The Role of Outcomes Research in Improving the Quality of Medical Care

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The proliferation of medical technology, including prescription drugs, devices, and procedures, is often perceived in the current managed healthcare environment as a threat to the ability of managed care organizations to control healthcare costs. However, the ongoing need for new technologies and innovations in medical care demands that we look beyond the cost of developing and acquiring such technologies to the outcomes being achieved with the resources invested. We need to document not only costs and/or clinical outcomes related to such technologies and interventions, but also measures such as cost effectiveness, patient quality of life, and patient satisfaction. Outcomes research is the term given to the assessment of such measures.

DEFINING OUTCOMES RESEARCH

Outcomes research is conducted primarily when there is little consensus in the medical community about the most effective course of treatment for certain conditions. In its broadest scope, outcomes research is used to determine how best to optimize clinical and economic outcomes, patient quality of life, patient functioning status, and patient satisfaction associated with an intervention. In this discussion, we focus primarily on economic outcomes research as a management tool to document and compare costs in conjunction with outcomes (Fig. 1).

Economic outcomes research can be conducted in many different settings and with many different tools currently available to the medical community, including modeling, randomized trials, and observational databases. Once the community has, through this research, reached consensus around the most effective treatment practices, assessment moves away from outcomes research and toward disease management. This process of "outcomes management" is depicted in Figure 2. For the purposes of this discussion, we focus on the right side of the figure, the outcomes research component, because it is not as well understood among the medical community.

WHEN TO CONDUCT OUTCOMES RESEARCH

Currently, the agenda for conducting outcomes research is being set primarily by the manufacturers of medical technology, such as pharmaceutical and biotechnology firms. This "top-down" approach to research is not always optimal, however, in that it does not necessarily address the questions most relevant to the providers of medical care or to patients. From the perspective of providers and patients, outcomes research is most appropriate when

- a condition is prevalent
- a condition or intervention is associated with a high cost
- the medical community's understanding of a condition or intervention is changing rapidly
- there is a wide variability in treatment approaches and costs for a condition
- there is a large disparity between the acquisition costs of competing interventions for a particular condition.

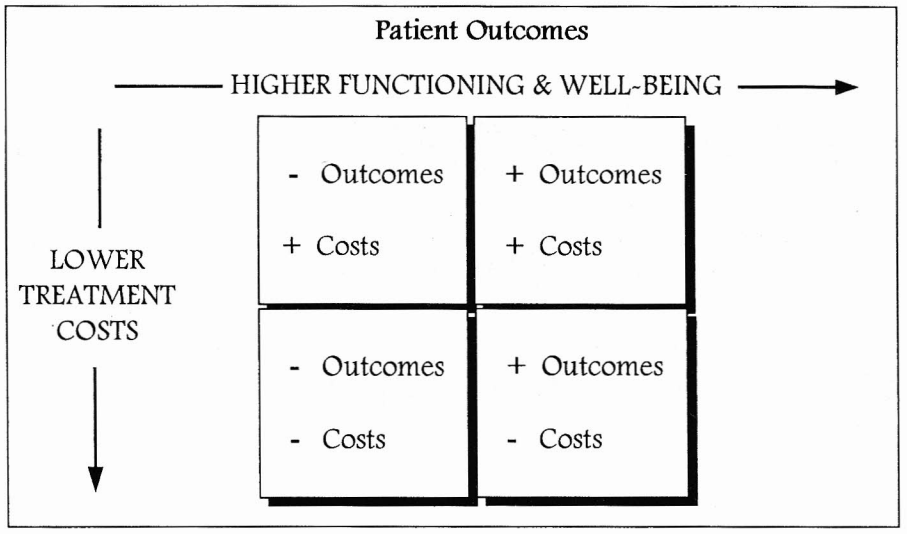

Figure 1. Outcomes research as a management tool.

Outcomes research may also be utilized when providers of care, including managed care organizations and other healthcare institutions, are in need of information for evaluation, accreditation, and quality control purposes (e.g., NCQA, HEDIS).

