Journal of Medical Economics

ISSN: 1369-6998 (Print) 1941-837X (Online) Journal homepage: informahealthcare.com/journals/ijme20

Comparative effectiveness research in the United States: a progress report

Sean D. Sullivan, Josh J. Carlson & Ryan N. Hansen

To cite this article: Sean D. Sullivan, Josh J. Carlson & Ryan N. Hansen (2013) Comparative effectiveness research in the United States: a progress report, Journal of Medical Economics, 16:2, 295-297, DOI: 10.3111/13696998.2012.754613

To link to this article: https://doi.org/10.3111/13696998.2012.754613

Published online: 11 Dec 2012.

Journal of Medical Economics Vol. 16, No. 2, 2013, 295–297

1369-6998
doi:10.3111/13696998.2012.754613

Article JMEJ-754613/-75461

Original article

Comparative effectiveness research in the United States: a progress report

Sean D. Sullivan
Josh J. Carlson
Ryan N. Hansen
Pharmaceutical Outcomes Research and Policy Program, University of Washington, Seattle, WA, USA

Address for correspondence:
Sean D. Sullivan, PhD, Stergachis Family Professor and Director, Pharmaceutical Outcomes Research and Policy Program, School of Pharmacy, University of Washington, 1959 NE Pacific Ave, H375Q, Box 357630, Seattle, WA 98195-7630, USA.
Tel.: 206-685-8153; Fax: 206-543-3835;
sdsull@uw.edu

Key words:
Comparative effectiveness research – Patient-centered outcomes – Cost-effectiveness

Accepted: 28 November 2012; published online: 11 December 2012
Citation: J Med Econ 2013; 16:295–7

Abstract

The United States, with vast resources devoted to medical care, is investing in a new approach designed to improve health care efficiency. The conduct and reporting of comparative effectiveness research (CER) promises to help clinicians, payers, patients and other stakeholders make better and more informed decisions. Expectations for CER are high and public and private investments are sizeable. This paper discussions the evolution of CER in the US and gives a brief report on its progress and challenges.

Introduction

There are many questions as to why the US, with vast resources devoted to healthcare, cannot effectively control ever-increasing healthcare expenses. Some point out that Americans view their healthcare system as exceptional and, as a consequence, have come to expect the best possible care, without regard to cost[1]. Others take the view that unnecessary government interference restrains competition that would otherwise make the healthcare system more efficient and, by extension, its institutions better purchasers of health technology. The reality is more complex, particularly when it comes to managing the adoption and use of healthcare technology. Recent investments in the development of a Comparative Effectiveness Research (CER) enterprise in the US hope to provide a politically palatable and sustainable approach to addressing the evidence needs of a diverse group of healthcare stakeholders. The hope for CER is that better and more relevant comparative evidence will be generated for stakeholders. In this commentary, we aim to describe how CER may reduce costs by improving the type and availability of evidence to support decisions (clinical and coverage) and to give an update on the status of CER in the US.

Evidence-based coverage and reimbursement

The decision by a healthcare payer to cover healthcare technology reflects a mix of evidence evaluation and legal considerations. Health insurance contracts and government health program statutes contain language that restricts these programs from denying treatments that are deemed *reasonable and medically necessary*. Historically, medical necessity has been defined by whether or not a new drug, device, or diagnostic was granted market authorization by the Food and Drug Administration (FDA). As a consequence, nearly every treatment approved by the FDA is listed for reimbursement soon after market launch—although the extent of reimbursement is subject to negotiation. More recently, judgments about medical necessity have been informed by consensus statements and evidence-based guidelines from professional societies (see, for example, the

National Comprehensive Cancer Network [NCCN] guidelines). Framers of these guidelines are well aware that their recommendations directly influence the reimbursement policies of most US payers.

