

Health Policy

NEWSLETTER

FROM THE EDITOR

Dare to Compare

Introduction

As part of the American Recovery and Reinvestment Act (ARRA), Congress mandated that the Institute of Medicine (IOM) establish a list of Comparative Effectiveness Research (CER) priorities by June 30, 2009. ARRA authorized a \$1.1 billion down payment to support national CER efforts. Of the total funds, \$400 million is to be released by the Secretary of Health and Human Services, and is likely to be targeted towards topics consistent with the IOM list. Another \$400 million is to be released by the National Institutes of Health (NIH), and the remaining \$300 million is to be dispersed by Agency for Healthcare Research and Quality (AHRQ). At the time of this writing, there were two Congressional proposals to sustain national CER efforts. In a recent interview about health reform, President Obama supported CER in saying "There's always going to be an asymmetry of information between patient and provider. Part of what I think government can do is to be an honest broker in assessing and evaluating treatment options."¹

What is Comparative Effectiveness Research?

The IOM Committee defined 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. 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."^{2,3}

The report states six characteristics of CER:

1. CER has the objective of directly informing a specific clinical decision from the patient perspective or a health policy decision from the population perspective.
2. CER compares at least two alternative interventions, each with the potential to be "best practice."
3. CER describes results at the population and subgroup levels.

4. CER measures outcomes - both benefits and harms - that are important to patients.

5. CER employs methods and data sources appropriate for the decision of interest.

6. CER is conducted in settings that are similar to those in which the intervention will be used in practice.

The premise of CER is simple: we should invest in the medical treatments that are proven to be effective in defined patient populations in real-world practice settings. CER can be conducted using a variety of approaches, including randomized trials, prospective observational studies, database analyses, and systematic reviews - all methods of population health research. CER is conducted in settings that are similar to those in which the intervention will be used in practice.

The IOM Committee created a list of 100 recommended priorities, through a structured review of potential topics identified through a national survey. The full list is available at: www.iom.edu/cerpriorities. Priorities in the top quartile include comparing the effectiveness of treatment strategies for: atrial fibrillation; hearing loss; dementia; prostate cancer; dental caries; ADHD and obesity in children; prevention of falls in older adults; chronic care management programs; biologics for inflammatory diseases; screening, prophylaxis and treatment programs for methicillin resistant staphylococcus aureus (MRSA) and healthcare acquired infection; and genetic and biomarker testing for certain cancers. A broad array of interventions was recommended to evaluate these priorities, including systems of care; pharmacological treatment; behavioral treatment; prevention; procedures; testing, monitoring, and evaluation; devices; standard of care; alternative treatment; provider-patient relationships; and treatment pathways.

CER provides clinicians and health plans with the ability to compare treatments to each other (or to usual care) rather than to placebo, and to understand the effectiveness of treatments in defined populations. Though manufacturers will continue

placebo-controlled trials in order to meet FDA requirements, CER will provide real-world evidence on competing treatments via head to head trials, observational studies, and database analyses (for example, patient registries or claims datasets). CER will also elucidate the effectiveness of treatments in groups typically underrepresented in clinical trials, such as children, the elderly, and minority groups.

Role of Economic Analysis in CER

Applied health economic analysis is an important component of CER because it reveals which treatments yield maximal value. Applied health economics involves weighing effectiveness and costs of competing treatment interventions, typically via formal cost effectiveness analyses. First published nearly two decades ago, best practices for cost effectiveness analysis have stood the test of time - with a significant increase in published studies in recent years.⁴ Opponents to including cost in CER fear that it may impede patients' access to expensive care; however, cost effectiveness analysis often recommends the use of more expensive treatments if they produce better outcomes. Thus, cost effectiveness does not necessarily translate to cost savings, but may instead mean better results for the dollars spent. This type of analysis becomes increasingly important when competing treatments are equally effective, or have marginal differences in effectiveness.⁵

Jefferson School of Population Health: Committed to Developing the CER Workforce

The IOM Committee report noted that the career pathways for CER are not clear, and there is a lack of federally funded graduate and post-graduate training programs aimed at grooming investigators in population health research. The committee predicted a "substantial need" for experts in the disciplines of CER, including outcomes research, observational data analysis, cost effectiveness, statistical modeling, and epidemiology.²

The Jefferson School of Population Health anticipates this growing national need for CER researchers. Through our existing two-year postdoctoral fellowships in applied health economics and outcomes research, JSPH has trained more than 30 professionals in the methods of CER during the past

15 years. This past year, we doubled the number of available fellowship slots from 2 to 4. Moving ahead, we are committed to further building the CER workforce with graduate-level degrees centered on CER methods, particularly a Master of Science degree in Applied Health Economics (presently in development). This degree will focus on the methods of cost effectiveness analysis, observational studies, health utility and quality of life outcomes research, and economic modeling. It will be the first in the US to emphasize important population

health interventions such as screening programs, vaccinations, occupational and physical therapy, surgical techniques, dietary modification and exercise regimens. We believe that the CER workforce of the future will be called upon to evaluate this broad array of population health interventions in addition to the traditional evaluation of new drugs and devices.

As we move forward in shaping this degree, we welcome your views and opinions. With your input, we hope to build a strong and

sustainable program which develops national leaders in CER. ■

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REFERENCES

1. Leonhardt D. After the great recession: An interview with President Obama. *New York Times Magazine*, May 3, 2009.
2. Institute of Medicine. *Initial national priorities for comparative effectiveness research*. Washington, DC: National Academies Press; 2009.
3. Sox HC and Greenfield S. Comparative Effectiveness Research: A Report From the Institute of Medicine. *Annals of Intern Med* 2009; 151(3):203-205. <http://www.annals.org/cgi/content/short/151/3/203>. Accessed August 20, 2009.
4. Pizzi LT and Singh VM. Pharmacoeconomics is coming of age (Macroscopy). *Clin Pharmacol & Ther*. 2008; 84(2):189-190.
5. Hoffman A and Pearson SD. Marginal medicine: Targeting comparative effectiveness research to reduce waste. *Health Affairs*. 2009; 28(4): w710-w718.