METHODS FOR CONDUCTING OUTCOMES RESEARCH

There are primarily five approaches to collecting economic outcomes data, each of which is described below in more detail. These options are not mutually exclusive. The collection of economic data for each of these approaches is dependent on the perspective and objectives of the study. For example, if the perspective of the analysis is societal, costs should include direct medical and nonmedical (e.g., transportation to obtain care) costs, as well as indirect costs, such as time lost from work or diminished productivity. If the perspective of the analysis is the payer, costs should include only direct medical costs as borne by the payer.

1. Primary Database Studies.

Databases containing primary (patient-specific) information include claims databases maintained by third-party payers (e.g., health maintenance organizations, pharmacy benefits managers, state or federal government entities) and automated patient record databases maintained by healthcare providers and institutions. These studies offer the advantage of being relatively quick and inexpensive to conduct. However, the quality and completeness of the data collected from such sources may be lacking.

2. Modeling Studies.

When primary data are not available to assess outcomes, data may be derived from expert opinion, published literature, secondary data sets, and/or clinical safety and efficacy trials to construct a model of the condition/intervention. Modeling is a fairly quick and inexpensive way to predict outcomes but rests on numerous assumptions that may not reflect real-world clinical practice.

3. "Piggyback" Studies.

Prospective economic data collection may be achieved by "piggybacking" onto planned clinical safety and efficacy trials. This approach offers the advantages of being less expensive than stand-alone economic trials and of being based on primary data collection, which is often blinded and randomized. The disadvantages are that sample sizes for safety and efficacy trials are often small, limiting the generalizability of the results; resource use is, at least to some degree, driven by the trial protocol, which does not necessarily reflect normal practice patterns; and patients who do not comply with the trial protocol are dropped from the study in contrast to "real-world" treatment, in which noncompliance with treatment is a common occurrence.

4. Naturalistic Prospective Studies.

Data are collected as usual care is provided, reflecting the true effectiveness of the intervention (as opposed to the efficacy in "piggyback" studies). The primary disadvantages of this approach are that it is expensive, time-consuming, and can provide "elegant answers to irrelevant questions" by the time the study is completed due to rapidly changing clinical practice.

5. Observational Databases.

Data are tracked longitudinally on patient clinical outcomes, resource use, workforce participation, quality of life, and satisfaction with care. These data are collected through a combination of patient self-report and physician-reported information. The data are then used to conduct cohort or case-control analyses to evaluate outcomes for patients under a variety of treatment scenarios. Such databases are expensive to create and maintain but offer the advantages of providing unbiased, clinically relevant analyses based on data from real-world settings, accommodating

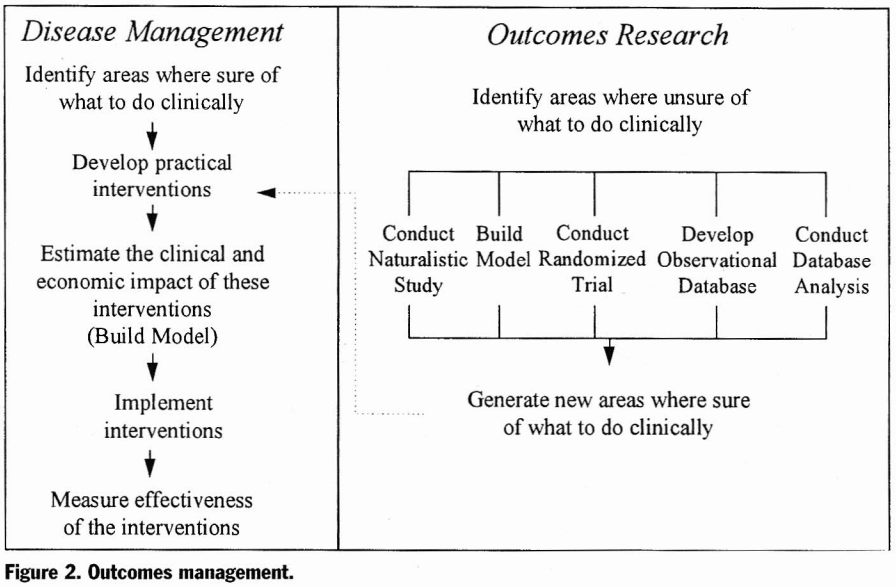

Figure 2. Outcomes management.

changes in practice patterns and treatments over time, and providing data useful to many different groups and for many different purposes.

