

# Comparative Effectiveness Research

## Valuable Insight or Government Intrusion?

F. Randy Vogenberg, RPh, PhD

Is the H1N1 influenza vaccine really safe? Do COX-2 inhibitors as a class actually raise the risk of heart attacks? Can prasugrel (Efient, Lilly) be used in place of clopidogrel bisulfate (Plavix, Bristol-Myers Squibb) in 2011? Conflicting studies, complicated science, and limited funding have created either a perfect opportunity or a perfect storm for comparative effectiveness research (CER).

### THE PROMISE

One way or another, health care reform will have an effect on our nation's health care delivery system starting sometime in 2010 and continuing throughout the next decade. The fits and starts of widespread reform have been positioned more narrowly for now, as health insurance reform moves through the Congress' legislative process in Washington, DC. Around the same time that health reform efforts began, the American Recovery and Reinvestment Act of 2009 (ARRA) initiated a process to develop CER through a federal board of experts.

CER is a relatively new term in U.S. policy discourse, but many other countries have embraced the concept for years.<sup>1</sup> Health technology assessment, as it is sometimes referred to, has played a central, if not transformative, role in contributing to evidence-based decision making and in identifying interventions that provide the most value for the money.<sup>1</sup> Basically, the goal of CER in the U.S. is to target spending of health care dollars on medical treatments that have proved to be effective in defined real-world populations. The development of CER can be accomplished through a variety of approaches such as randomized trials, systematic reviews, database analyses, and prospective observational studies. In an interview published in May 2009, President Obama saw this effort as "government being an honest broker in assessing and evaluating treatment options."<sup>2</sup>

This initial CER effort has held the promise of assisting patients, clinicians, purchasers, and policymakers in making informed decisions that will improve health care for both individuals and the population at large.<sup>3</sup> That objective is a tall order for an initiative by any organization, let alone the federal government, what with the imminence of systematic health care reform. Because of all the various stakeholders and their individual and competing interests, a committee convened by the Institute of Medicine (IOM) created a list of 100 recommended economic priorities that include a broad range of treatments, procedures, and tests. These priorities would

target atrial fibrillation, hearing loss, dementia, prostate cancer, and dental caries, among others. However, it may prove difficult for the promise of CER to provide real-world evidence of the superiority of one treatment over another.<sup>2</sup>

The responsibility of P&T committee members in all health care settings is to ensure the safety and health of patients for whom their organization is accountable under its mission and scope. Therefore, the promise of CER lies in its potential to aid all types of organizations that have a P&T committee to be of greater assistance than is currently the case.



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### THE REALITY

The rapid adoption of new technologies, including a robust specialty medication and testing pipeline, will continue to place P&T committees on the proverbial hot seat for decision making. So-called messenger products, such as interferons, proton pump inhibitors, and anti-tumor necrosis factor agents, offer a glimpse of possible early priorities for stakeholders in CER. Some health plans and prescription benefit managers (PBMs) are already exploring the interchangeability, substitution, and off-label uses for products, just as hospitals have been doing for more than 40 years. These products include anticoagulating drugs, single and combination antihypertensive medications, and diuretics.

As an early advocate for CER and better intra-agency collaborations to manage clinical and economic issues, Dr. Mark McClellan continues as a provocateur apart from his former roles as Commissioner of the FDA and Commissioner of the Centers for Medicare and Medicaid Services (CMS). He is currently Director of the Engelberg Center for Health Care Reform, a Senior Fellow in Economic Studies, and the Leonard D. Schaeffer Director's Chair in Health Policy Studies at the Brookings Institution in Washington, DC. Nonetheless, little has changed within the several federal health care agencies (the National Institutes of Health, the FDA, the Centers for Disease Control and Prevention, and the Agency for Healthcare Research and Quality), which have their own internal turf and funding battles to wage amidst the larger political wars being fought in the Potomac basin.

Yet all agree on the need for evidence and the dissemination of knowledge through various methods, including expert panels. In many ways, however, the limits of today's science and technology leave us to making well-reasoned (one hopes) gut decisions that may be vindicated in later analyses of outcomes—decisions such as making dietary adjustments to reduce cardiovascular disease and discontinuing estrogen replacement therapy that might have led to breast cancer.<sup>4</sup> That begs the question of to what extent a treatment approach can rely on the medical literature or medical experts. A similar debate took place during the early years of the HIV/AIDS

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epidemic in the U.S. when scientists were confronted with the juxtaposition of double-blind, randomized studies from the medical community versus open-label studies and registries by AIDS activists.

Susan Gilbert, a staff writer for The Hastings Center, argues that collecting the evidence is more complicated than it looks.<sup>5</sup> The “great hope” of CER, she explains, is that it will supply some of the missing science.

Will future academic practitioners or federal agencies be able to adequately apply community standards of practice and to determine the appropriateness of clinical care delivered to individuals or large groups of patients, as the promise of CER suggests? Is CER about only money and the economics of health care?

In looking at our history since 1965, with the start of large social health care programs such as Medicare and Medicaid, government program cost overruns have been a constant reality. During our recent history, economists’ and actuaries’ vastly underestimated budget projections for federally funded health care services programs, combined with congressional efforts to avoid the pain and stigma of short-term debt, have inadvertently created a steadily increasing mountain of debt that will likely not be paid for years to come.<sup>6</sup>

Will the reality of CER, therefore, focus more on economic benefits than on clinical outcome improvements, in an effort to generate program savings sooner rather than later? If so, how will this new economic focus affect P&T committee decision making in various organizations?

We must consider another aspect of CER that has been a cornerstone of value for P&T committees, namely, assessing new technologies for their safety, effectiveness, and potential ability to improve patient outcomes. Innovation (or the lack thereof) in developing medications and new devices will have a great impact on all stakeholders who make decisions in health care. CER does offer some promise for fostering the development of new products and information over the long term, but it could also dampen enthusiasm for investment in existing and potential treatments that are the lifeblood of health care outcomes research. This could happen because of an inability to demonstrate conclusive results, in a timely manner, that are required for investing in medical devices or because of a failure to conduct appropriate comparisons of emerging research compounds versus established pharmaceutical brands.

Some critics warn that government bodies could use CER to dictate “appropriate” care and to impose third-party oversight that would be so intrusive that it would interfere with the interaction between doctors and patients. Other experts disagree, however. Pauline Chen, a surgeon and another Hastings Center author, says that CER has the potential to strengthen the doctor–patient relationship by keeping us from being blindsided by our own best intentions.<sup>7</sup>

### CONCLUSION

During this next decade, we will continue to grapple with the reality versus the promise of CER for P&T committees in health plans, hospitals, and other institutions. With every new CER study result released by organizations in both public and private sectors, conflicts between the promise and the reality

become more apparent and increasingly difficult for P&T committees to reconcile. In these changing times, it may be useful to discuss with your colleagues how you and your P&T committee might benefit from CER.

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## EDITORIAL

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CU concludes:

As the nation begins to reform our health care system, we have an opportunity to take effective and accountable action to make health care safer for all Americans. The time to act is now. We cannot wait another decade.

To this I would only add, Amen.

As always, I am interested in your views. You can reach me at [david.nash@jefferson.edu](mailto:david.nash@jefferson.edu). Please visit my blog at <http://nashhealthpolicy.blogspot.com>.

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