A Growing Trend: The Expanding Use of Patient-reported Outcome Measures (PROMs) in Clinical Trials (2008-2023)

Yu J[1], Kuharic M[1,2], Nazari J[1], Joshi M[1], Pickard AS[1]
[1] Department of Pharmacy Systems, Outcomes and Policy, College of Pharmacy, University of Illinois Chicago
[2] Department of Medical Social Medicine, Feinberg School of Medicine, Northwestern University

RETZKY COLLEGE OF PHARMACY
The Department of Pharmacy Systems, Outcomes and Policy

PCR23

INTRODUCTION

- Patient-reported outcome measures (PROMs) capture self-assessed health outcomes to inform treatment effectiveness, patient-centered care and decision-making (FDA, 2009).
- In 2008, the FDA introduced the requirement of registration of clinical trials on ClinicalTrials.gov.
- Among PROMs, preference-based measures (PBMs) represent a subset that convert health status into utility values based on population preferences, allowing for quality-adjusted life year (QALY) calculations in economic evaluations (Brazier J, 2010).
- However, the overall landscape of PROM use in trials is far broader, and the frequency and types of PROMs used across studies remain areas of active investigation (Kluetz PG, 2018; Schnipper LE, 2016).

OBJECTIVE

- To examine the trends in the use of PROMs in clinical trials from 2008 to 2023 and compare the uptake of PROMs across regions, disease states, and adult vs pediatric populations.

METHODS

- An iterative search was performed from 2008 to 2023 through the ClinicalTrials.gov registry to identify trials with at least one PROM, including outcome measures of interest and PBMs: EQ-5D-3L/-5L/-Y, SF-36/-12/-8/-6D, EORTC QLQ-C30/QLU-C10D, PROMIS, HUI2/HUI3, FACIT, PedsQL, CHU9D, PROMIS-Pediatric, and CHQ.
- An algorithm was developed using Python script in Jupyter Notebook to search ClincalTrials.gov stratified by year.
- Annual trends, regional patterns, and disease-specific usage were summarized using descriptive analyses.

RESULTS

- From 2008 to 2023, overall PROM usage in clinical trials increased from 12.7% (n=1,783) to 23.5% (n=7,808) in adult trials [Figure 1], and from 8.0% (n=231) to 17.7% (n=1,118) in pediatric trials [Figure 2].
- Among PBMs for adults, SF was the most commonly used measure in aggregate across years (n=8,872), but EQ-5D showed more rapid growth in recent years, surpassing SF as the most utilized generic PROM in 2017 [Figure III]. In 2023, EQ5D (n=1,023) and SF (n=781).
- For pediatric PROMs, PedsQL was most used (Total: n=1,055; 2023: n=128), while CHQ had the highest annual growth (62.45%) [Figure IV].
- PROMs were most commonly used in adult trials addressing pathological conditions (n=5,110), nervous system diseases (n=4,530), and neoplasms (n=3,717) [Figure V]. In pediatric trials, PROMs were most used in trials addressing nervous system diseases (n=331) followed by congenital abnormalities (n=247) [Figure VI].
- Regional trends over this period indicated a preference for SF/EQ-5D in European adult trials (n=3,334; n=4,041). In North American adult trials, SF measures dominated (n=3,599 trials), followed by EQ-5D (n=1,506 trials) and notably high implementation of PROMIS (464 trials) compared to other regions [Figure VII].

FIGURE I: Adult Clinical Trials Incorporating PROMs by Year (%)

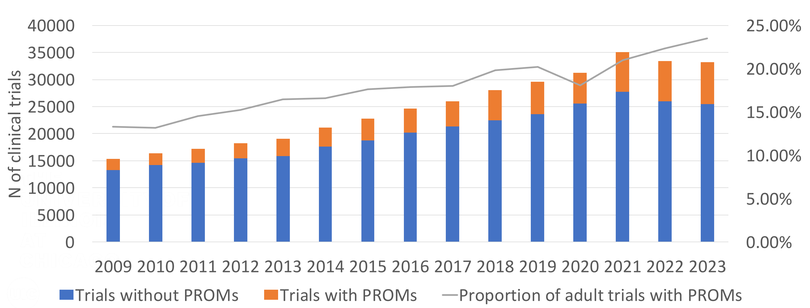


FIGURE II: Proportion of Pediatric Clinical Trials Incorporating PBM/PROMs by Year

