Patient satisfaction with use of complement inhibitor injector in paroxysmal nocturnal hemoglobinuria in the United States

Brian Mulherin[1,2]; Lawton Laurence[3]; Carl Friddle[3]; Jed Perler[4]; Logan Locklear[4]; Lisa Bailey[4]; Amanda Cassidy[4]; Dharmik Desai[3]; Carlos de Castro[5]

[1]Hematology Oncology of Indiana, Indianapolis, IN, USA; [2]Ascension St. Vincent Carmel, Carmel, IN, USA; [3]Apellis Pharmaceuticals, Inc., Waltham, MA, USA; [4]Trinity Life Sciences, Waltham, MA, USA; [5]Duke University, Durham, NC, USA

CONCLUSIONS

- ✔ These preliminary real-world findings suggest that most patients with PNH were satisfied with multiple aspects of their experience with the new single-use, wearable injector, and would likely recommend it to other patients with PNH
- ✔ All patients found the injector easy to use without assistance and most were confident in the administration
- ✔ Surveyed patients seemed to prefer the new device and were more confident using it than their previous device
- ✔ Although responses might have been impacted by memory and social desirability biases, these real-world findings support those from the initial human factors evaluation study

INTRODUCTION

- Paroxysmal nocturnal hemoglobinuria (PNH) is a rare, acquired hematological condition characterized by complement-mediated hemolysis that can lead to anemia and thrombosis[1,2]
- The natural course of PNH has improved with the development and approval of complement inhibitors, initially with the C5 inhibitors (C5i) eculizumab and ravulizumab,[3] followed by the first C3/C3b inhibitor pegcetacoplan[4–7]
- In contrast with eculizumab and ravulizumab administered intravenously in health care settings, pegcetacoplan can be self-administered at home using a general-use drug delivery system, such as an ambulatory, subcutaneous (SC) infusion pump[4,5]
- On September 28, 2023, a new wearable, single-use, SC pegcetacoplan injector with a hidden needle was approved by the US Food and Drug Administration after validation for easy, safe, and effective use in a human factors study[8,9]

OBJECTIVE

Describe patients' experience with the new single-use, wearable injector for PNH treatment in 2 real-world, US, 2023–2024 studies

METHODS

First study: US, cross-sectional, mixed methods (survey and interview) study to assess user experience with injector quantitatively and qualitatively (initiated November 2023, with target enrollment of 15 patients)

- Participants: adults with PNH diagnosed for ≥6 months, who self-administered pegcetacoplan for >3 months and switched to the new injector, with ≥6 weeks follow-up available
- Procedures:
  - A. Preliminary web-based survey including 18 questions with responses given on a 1–7 Likert scale (1-worst, 7-best) related to user's experience in performing pegcetacoplan administration with the injector
    - Domains of evaluation included confidence (3 items), ease of use (6 items), convenience (1 item), satisfaction (2 items), fear and anxiety (2 items), benefits (3 items), and preference over previous device (1 item)
  - B. Qualitative, 45-minute telephone interview to discuss and rate administration experience with the new injector compared with prior device and C5i therapies
    - Quantitative (rating of experience) evaluation included 10 questions with responses given on a 1–7 Likert scale (1-worst, 7-best) and 1 question ("overall administration experience") rated from 1 (worst) to 100 (best)
- Interim, quantitative analysis (as of July 15, 2024), ahead of full enrollment and analysis: report response ratings on select items from the web-based survey and interview

Second study: US, cross-sectional survey of user experience with injector (conducted in 2024)

- Participants: all patients who recently (~2 weeks) converted from an infusion pump to the new injector for their pegcetacoplan treatment and were accessible through the ApellisAssist Support program
- Procedures: email and text messaging surveys sent out by care coordinators from the ApellisAssist support program, consisting of 7 questions with multiple-choice answers
  1. How many times have you used the injector? 2, 3, 4, 5, 5+
  2. Are you satisfied with the injector? Yes/no
  3. Do you prefer the injector compared to your previous device? Yes/no
  4. Do you feel more confident about self-administration with the injector compared to the previous device? Yes/no
  5. It is faster to set up for the treatment with the injector compared to the previous device? Yes/no
  6. Does the hidden needle in the injector help you feel more at ease about your infusions? Yes/no
  7. Do you feel more mobile during your infusions with the injector compared to the previous device? Yes/no
- Analysis: report frequencies of responses

