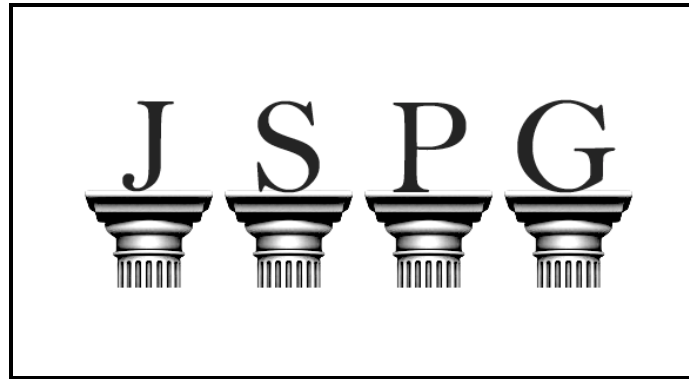


# **The Journal of Science Policy & Governance**



**OP-ED:  
IN MEDICINE, WE NEED BETTER DATA**

BY

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When a loved one's life is at risk, we all want the best possible healthcare. Yet, in many cases there isn't enough evidence for physicians to know whether one treatment is better than its alternative. To improve healthcare in the United States, we need to collect data about what works best for which patients and under which circumstances. We need data on the comparative effectiveness of available healthcare interventions. But these data may not be available because the mere collection and analysis of such data has come under political attack.

Researchers use comparative effectiveness research (CER) to compare the clinical effectiveness and risks of two medical treatments or tests that can be used for the same condition. CER results enable physicians, insurers, and policy makers to improve health services for individual patients and for the population. To advance health care in the U.S., it is necessary to garner support for CER from the medical community and the public, to prioritize the funding of well-designed CER studies, and to improve dissemination of CER results, despite the obstacles that stand in the way of doing so.

The reasoning behind CER is not new – physicians have compared medical treatments for centuries – but fears remain prevalent that CER could lead to patients losing access to beneficial services. Over 80% of respondents in a 2009 survey thought that guidelines based on CER could prevent physicians from providing individualized care. Over 70% thought that the guidelines would be vulnerable to corruption and abuse, could lead to rationing, and would not necessarily incorporate the latest scientific breakthroughs. Still, over half of respondents were convinced that the guidelines would improve care for most patients.<sup>1</sup> In 2010, amidst controversy about CER, the Affordable Care Act (ACA) established the Patient-Centered Outcomes Research Institute (PCORI) to fund and conduct CER studies.

Opponents of CER often link it to cost-effectiveness analysis (CEA) and rationing of care. They fear that the U.S. will take after the United Kingdom, where cost information is factored into whether

the government will pay for particular treatments.<sup>2</sup> In traditional CEA, health benefits are generally measured by increases in quality-adjusted life-years (QALYs) from an intervention. QALYs are a controversial measure of life: in addition to survival, they also include subjective factors such as quality of life, pain, illness, and disability.<sup>2</sup> As a result of the controversy, the PCORI in the U.S. is banned from using measures like dollars per life-year gained per treatment in recommendations that affect coverage of health care services, reimbursement, or incentive programs.<sup>2</sup>

PCORI researchers need to collect data on cost if we want to reduce wasteful spending. Even though the institute itself cannot make care recommendations based on cost to Medicare or Medicaid, the information they provide could lead to better decision-making by private insurers, who largely fund the institute through a tax.<sup>2</sup> Cost cutting is necessary to prevent escalating health care spending in the U.S.; reasonable guidelines based on CER are an antidote to across-the-board spending cuts.

Physicians who object to CER contend that recommendations based on CER “could miss the idiosyncratic ways in which a disease—or a treatment—may operate in particular patients.”<sup>3</sup> However, the opposite is actually true. Isolating how an intervention affects specific groups in a CER study further the pursuit of personalized, evidence-based medicine. Large, diverse CER studies are necessary to isolate patient-specific characteristics, such as age, sex, comorbidities, and genetics, which could influence the effect of the intervention.<sup>2</sup> CER can be used to evaluate when personalized medicine improves health outcomes to justify the costs.<sup>4</sup>

Practices in the clinic often lag behind the results of CER studies.<sup>3</sup> While researchers carefully develop and implement clinical trials, many fail to develop plans to disseminate results.<sup>3</sup> Funding implementation science is necessary to ensure that the results of CER studies reach patients. To encourage prompt sharing of results, a provision in the ACA requires that the results from all federally funded CER studies be made available in a database within 90 days of completion.<sup>5</sup>

Public and private interests do not always match. Sometimes, CER results will be contrary to the bottom lines of pharmaceutical companies. For instance, results of the ALLHAT (Antihypertensive and Lipid-Lowering Treatment to Prevent Heart Attack Trial) showed that older and cheaper thiazide-type diuretic drugs were just as effective as their newer, more expensive competitors. However, this comprehensive clinical trial did not greatly change prescribing patterns because of the lack of a dissemination plan, counter-marketing by drug companies, and some criticism by cardiovascular physicians considered to be hypertension experts in the medical community. The National Heart, Lung, and Blood Institute funded an implementation program to spread results of the study, but the program had limited reach.<sup>3</sup> To date, private industry has generally been more effective at pushing brand name drugs than the public sector has been at communicating CER results.<sup>3</sup> One potential solution to this issue is academic detailing: a practice in which trained personnel travel to physicians' offices to discuss CER results, similar to how drug representatives advocate for their products.

Physicians are also likely to ignore CER results if they choose to purchase and use an expensive new technology associated with higher reimbursement rates. Affordable Care Organizations (ACO) could change physicians' incentives by paying them based on the extent to which their clinical decisions match care suggested by CER. Certainly, there will be exceptions based on individual patients, and physicians should not be punished for those discrepancies. But in theory, general patterns of care ought to match the best practices supported by CER. The incentives need to be designed so that physicians make the most appropriate clinical decisions – not the least expensive ones.<sup>3</sup>

The goal of CER is simple: to help doctors choose the best medical care for their patients. Policy makers can help researchers with this goal by allocating adequate funds to CER and the implementation of its findings. While it is politically unfavorable, new legislation that better enables Medicare and Medicaid to use CER in making coverage decisions ought to be considered. Paying for

better and less expensive medications benefits both patients and taxpayers. Effective medicine that doesn't break the bank must transition from dream to reality to keep U.S. health care sustainable.

## References

1. Gerber, A. S., Patashnik, E. M., Doherty, D. & Dowling, C. A National Survey Reveals Public Skepticism About Research-Based Treatment Guidelines. *Health Affairs* (2010).
2. Garber, A. M. & Sox, H. C. The role of costs in comparative effectiveness research. *Health Aff (Millwood)* **29**, 1805–1811 (2010).
3. Avorn, J. & Fischer, M. 'Bench To Behavior': Translating Comparative Effectiveness Research Into Improved Clinical Practice. *Health Aff (Millwood)* **29**, 1891–1900 (2010).
4. Epstein, R. & Teagarden, J. R. Comparative Effectiveness And Personalized Medicine: Evolving Together Or Apart? *Health Aff (Millwood)* **29**, 1783–1787 (2010).
5. Benner, J. S., Morrison, M. R., Karnes, E. K., Kocot, S. L. & McClellan, M. An evaluation of recent federal spending on comparative effectiveness research: priorities, gaps, and next steps. *Health Aff (Millwood)* **29**, 1768–1776 (2010).

## Bio

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