Healthcare payers operate technology assessment programs that further support the coverage and reimbursement process. Yet, patient advocacy groups, medical societies, and the life sciences industry suggest that government or health insurance bureaucrats will use *Death Panels* to deny Americans life-saving treatments. They believe that healthcare professionals should be solely responsible for the selection of treatments, without interference from those who pay the bill. There remains a healthy skepticism about rationing, as many believe that decisions taken by private payers to restrict treatments are motivated by profit. Yet most Americans do not fully appreciate that care in the US is already rationed on the basis of income, race, age, and geography.

The availability and type of evidence

There is almost universal recognition that the evidence available to inform clinical and resource allocation decisions for new and oftentimes expensive health technologies is in short supply and of the wrong type. The research enterprise in the US is not providing enough relevant information to clinicians, payers, patients, and other stakeholders. How then can the institutions and participants of a learning healthcare system function properly when the evidence base for much of what it does is absent or not fit for purpose? At best, the available evidence comes either much too late in the life cycle of the technology or is derived from pre-licensing development programs designed to gain FDA approval. At worst, there simply is no evidence. In order to address this problem and with an eye toward improving patient outcomes and reducing cost trends, recent healthcare policy in the US has focused on prioritizing, generating, and disseminating evidence that matters to patients, clinicians, and purchasers.

Comparative effectiveness research as a policy solution

In 2006, Wilensky[2], the former head of the US Centers for Medicare and Medicaid Services, called for a massive investment of $5B USD per year to reinvent health services and clinical research to tackle the largely ignored, yet critically important questions in healthcare – those that address the comparative effectiveness and safety of healthcare interventions. In 2008, the Institute of Medicine (IOM) of the National Academy of Sciences called for the development and funding of a sustainable program of comparative effectiveness research (CER) in the US[3].

In 2009, at the beginning of the recession, the Obama administration pushed through a government-funded stimulus package (the American Reinvestment and Recovery Act—ARRA) aimed at supporting jobs and infrastructure as a bridge to a broader economic recovery. Within the stimulus package, the administration provided $1.1B USD in government funding to seed the development of a CER program. That same year, the IOM was tasked with defining CER and setting research priorities for the country. The IOM[4] 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 policymakers to make informed decisions that will improve healthcare at both the individual and population levels.

The Patient Protection and Affordable Care Act (PPACA – Pub L. 111-148) of 2010 (p. 33) was wide ranging in its attempt to provide near universal healthcare coverage to US citizens and address necessary insurance and payment reforms within the healthcare system. A key provision in the Act was to extend the earlier investment in CER by establishing a public/private institute charged with assessing and funding CER priorities. The Patient-Centered Outcomes Research Institute (PCORI) is supported through federal and private sector funding[5].

The PCORI mission is to fund and communicate CER that:

> . . . considers the range of outcomes that are important to patients, and that attends to the possibility that the comparative effectiveness of treatments could differ for various patient groups (p. 2194)[6].

A progress report

Leading up to the debate over PPACA, the head of the US Congressional Budget Office, Peter Orzag, submitted a letter to Congress suggesting that federal support for CER would reduce healthcare costs. For many, this letter revealed the potential for CER to address the healthcare cost problem in the US. For the research and policy community, it created a testable hypothesis. Much of the research funded by the ARRA investment is just now being reported. The portfolio of funded projects and programs is wide ranging and includes support for capacity building in the form of training of CER scientists. The federal government requires transparency in the reporting of ARRA funds and has commissioned a review of the effectiveness of its investments in CER[7,8].

PCORI, charged with leading the campaign to produce CER evidence, has spent its first 2 years defining itself and patient-centered outcomes research, establishing

processes, and supporting small projects and position papers on methods. It has only recently released funding announcements for major research programs. The impact of PCORI's work to-date is unclear. A clause in the enabling legislation stipulates that PCORI will expire in 2019 unless re-authorized by Congress. In a recent paper critical of PCORI's slow start, Sox[9] points out that PCORI has a very short time to prove its worth to Congress. Although, PCORI's desire to allow researchers and stakeholders to naturally define specific clinical areas for research priority may have merit, it may prove unbearably slow and lead to the demise of PCORI[9].

