Outcomes Research
Myths and Realities
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Outcomes research is growing in visibility and stature, yet misconceptions persist. Many people remain unfamiliar with outcomes research and what distinguishes it from other research areas that study patient outcomes. Although I have previously framed the scope and purpose of outcomes research, I would like to provide further clarification about the field.

Although the exact origins of the term “outcomes research” are unclear, Ellwood coined the term “outcomes management” in his 1988 Shattuck Lecture, in which he envisioned a future in which patient management would be driven by the experience of how similar patients fared as a consequence of alternative treatments. Clancy and Eisenberg marked the entry of outcomes research into the scientific lexicon in 1998 with a classic article in Science that stated that “outcomes research—the study of the end results of health services that takes patients’ experiences, preferences, and values into account—is intended to provide scientific evidence relating to decisions made by all who participate in health care.” This elegant definition is both simple and profound. Outcomes research seeks to inform decisions and to hold pre-eminent the perspective of patients and the public. By saying that the research should assist those who participate in health care, they emphasize the needs of those who receive, provide, organize, and pay for health care, including the public.

Eisenberg, as Director of the Agency for Healthcare Research and Quality, often quoted Representative John Porter’s remarks from a 1998 Congressional appropriations hearing. Porter stated, “What we really want to get at is not how many reports have been done, but how many people’s lives are being bettered by what has been accomplished.”

Eisenberg frequently used the quote to emphasize the need for questions about what research has accomplished, and by extension, what our efforts in health care and public health have produced to improve people’s lives. The implicit message was that the assessment of our success depended on our knowledge of what is important to those we seek to help.

The vision of outcomes research crystallized from the sense that, amid the growing complexity of medical care and the health care system, research was needed to guide practice and policy by focusing attention on the experience of patients and the public. The growing body of work that questioned the safety, effectiveness, efficiency, equity, patient-centeredness, and timeliness of current strategies to promote health and health care was leading to calls for increased accountability and innovation. At its core, outcomes research is a domain in which the convergence of multiple disciplines is applied to generate knowledge of proximate and tangible importance to the delivery and organization of health care. In this Editor’s Perspective, I would like to specifically address some of the common misunderstandings about outcomes research.

Myth: Outcomes Research is a Singular Discipline
Reality: Like clinical science, outcomes research is not a single field, but rather the nexus at which disciplines converge to generate scholarship. At the vanguard of collaborative research, the field may connect to the basic biological sciences, epidemiological sciences, clinical sciences, social sciences, and statistical sciences, with an emphasis on cross-discipline, national, and multi-national partnerships to address clinically important questions.

Myth: Outcomes Research is Defined by Its Focus on Secondary Data Analysis
Reality: A common misperception is that outcomes research is defined by its use of observational methodology and, particularly, existing datasets. In reality, outcomes research is defined by the very nature of the questions that it addresses, which seek to provide insight about making our efforts more effective, efficient, equitable, timely, and patient-centered.

The articles already published in the first 3 issues of this journal illustrate the breadth of methodologies used. We have published retrospective observational studies, prospective observational studies, randomized trials, an economic analysis, surveys, and a meta-analysis. Moreover, we anticipate publishing qualitative studies and other methodological approaches.

Among these methodologies, there is no clear hierarchy in approach. Although randomized trials are often considered the best methodological approach in clinical research, this view derives from a narrow perception of the type of knowledge being sought. For studies of the efficacy of an intervention that attempts to minimize bias, a double-blind randomized trial may be the best approach. However, if the goal is to examine variation in practice, an observational design is the only way to address how care differs and what patterns may ultimately be in the best interests of patients. To describe costs of care, economic studies of actual practice are clearly superior to clinical trials in which patient management...
is often dictated by complex protocols that bear little resemblance to real-world clinical care. To study areas of practice that involve patient–doctor interactions, or to identify new factors that may be associated with better care and clinical outcomes, a qualitative design might be optimal. In the selection of a study design, it is important to characterize the question and choose a methodology based on the strengths, weaknesses, and feasibility of all alternatives.

**Myth: Outcomes Research is About “T2” Translational Research**

Reality: Although there is a recent emphasis on moving basic discoveries more quickly through developmental regulatory and testing stages and into clinical practice, outcomes research is not merely at the end of that line. It is true that T2 translational research, which is focused on the translation of results from clinical studies into everyday clinical practice and health care decision-making, is part of outcomes research. However, the scope of outcomes research is far more expansive. The unidirectional models of knowledge generation, dissemination, and application miss the key role of outcomes research as a feedback loop that continually reassesses what is being achieved. There is often much more to know about a phase III clinical strategy than is revealed within a clinical trial or a formative group of clinical studies. Rather than just developing practice strategies, there is a continuing need to assess the safety and effectiveness of treatments as they are disseminated beyond the clinical study environment. Moreover, outcomes research can refine the interpretation of prior evidence and lead to continuous assessments of and modifications in practice and policy.

**Myth: Outcomes Research is a Fad**

Reality: Outcomes research is here to stay. With concerns about the cost of health care, the quality of medical care and public health efforts, and a focus on health care reform, rigorous scholarship is paramount to future decision-making. Policy-makers, clinicians, and patients increasingly seek information about comparative effectiveness of therapies, the value of various clinical strategies, and the utility of innovative approaches. In this context, pragmatic research that brings together experts from disparate disciplines will increasingly be at the forefront of successful efforts to address the daunting challenges of the health care environment.

The foundation for outcomes research in the area of cardiovascular disease and stroke has strengthened, due in large part to the support of the American Heart Association (AHA). The Scientific Forum on Quality of Care and Outcomes Research in Cardiovascular Disease and Stroke annual conference, which will celebrate its 10th year in 2009, attracts more than 600 attendees annually and serves as a venue for collaboration and advancement of the latest methods in the field. The AHA Quality of Care and Outcomes Research Interdisciplinary Group, now an AHA Council, enables the outcomes community to participate formally in the AHA governance structure, support trainees, and lead important statements relevant to advancing cardiovascular health. In addition, Circulation: Cardiovascular Quality and Outcomes was established to promote the best values of outcomes research and serve as a vehicle to publish exemplary scholarship.

With regard to funding, the 4 recently established AHA Outcomes Research Centers, with support of more than $10 million, provide a strong infrastructure at national universities. The trainee positions supplement other training opportunities through the Robert Wood Johnson Clinical Scholars Program, the National Heart, Lung, and Blood Institute (NHLBI) Training Centers, and the Veterans Administration. Importantly, each center has the capacity to offer training to the next generation of outcomes researchers.

The AHA, however, is not the only organization to recognize the need and importance of outcomes research. The NHLBI formed a working group to set priorities in outcomes research and is committed to funding meritorious work in this field. The promise of electronic health records to yield powerful research insight is being harnessed by groups such as the NHLBI-funded Cardiovascular Research Network. The Agency for Healthcare Research and Quality has long demonstrated its commitment to outcomes research in the form of training programs and grants. Hospitals and health care systems are also investing in research institutes that can be internal resources as well as prominent national and international contributors. Industry provides an additional source of funding, although potential conflicts of interest need to be managed.

**Summary**

The field of outcomes research is a common ground on which diverse disciplines and experts work together to augment and improve our efforts to promote the health of populations and patients. With the need for scholarship to guide policy and practice, the support of a growing number of organizations, and the promise of collaboration among innovators across disciplines, outcomes research will continue to play a vital role in the improvement of health and health care in the new millennium.

**Disclosures**

None.

**References**


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