaries and of these, no problems were reported (Figure 4).

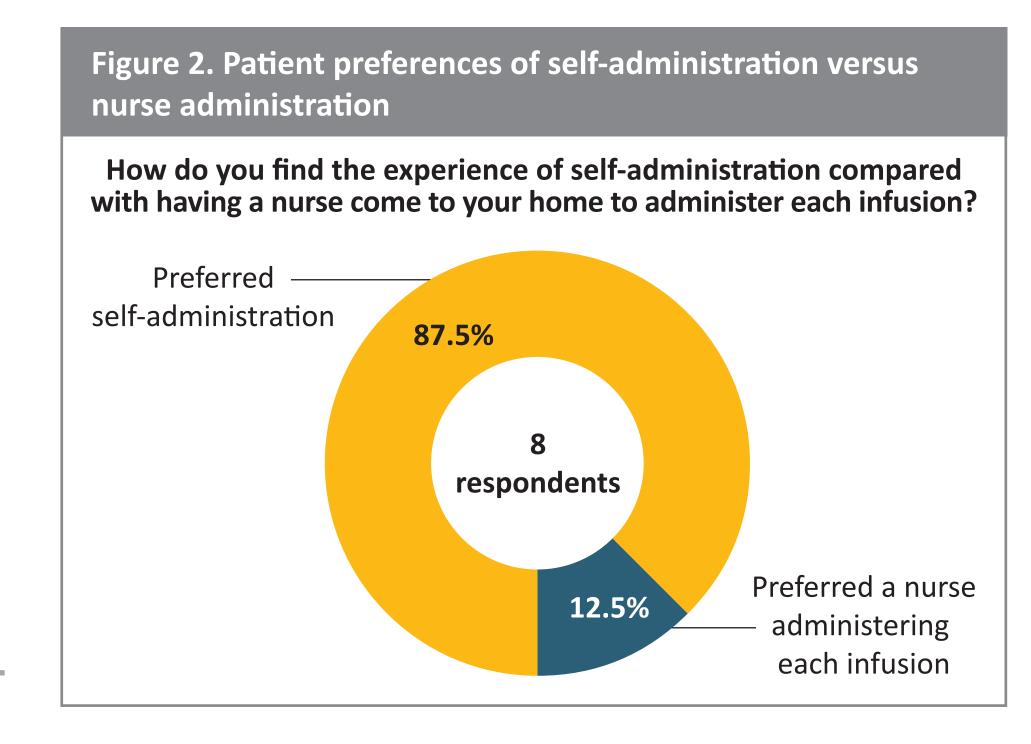


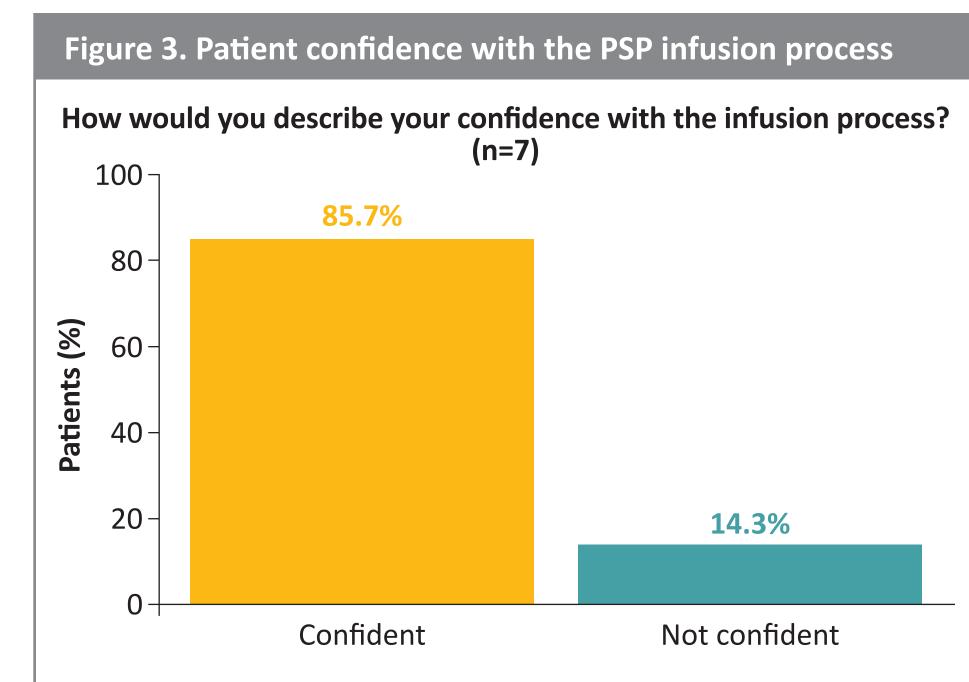
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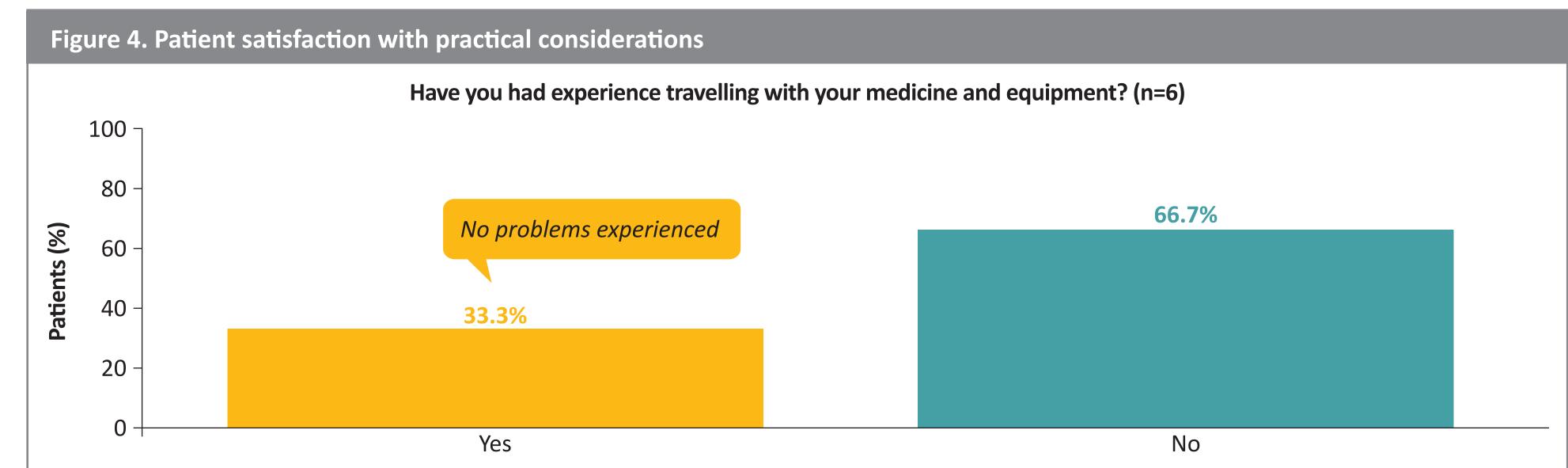
felt confident with the infusion process, all acknowledging the service and support provided for building this confidence (Figure 3).











#### References

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**Disclosures** LA: consultant at Alexion and Sobi; advisory board member at Alexion, AstraZeneca and Sobi; received honoraria from Alexion and Sobi. RJK: fees/research funds from Alexion, Amgen, Florio, Novartis, Omeros, Otsuka, Roche and Sobi; travel benefits for ASH 2022/23/24 from Sobi. AK: consultant for Akari, Alexion, Amgen, Apellis, BioCryst, Celgene/BMS, F. Hoffmann-La Roche, Novartis, Novo Nordisk, Pfizer, Ra Pharma and Sobi; receives honoraria from Achillion, Agios, Akari, Alexion, Amgen, Apellis, BioCryst, Celgene/ BMS, F. Hoffmann-La Roche, Novartis, Novo Nordisk, Pfizer, Ra Pharma, Silence Therapeutics and Sobi; member of board of directors at Amgen, Celgene/BMS, F. Hoffmann-La Roche and Novartis; receives research funding from Celgene/BMS and Novartis; speaker for Achillion, Akari, Alexion, Amgen, Apellis, BioCryst, Celgene/BMS, F. Hoffmann-La