Despite the slow start, there is reason for optimism. Interest in the methods and applications of CER has moved rapidly into the research community. There are reports too numerous to count on the types and appropriateness of experimental, observational, and systematic review methods to establish comparative effectiveness and harms. Along with PCORI's own methodology committee, groups of researchers have formed to consider and publish good scientific practices of CER[10,11]. These recommendations will serve as excellent guides to evaluate the results of CER studies funded by PCORI and other CER funders moving forward.

CER and cost-effectiveness

The PPACA does not preclude PCORI from either conducting or funding cost-effectiveness research. It does, however, contain language that restricts PCORI from developing a cost-effectiveness threshold (specifically, cost per QALY) for the US or making recommendations to Federal healthcare programs about the cost-effectiveness of specific interventions. Some view this as an explicit message from Congress that the US will not develop an agency like the National Institute for Clinical Excellence (NICE).

Nevertheless, information about the costs and benefits of medical interventions is important to stakeholders and the methods to estimate cost-effectiveness are well described and mature. An increasing number of payers in the US are requesting information on the cost-effectiveness of drugs and other interventions. One hopes that PCORI will find a way to support research that helps these stakeholders understand and consider the value of interventions.

A final comment

In order to bend the cost curve in the US, the results of high impact CER will need to be disseminated and rapidly taken up in clinical practice and throughout the healthcare system. Healthcare payers will need to access the information and be willing to change reimbursement policies to match findings from these studies. Provider groups need to adopt and apply high value treatments in order to improve outcomes for their patients. Accountable care organizations and patient-centered medical home models appear ready to do so, but many of these models are so new that they remain under conceptualization and development. Importantly, patients and their care-givers also must be willing to learn about and use comparative information in discussions with providers about what treatments are best for them.

The expectations in the US for CER remain high. Comparative effectiveness research in the US is relatively young, but will need to grow up fast in order to meet these expectations and reward the sizable public investments with improved health and efficient healthcare.

Transparency

Declaration of funding
JJC and RNH are AHRQ-funded K12 Scholars in Comparative Effectiveness Research and Patient-Centered Outcomes Research (K12HS019482, K12HS021686).

Declaration of financial and other relationships
The authors have no financial or other conflicts to report.

Acknowledgments
The authors thank Paul Kraegel, MSW, for editorial support.

References

1. Neumann PJ. American Exceptionalism and American Health Care: implications for the US Debate on Cost-Effectiveness Analysis. London, UK: Office of Health Economics Briefing, 2009. No. 47
2. Wilensky G. Developing a center for comparative effectiveness information. Health Affairs 2006;25:572-85
3. Eden J, Wheatley B, McNeil B, et al, editors. Knowing what works in health care: a roadmap for the nation. Washington, DC: National Academies Press, 2008
4. Institute of Medicine. Initial national priorities for comparative effectiveness research. Washington, DC: National Academies Press, 2009
5. Washington AE, Lipstein SH. The Patient-Centered Outcomes Research Institute—promoting better information, decisions, and health. N Engl J Med 2011;365:e31
6. Selby JV, Fleurence R, Lauer M, et al. Reviewing hypothetical migraine studies using funding criteria from the Patient Centered Outcomes Research Institute. Health Affairs 2012;10:2193-9.
7. www.recovery.gov. Accessed October 20, 2012
8. http://www.mathematica-mpr.com/publications/PDFs/labor/recovery_act_designeval.pdf. Accessed October 20, 2012
9. Sox HC. The Patient-Centered Outcomes Research Institute should focus on high-impact problems that can be solved quickly. Health Affairs 2012; 31:2176-82
10. Luce BR, Drummond MF, Dubois RW. Principles for planning and conducting comparative effectiveness research. J Comp Eff Res 2012;1:431-40
11. Patient-Centered Outcomes Research Institute. Preliminary draft methodology report: ''our questions, our decisions: standards for patient- centered outcomes research''. Washington, DC: PCORI, 2012. http://www.pcori.org/assets/ Preliminary-Draft-Methodology- Report.pdf. Accessed November 15, 2012

