Comparative Effectiveness Research in Hand Surgery

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KEYWORDS

• Comparative effectiveness research • Hand surgery • Large databases
• Patient-reported outcomes

KEY POINTS

- The US Institute of Medicine in 2008 started a national initiative of research known as comparative effectiveness research (CER), which will support better decision making about interventions in health care.
- CER focuses on interventions that occur within real-world environments, therefore the conclusions can be generalized to a broad population.
- CER conducted through large electronic databases allows researchers to evaluate how current health care practices affect the outcomes of care.
- To date, there is minimal comparative effectiveness evidence in hand surgery, which is partly attributed to the lack of relevant outcomes information included in electronic databases.
- Inclusion of patient-related outcomes into electronic databases will facilitate the adaptation of CER into hand surgery.

INTRODUCTION

The US Institute of Medicine (IOM) in 2008 started a national initiative known comparative effectiveness research (CER) to support better decision making about interventions in medicine.[1] Clinical decision making varies based on patient factors, clinicians' experience, and regional preferences. These decisions are often made without supportive evidence. Inconsistent clinical practice is well recognized and raises concerns about the appropriateness and economics of current medicine, which is evident from the cost and outcome differences that exist in health care across the United States.[2] The IOM attributes inconsistencies in health care delivery to the lack of information available to make well-informed decisions in everyday clinical medicine.

The IOM publicized the need for high-impact research to improve the quality and efficiency of health care in a comprehensive report in 2008.[3] In response, legislators passed the American Recovery and Reinvestment Act (ARRA) of 2009, which allocated $1.1 billion to fund CER.[1] The Federal Coordinating Council and an appointed IOM committee were charged with identifying high-priority research topics and to allocate funds from the ARRA. The President distributed these funds to the National Institutes of Health (NIH), Agency for Health Research and Quality (AHRQ), and Office of the Secretary of the US Department of Health

Supported in part by grants from the National Institute of Arthritis and Musculoskeletal and Skin Diseases (2R01AR047328 - 06, R01 AR062066) and a Midcareer Investigator Award in Patient-Oriented Research (2K24-AR053120-06) to Dr. Kevin C. Chung.

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Hand Clin 30 (2014) 319–327
http://dx.doi.org/10.1016/j.hcl.2014.04.001
0749-0712/14/$ – see front matter

and Human Services (DHHS). In 2010, as part of the Affordable Care Act, legislators established an ongoing national program in CER, the Patient-Centered Outcomes Research Institute (PCORI).[4]

CER is not a novel concept, but represents a research movement propagated by a large investment by the federal government. CER can be conceptualized as a form of outcomes research. This research asks which intervention is most effective, for whom, and under what circumstances.[5] Through encouragement from the IOM, CER trials often use large electronic databases to study current health practices and outcomes. They are often referred to as pragmatic trials, because they reflect routine clinical practice.[6]

Hand surgery has embraced outcomes research and, to better evaluate effectiveness of interventions, have developed questionnaires such as the Michigan Hand Outcomes Questionnaire (MHQ). The MHQ is a subjective evaluation tool used to measure outcomes such as hand function and pain.[7] These instruments report patient-related outcomes (PROs), a recognized and standardized method of reporting patients' perspectives on interventions.[8] PROs provide the patients' perspectives on treatment benefit and can be the outcome of greatest importance.[8] Therefore, incorporating PROs into CER greatly enhances the quality of hand surgery research, thus providing better evidence on which to base clinical decisions.

DEFINITION OF CER

CER is designed to provide information about the relative effectiveness of different medical interventions to improve the quality and value of care.[9] The IOM defines 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."[3] When conducting CER, investigators are asking themselves how an intervention compares, both overall and in subsets of the population.[10] Therefore, researchers seek to determine what interventions are appropriate for particular patients and populations within a variety of circumstances. CER investigates interventions, tests (eg, diagnostic, therapeutic), prevention strategies, care delivery, and quality of care.[4]

The IOM adds 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 level."[8] Thus, information should lead to more standardized care, and should recognize that decisions may vary if individuals are categorized in a particular subset. The IOM clearly identifies multiple stakeholders, outside the doctor-patient relationship, including payers and policy makers. In acknowledging these stakeholders, there is an underlying national objective of optimizing health outcomes within financial and resource constraints.[11]

METHODOLOGY OF CER

CER is the study of 2 different but accepted standard practices, neither of which is superior based on available medical evidence.[6] Interventions are simple and occur within practical clinical settings. A key component is the use of real-world data, therefore reflecting patients who are typical of day-to-day clinical care.[1] Without specific patient inclusion criteria, conclusions are drawn from a population representative of those who would receive the intervention in a normal clinical setting.[9] Observational CER studies include patient cohorts numbering in the thousands that are achieved through large medical databases. Outcomes of CER studies are intended to be clinically relevant, meaningful to the patient and general public, and subject to minimal ascertainment bias.[4,12]

CER methodologies are in contrast with classical double-blinded randomized controlled trials (RCTs), which are conducted on highly selected populations with rigorous inclusion and exclusion criteria. Study enrollment in RCTs includes patients with few comorbidities in order to optimize statistical power and the benefit/risk trade-off.[11] These so-called efficacy studies assess whether an intervention is efficacious under ideal, controlled settings.[1,11] The outcome measures of efficacy trials are often arbitrary and less clinically relevant.[6] RCTs answer the question "does this work?"[1] Alternatively, CER provides decision makers with the answer to "is this better than that?."[1] In simplistic terms, CER studies are less controlled, with fewer exclusion criteria, and therefore the conclusions can be generalized to a large population. The IOM supports the broad use of evidence to evaluate effectiveness,[11] including systematic reviews, retrospective database analysis, prospective observational studies, or pragmatic RCTs. Despite this statement, there is strong emphasis placed on observational, database research. **Table 1** shows the characteristic differences between classic RCTs and observational, database-conducted CER.

THE VALUE OF ELECTRONIC DATABASES IN CER

Electronic databases, including administrative claims databases and electronic health record