

Assessing the quality of hemophilia care: how suitable are patient-centered outcomes to compare hospital performance?

Diaz Prameyllawati^{1,2}, Samantha Gouw^{3,4}, Hester Lingsma¹, Marjon Cnossen⁵, Floor Heubel-Moenen^{6,7}, Frank Leebeek⁸, Nick van Es⁹, Johanna van der Bom⁴, Renske ten Ham², Martijn Oude Voshaar¹

BACKGROUND

Patient-centered outcomes provide valuable measures of treatment effectiveness in hemophilia trials, but their suitability for comparing hospital performance, a prospective route to improve quality of care, remains uncertain.

METHODS

- **Data:** 807 adults with hemophilia registered at six treatment centers (questionnaire-based study 2018-2019)
- **Evaluated outcome indicator:** five clinical and seven patient-reported outcomes
- **Evaluation criteria**
 - Validity → Spearman's rank correlation between case-mix adjusted and unadjusted hospital scores (*high correlations indicates minimal case-mix influence*)
 - Reliability → rankability percentage or proportion of between-hospital variation not attributable to chance (*higher rankability indicates greater reliability*)

AIM

To evaluate the suitability of patient-centered outcomes as quality indicators for comparing hospital performance

CONCLUSION

Clinical outcomes outperform patient-reported outcomes as quality indicators for hospital comparisons

RESULTS

Outcome indicator	Spearman's CC (95% CI)	Rankability (95% CI)
<i>Clinical outcomes</i>		
Zero-treated bleeds	0.89 (0.50-1.00)	0.16 (0.00-0.65)
Zero-treated joint bleeds	0.89 (0.40-1.00)	0.10 (0.00-0.65)
Annual bleeding rate	0.94 (0.40-1.00)	0.55 (0.00-0.96)
Annual joint bleeding rate	0.94 (0.50-1.00)	0.67 (0.00-0.90)
Joint score	0.77 (-0.50-1.00)	0.48 (0.00-0.82)
<i>Patient-reported outcomes</i>		
General physical functioning	0.03 (-1.00-1.00)	0.10 (0.00-0.56)
Hemophilia-specific physical functioning	0.37 (-0.80-1.00)	0.12 (0.00-0.56)
Social functioning	0.89 (0.00-1.00)	0.03 (0.00-0.44)
Pain	0.43 (-0.50-1.00)	0.17 (0.00-0.72)
Anxiety	0.94 (0.50-1.00)	0.00 (0.00-0.27)
Depression	0.94 (0.40-1.00)	0.00 (0.00-0.27)
Hemophilia-specific treatment satisfaction	0.77 (0.20-1.00)	0.31 (0.00-0.80)

Case mix: age, education, income, severity, inhibitor, hypertension, hypercholesterolemia, DM type 1 and 2, COPD, psychological complaints, hepatitis C

AFFILIATION

¹Department of Public Health, Erasmus MC; ²Department of Epidemiology and Health Economics, Julius Center for Health Sciences and Primary Care, University Medical Center Utrecht; ³Department of Pediatric Hematology, Amsterdam UMC; ⁴Department of Clinical Epidemiology, Leiden University Medical Center; ⁵Department of Pediatric Hematology and Oncology, Erasmus MC Sophia Children's Hospital; ⁶Department of Hematology, Maastricht University Medical Center; ⁷CARIM – School for Cardiovascular Disease, Maastricht University; ⁸Department of Hematology, Erasmus MC; ⁹Department of Vascular Medicine, Academic Medical Center Amsterdam

FURTHER INFORMATION

SYMPHONY consortium aims to orchestrate personalized treatment in patients with an inherited bleeding disorder. This research was funded by NWO-NWA consortium (NWA.1160.18.038; Principal Investigator: Prof. Marjon H. Cnossen; project manager: Dr. Simone H. Reitsma). More information: www.symphonyc consortium.nl.

✉ d.prameyllawati@erasmusmc.nl

in [linkedin.com/in/prameyllawati](https://www.linkedin.com/in/prameyllawati)