

Health State Utility Values for Patients with Paroxysmal Nocturnal Hemoglobinuria using EQ-5D-5L

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KEY FINDINGS & CONCLUSIONS

- The analysis showed that patients with PNH who neither required transfusions nor presented anemia had the highest utility values compared to other health states.
- Treatment-specific utilities indicated higher values for iptacopan than for C5i, for all health states, reflecting potential additional value of orally administered iptacopan.
- Treatments which allow hemoglobin normalization and transfusion avoidance for a majority of patients will contribute to improvement of patient outcomes.

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INTRODUCTION

- Paroxysmal nocturnal hemoglobinuria (PNH) is a rare blood disorder characterized by complement mediated hemolysis.¹
- Patients with PNH often experience anemia, fatigue and require blood transfusions.²
- With several treatments approved (including complement 5 inhibitors [C5i]: intravenous infusions,^{3,4,5,6} iptacopan: first oral monotherapy, factor-B inhibitor^{7,8} etc), health state utilities are important parameters in economic models that aid in decision making in health technology assessments.

AIM

- This analysis aimed to derive health state utility values for a cost-effectiveness model of iptacopan vs C5i in PNH patients.

METHODS

- EQ-5D-5L data were collected from pivotal trials of iptacopan in adults with PNH, APPOINT-PNH⁹ and APPLY-PNH.⁹
- Responses were mapped to EQ-5D-3L, then to a utility index based on UK tariffs.¹⁰
- A mixed linear model for repeated measures was fit to derive utility values for three health states: 1) "no transfusion and no anemia", 2) "no transfusion and anemia" and 3) "transfusion" (received within 4 weeks prior to a study visit).
- Anemia was defined based on two threshold levels of hemoglobin (Hb): 1) Hb <10 g/dL and 2) <10.5 g/dL.
- Data from both trials were pooled for model fitting to enhance sample size and precision of model coefficients.
- Covariates included in the final model were health state, treatment (iptacopan vs C5i), follow-up visit, baseline utility, and study (APPLY-PNH vs APPOINT-PNH) (Table 1).
- For both thresholds of Hb, treatment-pooled (iptacopan and C5i arms) and treatment-specific health state utilities are presented.

