Suitability of cost-effectiveness thresholds for drug reimbursement decision-making in the US market

Ulrich Neumann[1], Michael Ciarametaro[2], Jordan Banks[2]

[1]Johnson & Johnson, Titusville, NJ, USA; [2]Avalere Health, Washington, DC, USA

Background

- In recent pharmaceutical policy discussions, proposals have emerged to incorporate cost-utility analysis or more generally threshold-based cost-effectiveness analysis (CEA), either directly or indirectly, as a basis for determining reimbursement in the United States (US).[1–5]
- At its core, a cost-utility analysis estimates the incremental cost per additional unit of health improvement for a given treatment and compares this value to a predetermined monetary threshold that purports to represent the maximum cost society is willing or able to pay (for one unit of health).[6] That benchmark, the threshold, is significant because it serves as the principal decision criterion for whether medicines should be funded or covered.[7]
- Calls for the adoption of threshold-based CEA risk concealing ongoing disagreements among economists about whether and how to establish thresholds, as well as practical challenges faced by decision-makers when applying them in Ex-US health systems.

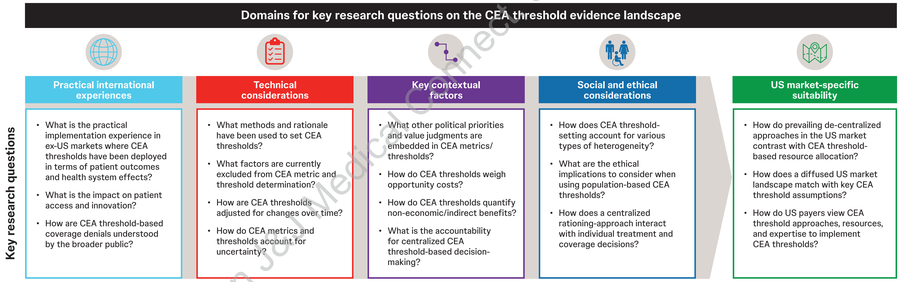
Research objective

- This qualitative mixed-method study explores the suitability of CEA thresholds to determine value in the US market setting—accounting for distinct economic, cultural, ethical, and policy complexities relevant to US stakeholders.

Methods

- To survey the evidence landscape on CEA thresholds, a study framework with five initial domains was developed: international implementation experiences, technical considerations, contextual factors, social and ethical dimensions, and suitability for the US market (**Figure 1**).
- A targeted literature review synthesized insights from peer-reviewed and scientific publications to address key research questions across the five domains (for search methodology and full references, see suppl. digital PDF).
- Ten semi-structured, double-blinded, in-depth interviews explored international applications and US-specific transferability.
  - Round 1: Five threshold experts expanded on literature findings and shared practical ex-US experiences (interviewees included two international economists and three former Health Technology Assessment [HTA] leaders from the UK, Canada, and Germany).
  - Round 2: Five experienced US stakeholders assessed relevance and transferability for US decision-making (interviewees included one former VP of Pharmacy/Health Insights at a large US payer; one former Chief Actuary at a national plan; one former VP at an employer benefits organization; one US healthcare economist; one healthcare ethics and justice expert).
- Insights from literature and interviews were systematically coded and thematically synthesized. Due to the study's exploratory design, results provide valuable qualitative insights into stakeholder sentiments but are naturally subject to potential bias and reflect expert opinion which has not been statistically quantified in this research.

Figure 1. Research domain framework



CEA, cost-effectiveness analysis; **US**, United States.

Key results

Methodological considerations around threshold-based CEA

- The pharmacoeconomic literature discusses various approaches to operationalizing thresholds, yet little consensus exists on how their values should be determined or how they should be used by decision-makers. As a result, thresholds diverge widely between different methods.[8–12]
- In most CEAs, thresholds benchmark cost per quality-adjusted life years (QALYs), a composite summary metric of health that the US National Disability Council found to be discriminatory, the US Congress banned for use in Medicare, and the US Center for Medicare and Medicaid Services disallowed from consideration for drug price regulations as part of the 2022 Inflation Reduction Act.[13–15]
- Beyond concerns of discrimination, studies highlight a range of conceptual and technical limitations with properly estimating QALYs.[16–19]
- Systematic comparisons suggest that the quality and quantity of empirical data on a country's willingness-to-pay per QALY, opportunity costs, and implicit threshold assumptions are poor, considering the importance of managing healthcare investment appropriately.[20]
- Some scholars propose methodological changes, such as disease-adjusted threshold values, reduced reliance on composite metrics in favor of condition-sensitive clinical measures, and the inclusion of broader conceptions of value.[21–23]
- Others continue to defend QALYs, argue that replacements complicate finding empirical bases for decision-making or contend that threshold adjustment would deviate from foundational CEA principles of standardizing assessments and maximizing aggregate health.[24,25]

