Patient preference for PNH treatments: analysis from the Q-methodology of treatment attributes

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Jesse Fishman[1]; Koo Wilson[2]; Simon Middleton[3]; Leah Ralph[3]; Juan Salcines[3]

[1]Apellis Pharmaceuticals, Inc., Waltham, MA, USA; [2]Swedish Orphan Biovitrum AB, Stockholm, Sweden; [3]L.E.K. Consulting, Boston, MA, USA

CONCLUSIONS

- ✓ Patients with paroxysmal nocturnal hemoglobinuria (PNH), some of whom had aplastic anemia, reported that PNH and associated symptoms are impacting their mobility, daily activities, and experience of anxiety or depression
- ✓ Clinical outcomes (fatigue, blood transfusions) and dosing frequency were the most important attributes of PNH treatments
- ✓ Patients reported that dosing frequency may impact compliance

INTRODUCTION

- PNH is an acquired, rare, chronic, potentially life-threatening disease, characterized by complement dysregulation and red blood cell destruction[1]
- Patients with PNH can experience a variety of symptoms, including hemolysis and thrombosis, and may require blood transfusions, potentially leading to reduced quality of life (QoL) and fatigue[1,2]
- Complement component 5 (C5) inhibitors (eculizumab and ravulizumab) have significantly improved life prognosis for patients with PNH; however, some patients continue to experience challenges with a burdensome administration regimen and persistent symptoms[3–5]
- Pegcetacoplan, an approved subcutaneously administered complement component 3 (C3) inhibitor, improved hematological outcomes as compared with eculizumab treatment over 48 weeks in patients with PNH who were anemic while on eculizumab[6–9]
- New drugs are under development focusing on improving clinical outcomes as well as different administration options

OBJECTIVE

Quantitatively assess patient perspective of PNH impact on QoL, and effect of product characteristics on treatment preferences and compliance

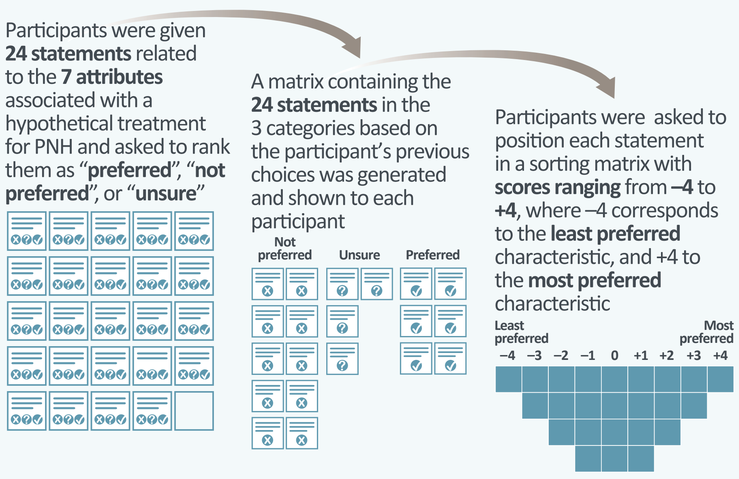
METHODS

- Adult (≥18 years) patients with PNH were recruited from the United States (n=117), Canada (n=12), France (n=18), and Germany (n=9) via PNH patient associations; patients were included regardless of treatment history or time of diagnosis; patients received honoraria
- Patients completed a 30-minute online survey, with 3 main sections:
  - Demographics (excluding identifiable information)
  - Impact of PNH and hemolytic symptoms on QoL (1–5 rating [very low–very high impact])
  - Existing PNH treatment preferences (ranking by Q-sort methodology) and impact on compliance (1–5 rating [least helpful–most helpful treatment features])
- Surveys were completed between September 2022 and June 2023

Q-sort methodology[10]

- Patients were presented statements on PNH treatments and rated them as "preferred", "not preferred", or "unsure"
  - Patients then positioned statements into a scoring matrix ranking from least (–4) to most (+4) preferred (**Figure 1**)

Figure 1. Q-sort methodology



METHODS *(cont.)*

- The 24 statements presented to patients were developed from **7 attributes** of available PNH treatments selected from qualitative research (patient interviews [n=4] and key differentiation points between treatments), each divided into 3–5 levels (**Table 1**)

Table 1. Attributes and levels used to create PNH treatment statements

Attribute	Statement (abbreviated)
Level of fatigue (from PNH despite taking treatment)	1. You are unlikely to feel at all fatigued 2. You may feel somewhat fatigued 3. You may feel very much fatigued
Number of blood transfusions expected per year (while taking the treatment)	4. You are unlikely to require any blood transfusions 5. You may require 1 blood transfusion per year 6. You may require 2 blood transfusions per year 7. You may require 3 blood transfusions per year 8. You may require 4 or more blood transfusions per year
Route of administration	9. Subcutaneous infusion with syringe pump 10. Subcutaneous infusion with wearable on-body device 11. Intravenous infusion
Needle characteristics	12. Needle is 6–10 mm long and visible 13. Needle is 6–10 mm long and always hidden 14. Needle is 20–30 mm long and visible
Treatment setting	15. At hospital/local clinic by medical professional 16. At home by visiting medical professional 17. Self-administered at home/work
Mobility during treatment administration	18. Allows for mobility to complete daily tasks 19. Allows for limited mobility 20. Not able to move
Dosing frequency	21. Twice per week 22. Once per week 23. Once every 2 weeks 24. Once every 8 weeks

RESULTS

Demographic and clinical characteristics

- Most of the 156 patients with PNH who participated were females (73.1%) and of younger age categories (range 18 to <70 years) (**Table 2**)
- Average time since diagnosis was 9.9 years
- Most patients (85.3%) were currently taking complement inhibitor therapy for a mean ± SD of 6 ± 5 years (**Table 2**)