No matter which approach to data collection is taken, the results must then be analyzed. As described below, there are five primary types of economic analyses available to the medical community.

1. Cost-Minimization Analysis.

When efficacy or effectiveness outcomes are equivalent between two treatments, costs are simply identified and compared. This type of analysis goes beyond acquisition costs to include the costs of treatment failures, adverse events, and monitoring.

2. Cost-of-Treatment Analysis.

The total cost of an intervention or treatment is compared to the total cost of standard care for a specific condition.

3. Cost-of-Illness Analysis.

The direct and indirect costs associated with an illness are combined with a measure of either the incidence or prevalence of the illness to ascertain the economic impact to a specific population (usually society).[1]

4. Cost-Effectiveness Analysis.

The total cost and effectiveness of one intervention or treatment is compared to the total cost and effectiveness of another relevant intervention or treatment to determine marginal benefit.[2] An intervention or treatment is found to be cost-effective in comparison to another if it is either (a) less costly and at least as effective, (b) more effective and more costly, with the added benefit worth the added cost (also called incremental cost effectiveness), (c) less effective and less costly, with the added benefit of the alternative not worth the added cost, and (d) cost saving with equal or better effectiveness.[3]

5. Cost-Utility Analysis.

This is one type of cost-effectiveness analysis in which quality-of-life effects are incorporated into the economic assessment. Years of life expectancy are adjusted to reflect the associated impact of the intervention or treatment on quality of life, and the result is expressed in terms of cost per quality-adjusted life years (QALYs) saved attributable to the intervention or treatment.

6. Cost-Benefit Analysis.

This kind of analysis attempts to assign a dollar value to the lives saved (by either a human-capital or willingness-to-pay methodology) or improvements in quality of life attained as a result of an intervention or treatment. This type of analysis is not used as often as those discussed above because it requires making judgments about the monetary value of human life.

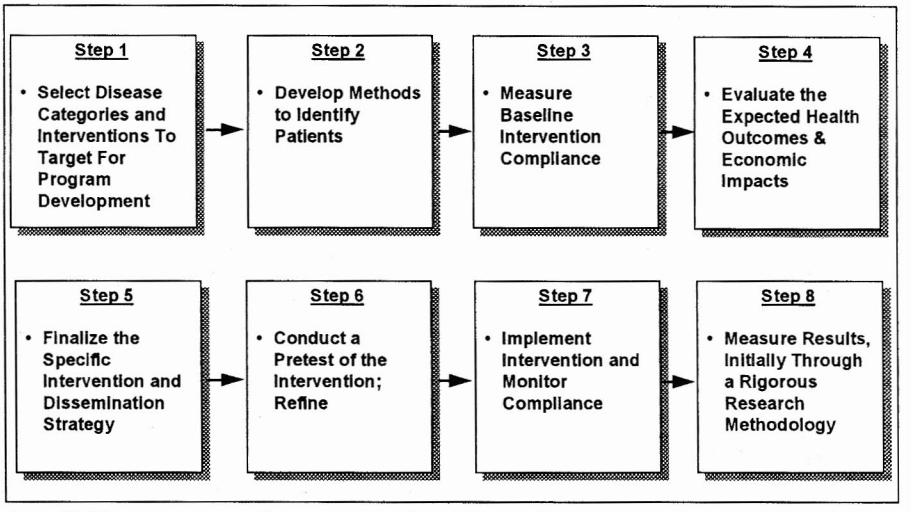

Figure 3. Disease management—process overview.

EXAMPLES OF OUTCOMES RESEARCH IN PRACTICE

The following studies, which are all currently being conducted, are described so that they might further elucidate the methodology of outcomes research in practice.

Modeling/Cost-Utility Study

A cost-utility analysis of a new medical device versus the standard surgical treatment for steroid-resistant immune thrombocytopenic purpura. Because no clinical trials have been conducted to date directly comparing the two treatments, a model is being constructed with data obtained from published literature, clinical trial results of the device, and expert opinion to represent the outcomes of each treatment over a 5-year period. Cost and effectiveness of each treatment, as represented by quality-adjusted life years, are combined into cost-effectiveness ratios and compared.