Key learnings from international experiences with CEA thresholds

- The literature and interviewed HTA experts with experience in implementing thresholds expressed skepticism about their effectiveness in driving value-for-money resource allocation, while highlighting concerns about negative effects on market competition and incentives for innovation.[26–28]
- Mirroring divergences in theory, practical experiences with thresholds show considerable variability—ranging from rejection in some countries to adoption of multiple arbitrarily set threshold values, use of bypass mechanisms, or contextual adjustments to ease implementation.[27,29]
- Contrary to CEA theory, experience in ex-US countries suggests that pharmaceutical budgets are not statically fixed and thresholds are neither required nor sufficient to make efficient resource decisions.[30]
- Interviewees confirmed literature findings that decisions based on thresholds assuming uniform health benefits for all patients are ethically fraught and empirically flawed, ignoring various sources of heterogeneity across the population.[31,32]
- Research indicates that patients in countries where HTA relies on thresholds have experienced reduced access to treatments compared to those in countries that do not rely on them.[33,34]

Applicability and feasibility in the US market context

- Interviewees found it challenging to reconcile foundational principles of threshold-based CEA with cultural values in the US, where skepticism toward centralized decision-making in healthcare and a strong emphasis on individual patient empowerment prevail.
- Factors cited for the rejection of threshold-based approaches included: diverse population needs, multiple decision levels, flexibility to prioritize local preferences, non-transferable calculations and incompatibility with payer metrics and management, data limitations, and issues with static (fixed) outputs that ignore evolving market dynamics.
- Interviewees stated that US payers prefer flexible, localized agency over the use of explicit, predetermined thresholds.
- Payer representatives unanimously expressed that everyone should have equal access to necessary care and rejected prioritizing reimbursement based on calculating beneficiaries' relative capacity to benefit from a new treatment (particularly when equations are influenced by prior health status, such as is required by metrics like QALYs).
- Concerns were raised about transparency and accountability of deploying mathematical formulae to determine reimbursement or to ration access within Pharmacy and Therapeutics (P&T) and value committees.

Discussion

- Conceptual and empirical tensions surrounding the setting and application of thresholds reflect a vibrant academic debate. Unresolved complexities likely contribute to hesitancy among US stakeholders tasked with making real-world decisions to view CEA thresholds as 'fit for purpose'.
- Economic evidence indicates that local production functions across the market-driven US system vary widely due to differing payer populations and priorities.[35] Research participants suggest substantial differences between the objective functions of different US payers and fundamental assumptions underlying CEA approaches based on QALY-maximization (e.g., uniform valuation of health gains, fixed budgets, centralized allocation, and health priorities).
- This research suggests that framing healthcare decisions through the lens of societal resource scarcity, assigning a monetary value to human life, and explicit and centralized rationing of care based on standardized formulae, all conflict with prevailing moral intuitions in the US and its preferences for individual choice, autonomy, and personal freedom.
- Although economic evaluations can provide valuable information for guiding efficient resource allocation, decision-making in US healthcare incorporates broader societal values, including promoting patient-centered care, individualized treatment, and fostering innovation.[35,36]

Poster Number 133

Conclusions

Various efforts have been made in recent years to directly or indirectly promote more prominent use of threshold-based CEA in the US.

US policy discourse has mostly focused on criticisms of QALYs, putting comparatively less emphasis on the role of CEA thresholds as a determinant of reimbursement.

Participants in this study highlighted a dissonance between threshold-based CEA and the realities of the decentralized US healthcare system, where value and price are shaped by the dynamic interactions of various independent actors and market forces.[37]

Some economists suggest that a rejection of threshold-based CEA is warranted in the US as private payers' adoption of reimbursement based on CEA criteria could result in both static and dynamic inefficiencies.[38]

We conclude that beyond cultural and normative considerations, the decentralized and dispersed nature of the market-driven US healthcare system is distinct enough to warrant appropriately tailored economic approaches to value and pricing, rather than importing mechanistic 'one-size-fits-all' equations.

Acknowledgments

We thank Brooke Fruman and Rosalie Hoyle at Avalere Health for supporting the project management of this research.

Disclosures

UN is an employee of Johnson & Johnson and holds shares in the company. **MC** and **JB** are employees of Avalere Health.

Funding

This study was funded by Johnson & Johnson. Editorial and design support was provided by Twist Medical and was funded by Johnson & Johnson.