Roche Ltd, Novartis, Novo Nordisk, Pfizer, Ra Pharma and Sobi. JL: receives speaker/advisory fees for Alexion/AstraZeneca, Novartis, Pfizer, Roche and Sobi; consultant at Alexion, Novartis and Sobi; advisory board member at Alexion, AstraZeneca, Novartis and Sobi; received honoraria from Alexion, Novartis and Sobi. RT: receives honoraria from Alexion AstraZeneca Rare Disease, Novartis and Sobi. Swedish Orphan Biovitrum AB; member of the board of directors or advisory committees at Alexion AstraZeneca Rare Disease, Novartis and Swedish Orphan Biovitrum AB. JS: Employee of HealthNet Homecare (UK) Ltd. SP: employee of Swedish Orphan Biovitrum AG. MG: consultant for Biocryst and Regeneron Pharmaceuticals; member of the board of directors or advisory committees at Alexion, Amgen, Novartis, Omeros, Pfizer and Sobi; speaker for Alexion, Amgen, Novartis and Pfizer.

## **Abbreviations**

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

## **Acknowledgements**

We thank the patients and investigators who participated in this study. The authors acknowledge Sana Yaar, PhD, Costello Medical, UK, for medical writing and editorial assistance, and Melanie Howes, Costello Medical, UK for design assistance, funded by Sobi. This study is funded by Sobi



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