



Principles for Comparative Effectiveness Research

The Institute of Medicine (IOM) defines Comparative Effectiveness Research (CER) as the direct generation of clinical information on the relative merits or outcomes of one intervention in comparison to one or more others, and secondary comparative effectiveness research involves the synthesis of primary studies to allow conclusions to be drawn. Secondary comparisons of the relative merits of different diagnostic or treatment interventions can be done through collective analysis of the results of multiple head-to-head studies, or indirectly, in which the treatment options have not been directly compared to each other in a clinical evaluation, and inferences must be drawn based on the relative effect of each intervention to a specific comparison, often a placebo. (IOM, “Learning What Works Best: The Nation’s Need for Evidence on Comparative Effectiveness in Health Care,” September 2007.)

Value

Value can be thought of as the best balance between benefits and costs, and better value as improved clinical outcomes, quality, and/or patient satisfaction per dollar spent. Improving value in the U.S. health care system will require both clinical and cost information. Quality comparative clinical effectiveness research (CER) will improve health care value by enhancing physician clinical judgment and fostering the delivery of patient-centered care, which is cost effective.

Independence

A federally sponsored CER entity should be an objective, independent authority that produces valid, scientifically rigorous research.

Stable Funding

The entity should have secure and sufficient funding in order to maintain the necessary infrastructure and resources to produce quality CER. Funding source(s) must safeguard the independence of a federally sponsored CER entity.

Rigorous Scientifically Sound Methodology

CER should be conducted using rigorous scientific methods to ensure that conclusions from such research are evidence-based and valid for the population studied. The primary responsibility for the conduct of CER and selection of CER methodologies must rest with physicians and researchers.

Transparent Process

The processes for setting research priorities, establishing accepted methodologies, selecting researchers or research organizations, and disseminating findings must be transparent and provide physicians and researchers a central and significant role.

Significant Patient and Physician Oversight Role

The oversight body of the CER entity must provide patients, physicians (MD, DO), including clinical practice physicians, and independent scientific researchers with

substantial representation and a central decision-making role(s). Both physicians and patients are uniquely motivated to provide/receive quality care while maximizing value.

Conflicts of Interest Disclosed and Minimized

All conflicts of interest must be disclosed and safeguards developed to minimize actual, potential and perceived conflicts of interest to ensure that stakeholders with such conflicts of interest do not undermine the integrity and legitimacy of the research findings and conclusions.

Scope of Research

CER should include assessments of diagnostic and treatment modalities for a given disease or condition in a defined population of patients. Diagnostic and treatment modalities should include drugs, biologics, imaging and laboratory tests, medical devices, health services, or combinations. It should not be limited to new treatments. In addition, the findings should be re-evaluated periodically, as needed, based on the development of new alternatives and the emergence of new safety or efficacy data. The priority areas of CER should be on high volume, high cost diagnosis, treatment, and health services for which there is significant variation in practice. Research priorities and methodology should factor in any systematic variations in disease prevalence or response across groups by race, ethnicity, gender, age, geography, and economic status.

Dissemination of Research

The CER entity must work with health care professionals and health care professional organizations to effectively disseminate the results in a timely manner by significantly expanding dissemination capacity and intensifying efforts to communicate to physicians utilizing a variety of strategies and methods. All research findings must be readily and easily accessible to physicians as well as the public without limits imposed by the federally supported CER entity. The highest priority should be placed on targeting health care professionals and their organizations to ensure rapid dissemination to those who develop diagnostic and treatment plans.

Coverage and Payment

The CER entity must not have a role in making or recommending coverage or payment decisions for payers.

Patient Variation and Physician Discretion

Physician discretion in the treatment of individual patients remains central to the practice of medicine. CER evidence cannot adequately address the wide array of patients with their unique clinical characteristics, co-morbidities and certain genetic characteristics. In addition, patient autonomy and choice may play a significant role in both CER findings and diagnostic/treatment planning in the clinical setting. As a result, sufficient information should be made available on the limitations and exceptions of CER studies so that physicians who are making individualized treatment plans will be able to differentiate patients to whom the study findings apply from those for whom the study is not representative.