Presented at: Academy of Managed Care Pharmacy Nexus Annual Meeting; National Harbor, MD, USA; October 27–30, 2025

REFERENCES:

1. Sullivan SD, et al. *J Manag Care Spec Pharm.* 2015;21:269–75. **2.** ICER. Who we are: History & impact. Available at: https://icer.org/who-we-are/history-impact/ (accessed April 28, 2025). **3.** Gould J. CVS Shifts to ICER Value-Based Drug Pricing Strategy. First Report Managed Care; September 1, 2008. Available at: https://www.hmpgloballearningnetwork.com/site/frmc/article/cvs-shifts-icer-value-based-drug-pricing-strategy (accessed April 28, 2025). **4.** Thokala P, et al. *J Manag Care Spec Pharm.* 2020;26:1162–70. **5.** Usdin S. ICER shows where IRA could be heading. BioCentury; October 2, 2023. Available at: https://www.biocentury.com/article/649386/icer-shows-where-ira-could-be-heading (accessed April 24, 2025). **6.** Bertram MY, et al. *Bull World Health Organ.* 2016;94:925–30. **7.** Berdud M, et al. *Front Health Serv.* 2023;3:1055471. **8.** Thokala P, et al. *Pharmacoeconomics.* 2018;36:509–22. **9.** Cubi-Molla P, et al. Are cost-effectiveness thresholds fit for purpose for real-world decision making? OHE Contract Research; 2020. Available at: https://www.ohe.org/publications/are-cost-effectiveness-thresholds-fit-for-purpose/ (accessed April 28, 2025). **10.** Sampson C, et al. *Appl Health Econ Health Policy.* 2022;20:651–67. **11.** Schwarzer R, et al. *J Comp Eff Res.* 2015;4:485–504. **12.** Ryen L and Svensson M. *Health Econ.* 2015;24:1289–1301. **13.** National Council on Disability (NCD). Quality-adjusted life years and the devaluation of life with disability; 2019. Available at: https://www.ncd.gov/assets/uploads/reports/2019/ncd_quality_adjusted_life_report_508.pdf (accessed April 28, 2025). **14.** Patient Protection and Affordable Care Act, 42 U.S.C. § 1320e-1(e); 2010. Available at: https://www.congress.gov/bill/111th-congress/house-bill/3590 (accessed April 28, 2025). **15.** Centers for Medicare & Medicaid Services. Medicare drug price negotiation program: Initial guidance; March 2023. Available at: https://www.cms.gov/files/document/medicare-drug-price-negotiation-program-initial-guidance.pdf (accessed April 28, 2025). **16.** Browne J, et al. Is the QALY fit for purpose? *Am J Manag Care*; July 14, 2021. Available at: https://www.ajmc.com/view/is-the-qaly-fit-for-purpose- (accessed April 28, 2025). **17.** Schlander M, et al. *J Comp Eff Res.* 2018;3:389–422. **18.** Pettitt DA. *J Stem Cell Res Ther.* 2016;6:1–7. **19.** Berezniak A, et al. *PharmacoEconomics.* 2015;33:61–9. **20.** Cameron D, et al. *Glob Health Action.* 2018;11:1447828. **21.** Lakdawalla DN and Phelps CE. *Eur J Health Econ.* 2022;23:433–51. **22.** Brazier JE, et al. *Measuring and Valuing Health Benefits for Economic Evaluation.* Oxford University Press; 2017. **23.** Shafrin J, et al. *Forum Health Econ Policy.* 2024;27:29–116. **24.** Cohen JT. The much-maligned 'quality-adjusted life year' is a vital tool for health care policy. STAT; March 22, 2023. Available at: https://www.statnews.com/2023/03/22/the-much-maligned-quality-adjusted-life-year-is-a-vital-tool-for-health-care-policy (accessed April 24, 2025). **25.** Caro JJ, et al. *Pharmacoeconomics.* 2019;37:293–9. **26.** Brekke KR, et al. *J Health Econ.* 2023;90:102778. **27.** Luyten J and Denier Y. *Soc Justice Res.* 2019;32:155–71. **28.** Moradpour J and Hollis A. *Health Econ.* 2021;30:1139–51. **29.** Seixas BV, et al. *BMC Health Serv Res.* 2019;19:560. **30.** Vallejo-Torres L, et al. *Value Health.* 2016;19:558–66. **31.** Willis MS, et al. *Pharmacoeconomics* 2025 Mar 8. doi: 10.1007/s40273-025-01478-z. **32.** Marseille E and Kahn JG. *Philos Ethics Humanit Med.* 2019;14:1–7. **33.** Sehdev SR, et al. *Curr Oncol.* 2024;31:1803–16. **34.** Pharmaceutical Research and Manufacturers of America (PhRMA). Global access to new medicines report; April 2023. Available at: https://cdn.agilty.io/phrma/global/resources/import/pdfs/2023-04-20%20PhRMA%20Global%20Access%20to%20New%20Medicines%20Report%20FINAL-1.pdf (accessed April 28, 2025). **35.** Neumann PJ, et al. Cost-Effectiveness in Health and Medicine. 2nd ed. New York, NY: Oxford University Press; 2017:4. **36.** Neumann PJ, et al. The Right Price. Oxford University Press; 2021:95–110. **37.** Pauly MV. *Value Health.* 2017;20:278–82. **38.** Philipson T. *Nord J Health Econ.* 2017;1:58–61.