Table 1. Health state utility statistical model for APPLY-PNH and APPOINT-PNH

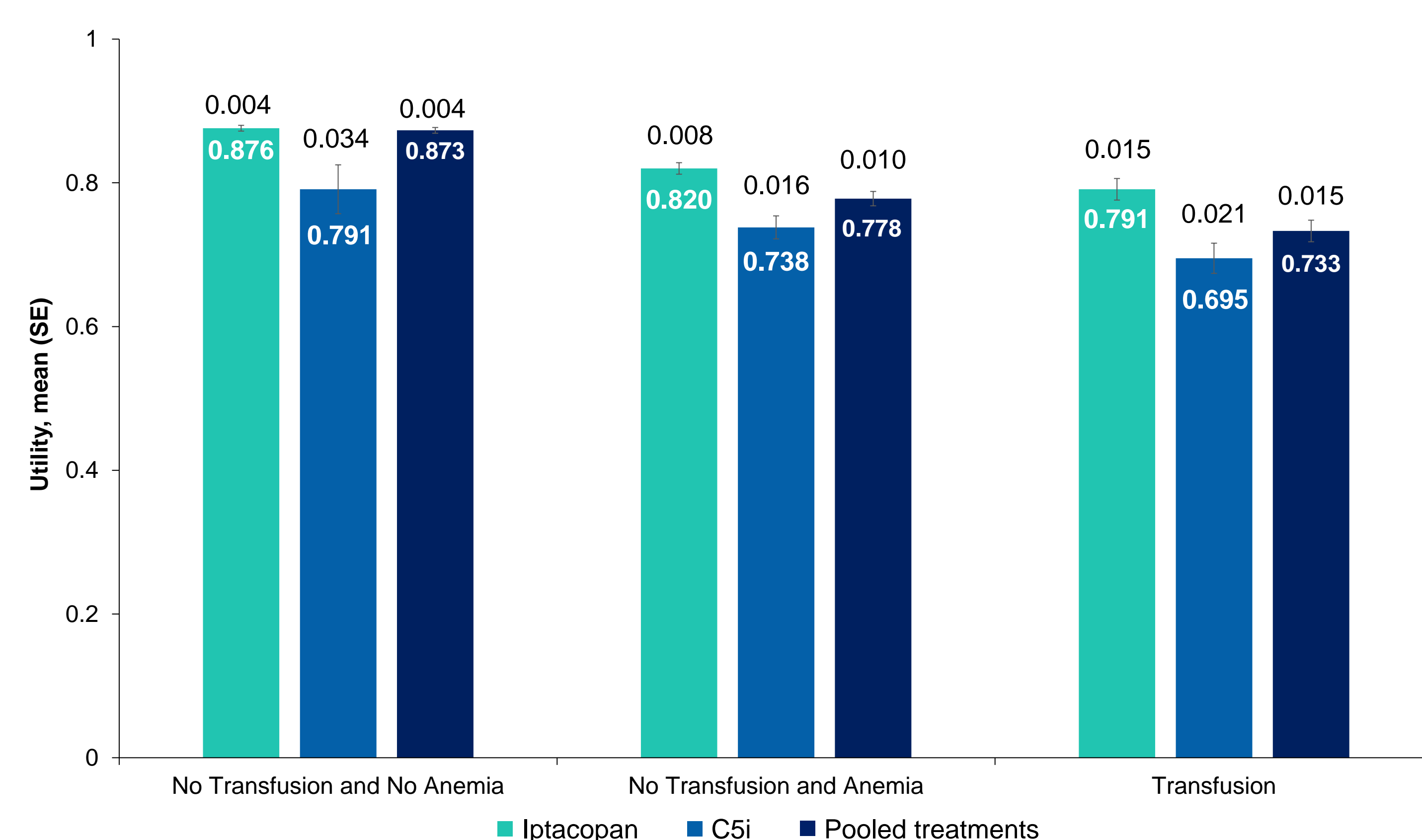
Covariate	Hb <10.0 g/dL		Hb <10.5 g/dL	
	Coefficient (SE)	95% CI (lower, upper)	Coefficient (SE)	95% CI (lower, upper)
Intercept	0.788 (0.028)	0.733, 0.843	0.790 (0.028)	0.735, 0.845
Health state (reference: Transfusion)				
No Transfusion and Anemia	0.008 (0.014)	-0.020, 0.036	0.007 (0.014)	-0.021, 0.035
No Transfusion and No Anemia	0.024 (0.016)	-0.008, 0.056	0.029 (0.016)	-0.003, 0.061
Treatment (iptacopan vs C5i)	0.075 (0.022)	0.032, 0.118	0.071 (0.022)	0.027, 0.114
Follow-up visit (reference: Day 168)				
Baseline (Day 1)	-0.079 (0.016)	-0.111, -0.048	-0.076 (0.016)	-0.107, -0.045
Day 14	-0.029 (0.014)	-0.056, -0.001	-0.026 (0.014)	-0.054, 0.002
Day 42	-0.015 (0.013)	-0.041, 0.011	-0.013 (0.013)	-0.039, 0.013
Day 84	-0.004 (0.013)	-0.030, 0.022	-0.003 (0.013)	-0.029, 0.023
Day 126	-0.013 (0.013)	-0.039, 0.013	-0.013 (0.013)	-0.039, 0.014
Day 140	-0.019 (0.013)	-0.045, 0.007	-0.019 (0.013)	-0.045, 0.007
Day 154	-0.010 (0.013)	-0.037, 0.016	-0.010 (0.013)	-0.036, 0.016
Baseline utility	0.488 (0.038)	0.413, 0.563	0.487 (0.038)	0.412, 0.562
Study (APPLY-PNH vs. APPOINT-PNH)	-0.017 (0.018)	-0.053, 0.018	-0.019 (0.018)	-0.055, 0.017

C5i: Complement 5 Inhibitor; CI: Confidence Interval; Hb: Hemoglobin; SE: Standard Error