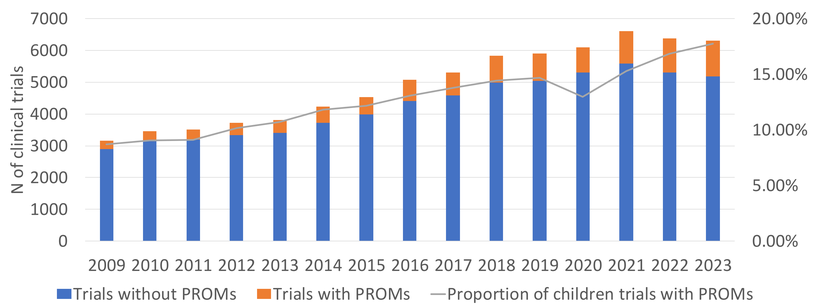


FIGURE III: Annual Trends of PBMs in Clinical Trials in the Adult Population (n)

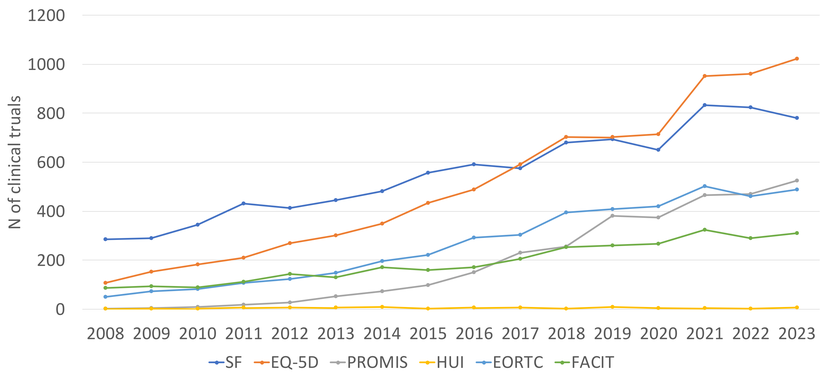


FIGURE IV: Annual Trends of PBMs in Clinical Trials in the Pediatric Population (n)

