

Introduction

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In 2009, the Institute of Medicine (IOM) defined comparative effectiveness research (CER) as “the generation and synthesis of evidence that compares the benefits and harms of alternative methods to prevent, diagnose, treat, and monitor a clinical condition or to improve the delivery of care.”¹ CER isn’t restricted to just comparing drug A versus drug B—it’s much broader than that. CER includes measuring interventions, approaches to care, and the delivery of care. The IOM also stated that “the purpose of CER is to assist consumers, clinicians, purchasers, and policy makers to make informed decisions that will improve health care at both the individual and population levels.”¹ The IOM clearly identifies these 4 different stakeholders (consumers, clinicians, purchasers, and policy makers) for CER, and they look at the impact both on the individual and across populations.

But the IOM isn’t the only organization defining CER. The Federal Coordinating Council for CER, established by the American Recovery and Reinvestment Act (ARRA) of 2009 to coordinate CER efforts, defines CER similarly, although it doesn’t specifically mention delivery of care or overall populations, and it lists only 3 stakeholders: patients, providers, and decision makers (although we can probably assume that “decision makers” includes both purchasers and policy makers).² These are subtle differences, perhaps, but interesting to note. The related discussions around definitions of stakeholders have also contributed to ongoing political disagreements as to who is the audience of CER, and therefore who should fund it.³

It is important to distinguish CER from patient-centered outcomes research. The Patient-Centered Outcomes Research Institute (PCORI), which replaced the Federal Coordinating Council in 2010, states that it will “commission research that is responsive to the values and interests of patients and will provide patients and their caregivers with reliable, evidence-based information for the health care choices they face.” In addition, the PCORI says it is committed to a “rigorous stakeholder-driven process that emphasizes patient engagement.”⁴ So the research direction of PCORI appears to be more patient-oriented than either the IOM or Federal Coordinating Council definitions. However, beginning in 2013, funding for PCORI will begin to include \$2-per-patient annual transfers from the Medicare Trust Fund and from private health plans.⁵ With these major stakeholders (health plans) contributing so substantially to the funding, will the work that PCORI produces actually assist these health plans in making the informed decisions that they need?

All of these definitions of CER, along with their variations, slight or otherwise, affect many different stakeholder groups, who all have different needs and expectations. As the U.S. health care system evolves, each of these stakeholders will have to evaluate what CER means to them, how they can

integrate CER to their advantage, how they can ensure that the CER being conducted is relevant to their needs, how they can address some of CER’s limitations⁶ and how they or their organizations will need to transform to more effectively use that data. Stakeholders will also need to evaluate how conducting and evaluating CER will fit with current Food and Drug Administration (FDA) regulations and guidance.⁶ And methodologies and infrastructures for conducting effective and meaningful CER must be systematically assessed and enhanced, and such efforts will require considerable dialogue before new “best practices” can be established.⁷ A 2010 article by Tunis discusses various aspects of CER infrastructure that will need to be addressed to assure such research will be designed, conducted and communicated with the greatest benefit to stakeholders.⁷

The gaps in knowledge left by randomized control trials are an ongoing source of concern for physicians, patients, policy makers, and payers. Without information about how well a new medication works when compared to other existing alternatives, in a real world setting, stakeholders are sometimes left with a “trial and error” approach to decision making, which can result in expensive lessons. CER can, if effectively designed and conducted, help fill some of those gaps. Of course, the key word is “effectively.” While CER itself is not new, the rapidly increasing demands of and expectations for CER will bring a host of implementation questions and issues. Integration of CER will not be simple or seamless, but the more dialogue we encourage now to identify and address some of those issues, the more effective the research will be. For example, in a 2010 commentary, Rubin warns against what he perceives as a tendency for researchers to use inadequate data sets for CER studies and recommends some ways to evaluate and select more relevant data sets.⁸ In a 2011 article, Alemayehu and Cappelleri point out some of the historical weaknesses in conducting and reporting observational and non-randomized studies, including sources of bias, and recommend steps to minimize bias in the design, analysis and reporting stages of a study.⁹

Without a doubt, the increase in CER will change the health care industry in nearly every area, from development costs to formulary decision making, from treatment decisions to product innovation. A key question for payers will be that of value. How does each stakeholder define the value of CER? How do they prioritize to get the most value from that research? It’s recognized that the higher-quality, higher-priced products may in fact result in lower costs over time. But there must be evidence that demonstrates this for the purchaser.¹⁰ And we must define parameters to measure success.

A global overview of CER was presented in the October 2010 issue of *Health Affairs* that complements many of the perspectives of the current report. In that issue, for example, Etheredge outlines the need for a high-performing CER system

and a national database of CER studies.¹¹ Robinson proposes how public and private insurers may adapt their policies to incorporate CER.¹² Pearson and Bach propose a payment model for Medicare that uses CER to encourage Medicare to pay equally for comparable medicines.¹³ And Chokshi et al. offer suggestions for increasing the clinical applicability for CER.¹⁴

For this supplement to the *Journal of Managed Care Pharmacy*, 3 opinion leaders contribute to the necessary and ongoing dialogue about CER by offering their perspectives on how health care reform in general, and CER specifically, may affect their areas. Penny Mohr, Vice President of Program Development at the Center for Medical Technology Policy, writes about ways she sees CER affecting the decisions the Centers for Medicaid and Medicare Services make for their growing population. Dr. Robert W. Dubois, Chief Science Officer at the National Pharmaceutical Council, addresses CER's possible impacts—both positive and negative—on the pharmaceutical industry. And, Dr. H. Eric Cannon, Chief of Pharmacy at SelectHealth, explores how managed care organizations have used CER in the past and some of the hard questions those organizations will need to answer in the future.

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