RESULTS

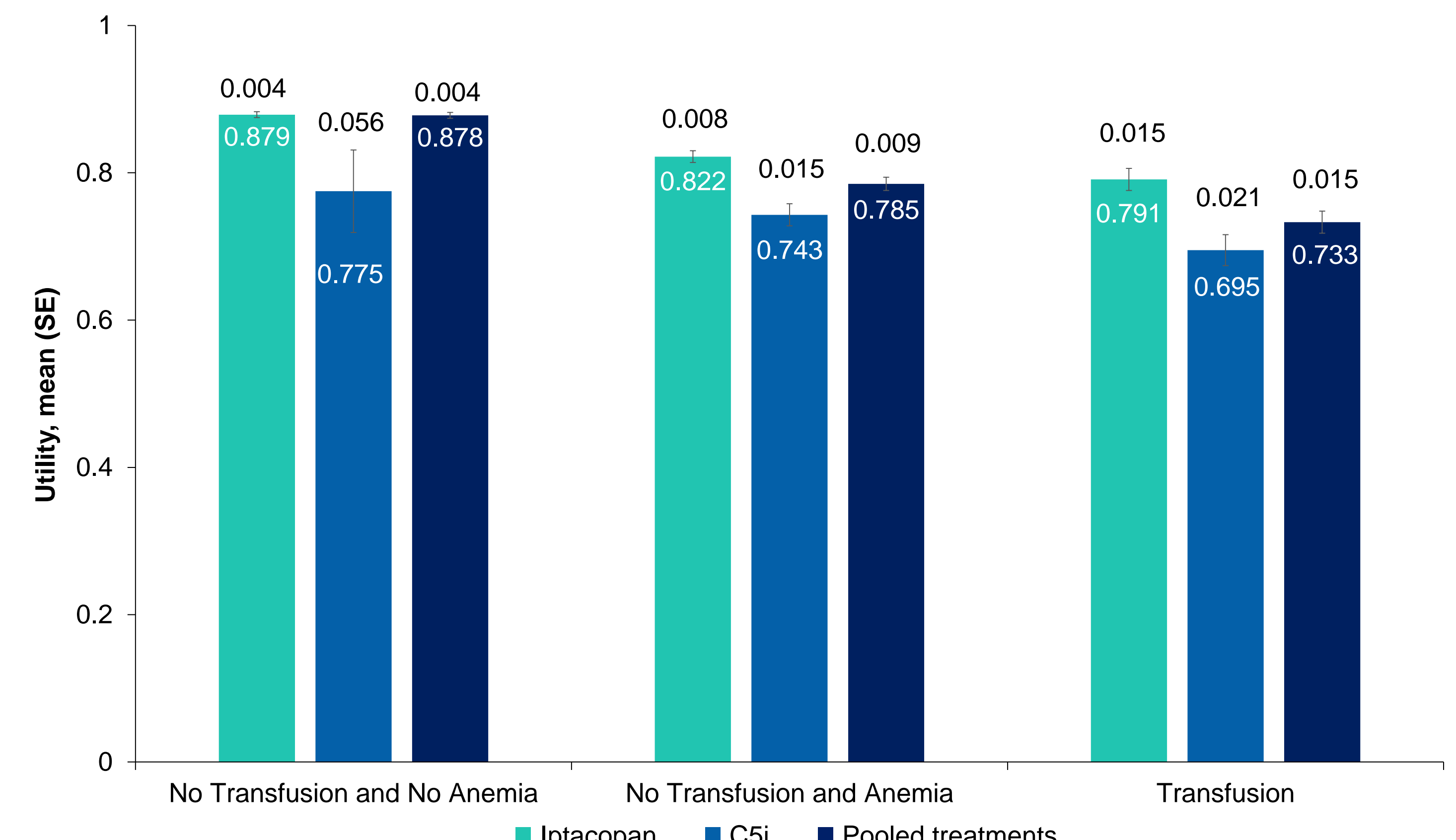
- Utility values when anemia was defined as Hb <10 g/dL (Figure 1).
 - Treatment-specific & by health states:** The utility values were higher with iptacopan compared to C5i, for all the three health states, respectively.
 - Treatment-pooled & by health states:** The utility values were higher among patients who were in the "no transfusion and no anemia" health state compared to the other two health states.
- Utility values when anemia was defined as Hb <10.5 g/dL (Figure 2).
 - Treatment-specific & by health states:** The utility values were higher with iptacopan compared to C5i, for all three health states, respectively.
 - Treatment-pooled & by health states:** The utility values were higher among patients who were in the "no transfusion and no anemia" health state compared to the other two health states.

Figure 1. Health state utility values for Hb <10.0 g/dL



C5i: Complement 5 Inhibitors; Hb: Hemoglobin; SE: Standard Error

Figure 2. Health state utility values for Hb <10.5 g/dL



References

- Brodsky RA et al. *Blood*. 2014;124:2804-11.
- Bektas M et al. *J Manag Care Spec Pharm*. 2020 Dec;26(12-b Suppl):S8-14.
- Food and Drug Administration (FDA). Soliris (eculizumab) package insert. Accessed on: September 23, 2024. Available at: https://www.accessdata.fda.gov/drugsatfda_docs/label/2020/125166s434lbl.pdf.
- European Medicines Agency. Soliris (eculizumab) Summary of product characteristics. Available at: https://www.ema.europa.eu/en/documents/product-information/soliris-epar-product-information_en.pdf.
- Food and Drug Administration (FDA). Ultomiris (ravulizumab) package insert. Accessed on: September 23, 2024. Available at: https://www.accessdata.fda.gov/drugsatfda_docs/label/2022/761108s021lbl.pdf.
- European Medicines Agency. Ultomiris (ravulizumab) Summary of product characteristics. Available at: https://www.ema.europa.eu/en/documents/product-information/ultomiris-epar-product-information_en.pdf.
- Food and Drug Administration (FDA). Fabhalta (iptacopan) package insert. Accessed on: September 23, 2024. Available at: https://www.accessdata.fda.gov/drugsatfda_docs/label/2024/218276s001lbl.pdf#page=19.
- European Medicines Agency. Fabhalta (iptacopan) Summary of product characteristics. Available at: https://www.ema.europa.eu/en/documents/product-information/fabhalta-epar-product-information_en.pdf.
- Peffault de Latour R et al. *N Engl J Med*. 2024 Mar 14;390(11):994-1008.
- Alava MH, et al. *Health Technol Assess*. 2020;24(34):1-68.

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