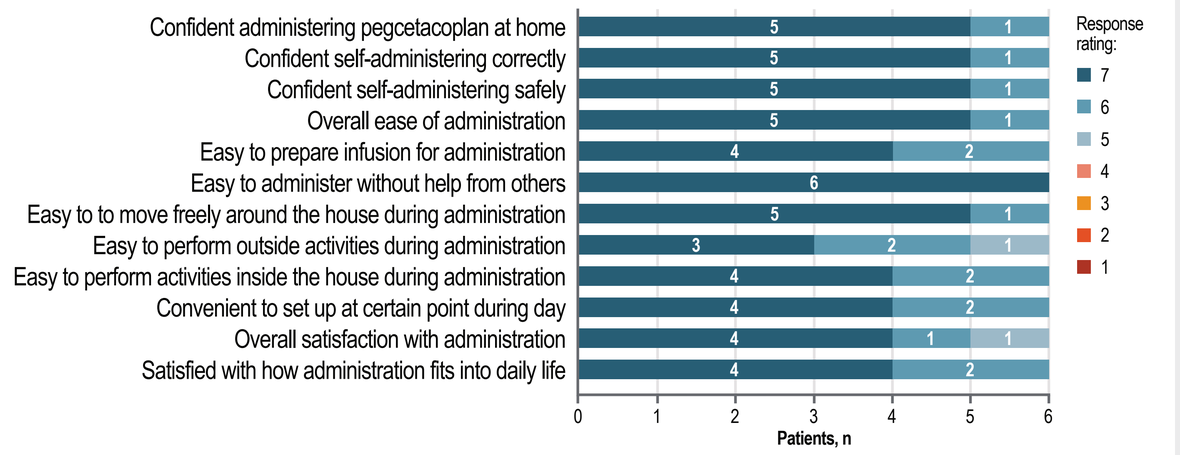
RESULTS

Injector users' experience mixed-methods study: 7 patients were recruited as of July 15, 2024

A. Preliminary web-based survey results (N = 6; 1 missing):

- On a scale of 1 to 7, where 1 was "not at all confident" and 7 was "very confident", 5 of 6 patients reported being very confident (7-rating) and 1 being confident (6-rating) about administering pegcetacoplan at home (**Figure 1**)
- On a scale of 1 to 7, where 1 was "not at all easy" and 7 was "very easy", 6 of 6 patients reported it was very easy (7-rating) to administer pegcetacoplan with the injector without help from others (**Figure 1**)

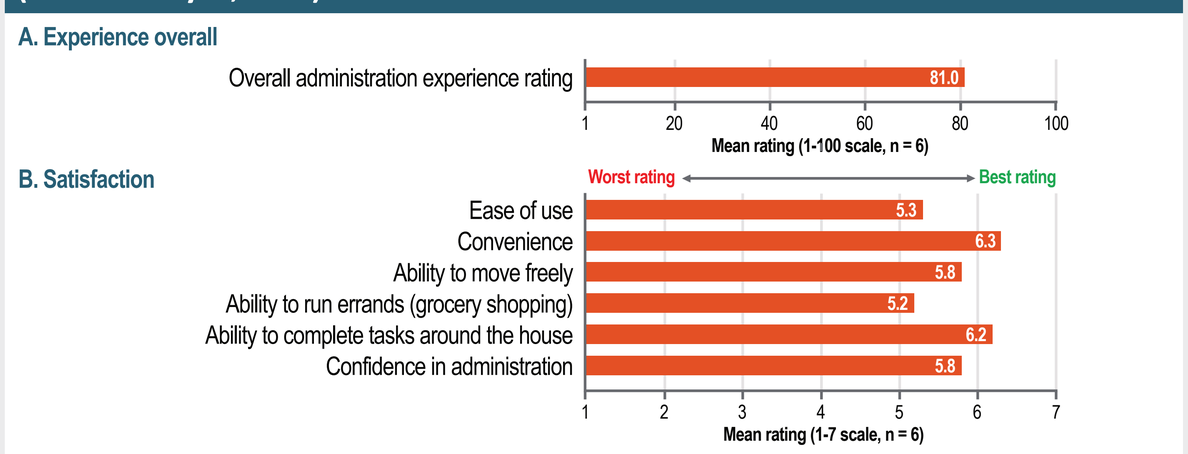
Figure 1. Patient responses to web-based survey from mixed-methods injector experience study (interim analysis; N = 6)



B. Preliminary telephone interview survey results (N = 7):

- Among 6 of the 7 patients who had evaluable data,
  - The mean "overall administration experience" ratings was 81 (1–100 scale from worst to best experience) (**Figure 2A**)
  - The mean experience satisfaction ratings (1–7-scale) were 6.3 for convenience, 6.2 for ability to complete tasks around the house, 5.8 for ability to move freely and confidence in the administration, 5.3 for ease of use, and 5.2 for ability to run errands (**Figure 2B**)
  - Notably, 5 of the 6 patients consistently generally gave high ratings for their experience with the new injector
- All 7 interviewed patients were very (n = 1) or extremely (n = 6) likely to recommend the new device to other patients with PNH (mean rating = 6.9; 1–7-scale [1-not at all likely; 7-extremely likely])