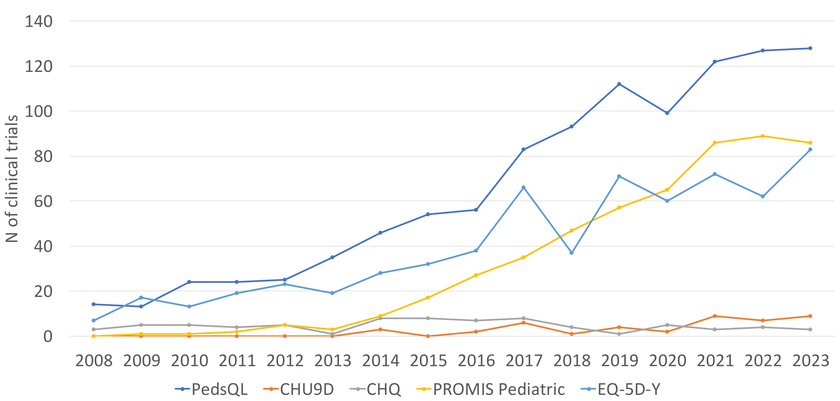


FIGURE V: Most Common Disease Areas Utilizing PROMs in Adults

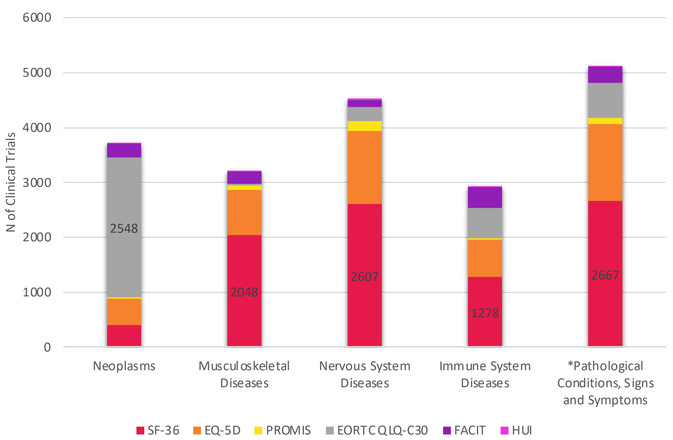


FIGURE VI: Most Common Disease Areas Utilizing PROMs in Adults

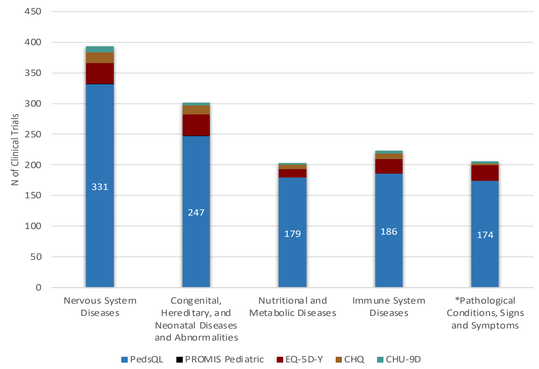
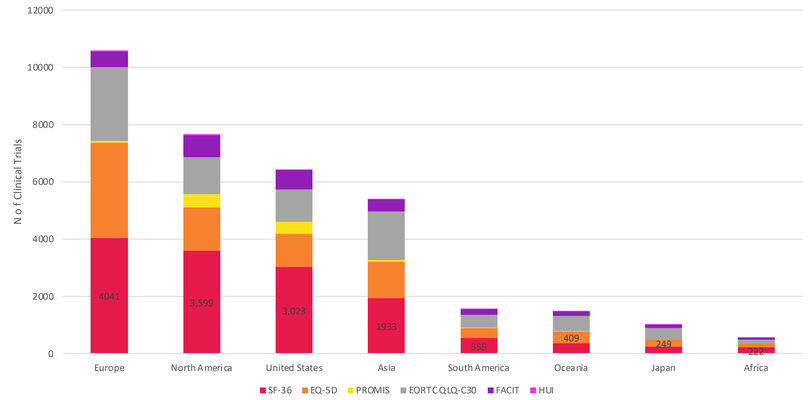


FIGURE VII: Geographic Distribution of PROM use in Adult Clinical Trials



DISCUSSION

- The use of PROMs in clinical trials for adult populations has grown steadily from 15% to 25% between 2008-2023, likely driven by guidance from both US and European agencies recommending PROMs to capture patient perspectives in drug development.
- Emergence of the EQ-5D becoming the most widely used measure beginning in 2018 may have been facilitated by widespread acceptance in pharmacoeconomic guidelines, ease of use, and accessibility (e.g. free for use) for academic and non-commercial research purposes.
- Geographic variations in PROM utilization highlight the influence of local regulatory frameworks, HTA guidance, and cultural and linguistic factors.
- Future efforts should focus on developing more standardized guidance, especially in pediatric populations.

CONCLUSION

- While the number of registered clinical trials has increased by 220%, the proportion of trials, including PROs, has approximately doubled.
- The emergence of PBMs as one of the more dominant types of PROMs being used in recent years may be a reflection of the importance of not just patient-centered outcome measures but also value based assessment/HTA.

REFERENCES

- U S. Food and Drug Administration. Guidance for Industry Patient Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims. Federal Register.2009;74(35):65132–3.
- Brazier, J., & Tsuchiya, A. (2010). Preference-based measures of health and their role in cost-effectiveness analysis. PharmacoEconomics, 28(8), 675-685.
- Kluetz PG, O'Connor DJ, Soltys K. Incorporating the patient experience into regulatory decision making in the USA, Europe, and Canada. Lancet Oncol. 2018;19(5):e267–e274.
- Schnipper LE, Davidson NE, Wollins DS, et al. Updating the American Society of Clinical Oncology Value Framework: Revisions and Reflections in Response to Comments Received. J Clin Oncol. 2016 08 20; 34(24):2925-34.
- Scoggins JF, Patrick DL. The use of patient-reported outcomes instruments in registered clinical trials: Evidence from ClinicalTrials.gov. Contemp Clin Trials. 2009; 30: 289-29214.

Acknowledgements: Justin Yu is supported by a Pfizer/UIC fellowship in HEOR (2024-26).