Primary Database/ Cost-of-Illness Analysis

A study of the cost of illness of acid-related disorders (ARDs) at the Northern California Kaiser Permanente Medical Group is being conducted in the following manner: patients are identified as having a diagnosis of ARDs through Kaiser's Outpatient Summary Clinical Record (OSCR) system; limited chart review is conducted to validate the OSCR diagnosis; ARDs-related resource use and costs are determined by Kaiser's automated systems; ARDs patients are matched to a control group of similar patients without ARDs; and the direct cost of treatment, as measured by the difference in average cost between the ARDs and non-ARDs group of patients, is combined with the indirect cost and prevalence of ARDs in the Northern California Kaiser system to estimate the overall cost of acid-related disorders to Kaiser.

Naturalistic Prospective/ Cost-Effectiveness Analysis

A study of the cost effectiveness of migraine headache treatment, also at the Northern California Kaiser Permanente Medical Group, is being conducted in the following manner: migraine headache sufferers are identified and enrolled when they present with an acute migraine to a Kaiser Permanente emergency room or outpatient clinic; patients are randomized to receive one of two migraine pharmacologic agents (patients are blinded to the treatment they receive so that the outcomes are not influenced by potential patient bias); through patient diaries, telephone interviews, and medical record reviews, clinical outcomes, such as headache resolution and recurrence, and economic outcomes, including direct and indirect resource use and costs, are monitored for 1 week post-treatment; and the cost effectiveness of each treatment is then assessed.

Pilot Observational Database

Approximately 500 patients with acid-related disorders (ARDs), including ulcer,

gastroesophageal reflux disease, and nonulcer dyspepsia, at 10 sites (primarily managed care sites) are being longitudinally tracked for 6 to 9 months for clinical, economic, quality-of-life, and patient satisfaction outcomes.

The results of these studies will be disseminated to the medical community in order to inform treatment decisions. In addition, as one component of the outcomes management process, the results will be operationalized through disease management interventions, as depicted in Figure 3.

THE FUTURE OF OUTCOMES RESEARCH

As we move from "the first wave of healthcare reform, containing costs, to the second wave, ensuring quality care," the need for uniform measures of quality and effectiveness that can be readily understood and implemented by the entire medical community will continue to grow.[4] In both Canada and Australia, guidelines for conducting outcomes research have already been established and disseminated. No such guidelines exist in the United States. However, the U.S. Food and Drug Administration (FDA) is beginning to explore the reporting of economic and quality-of-life outcomes in addition to clinical outcomes associated with new technologies. As such, the FDA is conducting ongoing hearings with experts in the field of medicine and outcomes research in an attempt to develop guidelines such as those in Canada and Australia. In addition, a Panel on Cost Effectiveness in Health and Medicine was created by the U.S. Public Health Service in 1993 to "enhance the comparability and quality of cost-effectiveness analyses" and to "resolve methodologic disputes in some areas and to develop a practical consensus in others in order to improve the policy relevance of this form of analysis."[5] The panel's recommendations, which should serve to advance the discussion of uniformity in outcomes measures in this country, will be published in the spring of 1996.

Furthermore, there is a growing demand from patients for outcomes data in order to make more informed treatment choices and to adequately judge the quality of the care they are receiving. This demand has led to the development of health plan "report cards," which seek to inform consumers of how well individual health plans are managing their medical care in terms of cost, patient quality-of-life, patient functioning, patient satisfaction, and clinical outcomes. The currently accepted standard in report cards is the Health Plan Employer Data and Information Set (HEDIS), which is produced by the National Committee for Quality Assurance (NCQA). However, the HEDIS report cards do not yet adequately link clinical measures to outcomes and are not standardized for nationwide use (NCQA will apparently release an updated national version of the HEDIS reports in 1997). Thus, the Foundation for Accountability (FAcct), a diverse group of consumers, payers, and other parties interested in the quality of healthcare, was recently formed to expand on the concept of the HEDIS report cards by proposing uniform outcomes measures. FAcct's first guidebook, focusing initially on the conditions of asthma, breast cancer, coronary artery disease, diabetes, depression, and low back pain, will also be released in the spring of 1996.[6] STI

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