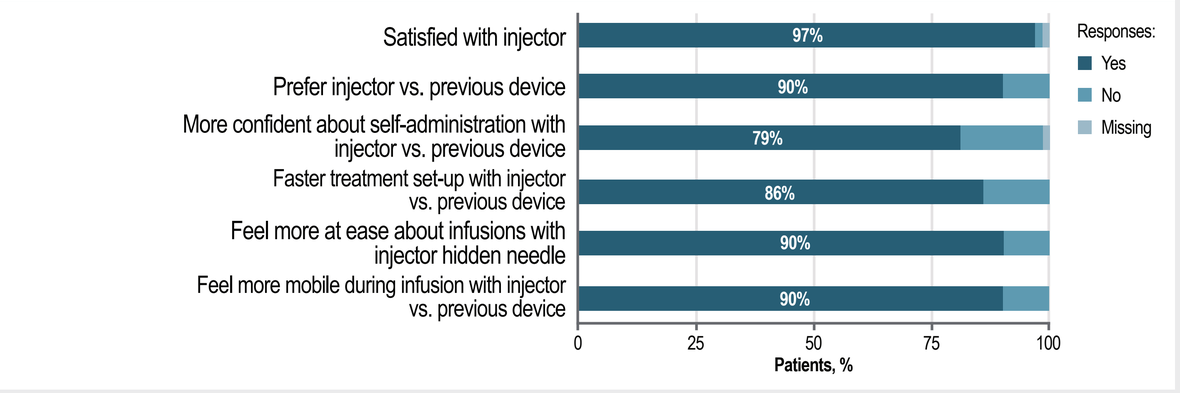
Figure 2. Patient responses to interview from mixed-methods injector experience study (interim analysis; N = 7)



Injector users email/text messaging survey: 58 patients completed and returned the survey

- Of these, 72% (42/58) had used the injector ≥5 times
- Almost all patients (97%; 56/58) were satisfied with the new injector and preferred it over their previous device (90%; 52/58) (**Figure 3**)

Figure 3. Patient response to email/text messaging survey from ApellisAssist support program (N = 58)



2nd International PNH Interest Group (IPIG) Conference
May 15–16, 2025 | Paris, France

Data previously published: Brian Mulherin, Lawton Laurence, Carl Friddle, Jed Perler, Logan Locklear, Lisa Bailey, Amanda Cassidy, Dharmik Desai, Carlos M de Castro; Patient Satisfaction with Use of Complement Inhibitor Injector in Paroxysmal Nocturnal Hemoglobinuria in the United States. *Blood* 2024; 144 (Supplement 1): 5673. doi: https://doi.org/10.1182/blood-2024-200983

REFERENCES: 1. Schrezenmeier H, et al. *Ann Hematol*. 2020;99:1505–14. **2.** Waheed A, et al. *Blood Rev*. 2024;64:101158. **3.** Brodsky RA. *Blood*. 2021:137:1304–9. **4.** EMPAVELI® (pegcetacoplan) US PI 2024. **5.** ASPAVELI Summary of Product Characteristics 2024. **6.** Horneff R, et al. *Int J Mol Sci*. 2024;25:8698. **7.** Hillmen P, et al. *Int J Mol Sci*. 2024;25:9477. **8.** EMPAVELI® Injector (pegcetacoplan) Instructions for use 2023. **9.** Shahin H, et al. *TheResearchPost*. https://doi.org/10.53705/241008.

Abbreviations: C5i, complement 5 inhibitor; PNH, paroxysmal nocturnal hemoglobinuria; SC, subcutaneous.

Disclosures: BM has nothing to disclose. **HS**, **LLaurence**, **CF**, **DK**, and **DD** are employees of Apellis and hold stock or stock options. **JP**, **LLocklear**, **LB**, and **AC** are employees of Trinity Life Sciences; **LB** and **AC** hold stock or stock options. **CdC** reports consultancy at Apellis and Genentech; data safety monitoring board at Regeneron; advisory board at Omeros; honoraria from Novartis, Alexion, Biocryst, Apellis, and Genentech; and research funding from Alexion and Apellis.

Acknowledgments: These studies were funded by Apellis Pharmaceuticals, Inc. Writing assistance was provided by Catherine Champagne, PhD, of Kay Square Scientific (Newton Square, PA, USA), and was funded by Apellis Pharmaceuticals, Inc.

Prior publication: Data published in part in Mulherin B, et al. *Blood*. 2024;144[suppl 1]:5673–5674. https://doi.org/10.1182/blood-2024-200983