Table 2. Patient demographics, clinical, and treatment characteristics (N=156)

Demographics, n (%)	
Age range, years	
18–40	65 (41.7)
41–60	58 (37.2)
61–70+	33 (21.2)
Female sex	114 (73.1)
Clinical characteristics, mean ± SD (unless otherwise noted)	
Years since initial PNH diagnosis	9.9 ± 9.3
Diagnosed with aplastic anemia, n (%)	55 (35.3)
Previously received blood transfusions, n (%)[a]	84 (53.8)
Months since last hemoglobin concentration	0.8 ± 1.5
Last hemoglobin concentration (g/dL)	10.8 ± 1.9
Current treatment, n (%)[b]	
Anticoagulants	45 (28.8)
Supplements	111 (71.2)
Complement inhibitor therapy	133 (85.3)
Steroids or other immunosuppressants	15 (9.6)
Other	17 (10.9)
Years of current treatment duration, mean ± SD	
Anticoagulants	9.60 ± 7.7
Supplements	10.57 ± 10.3
Complement inhibitor therapy	6.33 ± 4.9
Steroids or other immunosuppressants	12.07 ± 11.1
Other	4.35 ± 5.0

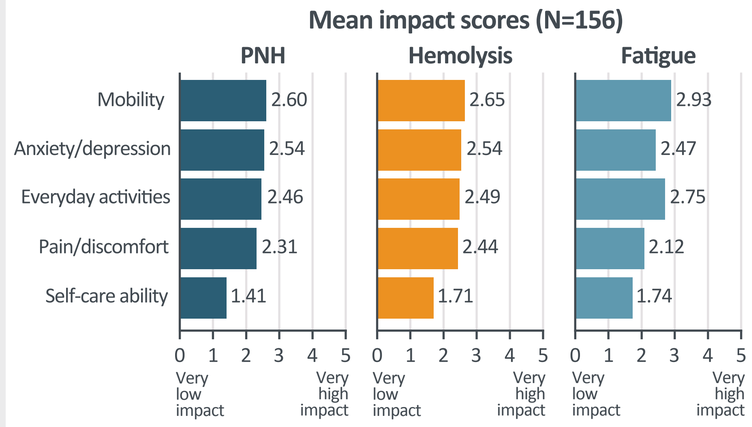
[a]Over their lifetime.
[b]Several patients were currently under multiple treatment regimens.
SD, standard deviation.

RESULTS *(cont.)*

Disease burden on patients' quality of life

- Impacts of PNH in general, as well as breakthrough hemolysis and fatigue specifically, were moderate; impacts were greatest on mobility, ability to do everyday activities, and anxiety or depression (**Figure 2**)

Figure 2. Impact of PNH and related symptoms on patients' QoL

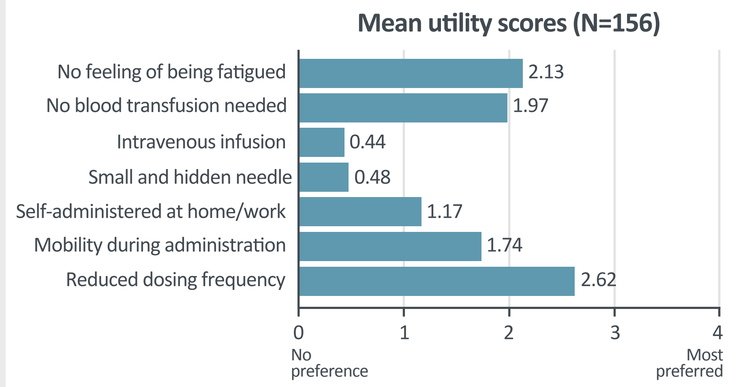


PNH, paroxysmal nocturnal hemoglobinuria; QoL, quality of life.

Treatment preferences

- Overall, the clinical outcomes of improved fatigue and reduced need for blood transfusions, as well as reduced dosing frequency had the greatest preference among patients (**Figure 3**)
- Mobility during administration and ability to self-administer at home or work were preferred, as well as a hidden and small-size needle (**Figure 3**)
- Scores were directionally similar when comparing patients in Europe and Canada versus the United States, but reduced dosing frequency (+2.77 vs +2.57) and self-administration (+1.56 vs +1.03) had a slightly greater importance than other clinical attributes for those in Europe and Canada versus the United States

Figure 3. Preference of available PNH treatment attributes (in isolation)

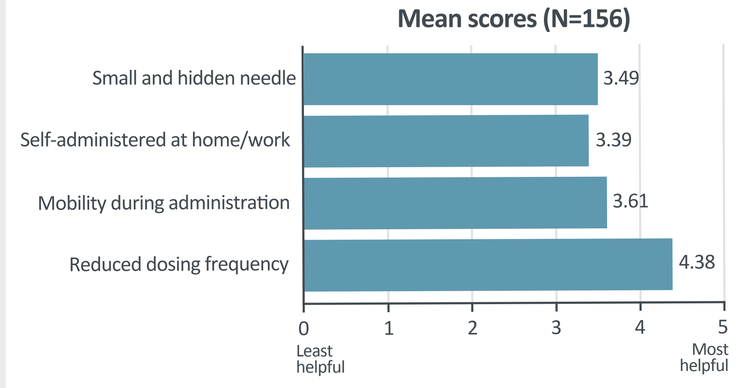


PNH, paroxysmal nocturnal hemoglobinuria.

Impact of treatment preferences on compliance

- Patients reported that dosing frequency had the greatest impact on treatment compliance versus other product characteristics (**Figure 4**)

Figure 4. Impact of product characteristics on treatment compliance



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