CHAPTER 1:  
INTRODUCTION

Problem Statement

Patients who are diagnosed with a particular medical condition frequently must choose among treatments that will minimize pain, reduce disability, and/or enhance their likelihood of survival. When information is not available on the outcomes of many treatments, patients cannot evaluate the trade-offs among the options. Patients are particularly interested in how people fare who are similar to them based on clinical characteristics (e.g., for prostate cancer the PSA value, Gleason Score, presence of other comorbid conditions) or demographic characteristics (e.g., gender, age, race/ethnicity). Information that does exist on the outcomes of care is often not well organized or easily accessible and interpretable to patients. Rarely, if ever, is the information tailored to the characteristics of an individual patient.

In addition, there are gaps in the outcomes information that hinder patients’ and health professionals’ ability to predict the consequences of treatment and the trade-offs between different treatment options. For example, one type of treatment for a condition may have undergone rigorous evaluation (e.g., randomized clinical trial) while the other treatments may have had no thorough review to evaluate their benefits and risks. Randomized controlled clinical trials (RCTs) are expensive and complex undertakings, and a substantial number of medical treatments never have received this level of evaluation. Although RCTs are the gold standard for determining whether a treatment is efficacious, they do not provide information regarding the effectiveness of treatment (i.e., what outcomes are achieved when the average patient sees the average physician). Until recently, clinical trials focused only on clinical endpoints such as survival, ignoring other outcomes of keen interest to patients (e.g., quality-of-life, daily functioning).

Clinical trial results are limited in their utility for helping patients make individual treatment choices. Clinical trials typically compare a specific treatment versus doing nothing, rather than comparing two different treatment alternatives. Clinical trials also tend to enroll a homogenous population of patients who are not representative of the broader population of patients with the condition. For example, a new medication may only be tested in a clinical trial on white males with no other comorbid conditions. Once we know the effect of the treatment for this narrow population of white, otherwise healthy males, the question is, does the medication exhibit the same effects in women and/or persons of color and for those with
comorbidities (e.g., high blood pressure, diabetes)? Having information routinely collected on a large number of average people treated with the range of alternative treatments would allow continual examination of whether it is reasonable to assume that non-studied populations are likely to experience the same benefits of treatment as observed for patients in the controlled studies.

The Food and Drug Administration (FDA), which has regulatory oversight for drugs and medical devices, reviews the evidence from clinical trials and approves drugs deemed safe and efficacious. Once approved, the drugs and devices are nearly always applied in a much broader population of patients than were included in the clinical trials. Problems with drugs or devices that do not get identified during the clinical trial stage often are revealed through post-marketing surveillance when the treatment is applied to a much larger and more diverse patient population. During the post-marketing surveillance stage, problems with a device or drug are identified and reported by patients and their doctors—although there is no systematic longitudinal tracking system for all treatments to facilitate better understanding of the differential impact of treatments on different types of patients. No federal regulatory structure provides oversight on the safety or efficacy of surgery unless the surgical treatment involves the use of a device. Again, the development of longitudinal data bases that track the safety and effectiveness of various treatments would be of considerable use to patients who seek to understand whether a given treatment option has benefit for them and what side effects or complications they might experience by selecting a particular treatment course.

Another problem with research information derived from clinical trials—which capture an intervention at a specific point in time—is that it may not accurately reflect current medical practice. Technological changes, which may occur once the therapy is applied in general practice, can improve patients’ outcomes. Changes in the formulation or dosing of drugs may reduce side effects. Use of technologies in the hands of less-skilled providers can result in outcomes being worse. Research studies are frozen at the point in time they were conducted, and their findings may not generalize once the treatment is applied broadly in the population and evolves in response to the experience gained from widespread use. An outcomes data base could provide up-to-date information on current experiences of persons undergoing different treatment options.

Outcomes research has added significantly to our understanding about how the choices among treatments affect patients’ experiences in everyday life. The tools used to evaluate patient outcomes increasingly are being used in clinical research studies and by doctors in everyday practice. Unfortunately, we lack this type of information on more-
established therapies that came into use before studies started incorporating the new measures. Moreover, the relevant information is not readily accessible when tough decisions have to be made. Further, even in the newer studies, different surveys may be used, which may make it difficult or impossible to compare the results of different treatments. For example, a recent review identified nine different quality-of-life surveys that are used in studies of men with prostate cancer (Sommers and Ramsey, 1999). They found that the surveys covered different domains of health and emphasized different outcome dimensions; additionally, that no one survey had been applied to all treatments or used with men across the age spectrum with different stages of cancer. So, even physicians dedicated to giving their patients the best information on what to expect from one treatment versus another would not be able to assemble comparable information. Standardizing the information routinely collected about people undergoing treatment for a disease would substantially improve the ability of patients and physicians to make decisions about treatment options.

**Helping Patients Make the Right Treatment Choices for Themselves**

Patients want and need information that allows them to understand the consequences of initiating treatment for a particular clinical condition and also to weigh tradeoffs between different treatments when options exist—so that they can make the right treatment choice given their circumstances and preferences. In the future, patients will be increasingly challenged to select among alternative treatment options given the growth in federal investment in the National Institutes of Health for conducting research to identify new and better treatments across a broad array of medical conditions.

The field of outcomes research has underscored a critical point about medical management and decision-making: For many clinical conditions, there is no single treatment or approach that is appropriate for every patient. Rather, there are many situations in which the medical outcomes for alternative treatment approaches are comparable but involve different tradeoffs in terms of risks, recovery time, and individual preferences for expected side effects of treatment. As Wennberg and colleagues (1993) observe, “when the outcomes of a particular treatment are multiple and when more than one treatment option exists, an optimal treatment choice for individuals depends on the evaluations they give to the risks and benefits associated with the outcomes that matter to them.” Patients require outcomes information to help them evaluate risks and benefits associated with alternative treatments—however, this type of information is rarely available, organized, and presented in a way that patients can understand and use to inform their choice of treatment.
In 1989, the Agency for Health Care Policy and Research (AHCPR) funded the Patient Outcomes Research Teams (PORTs) to attempt to answer questions about the effectiveness and cost-effectiveness of available treatments for common clinical conditions (e.g., back pain, acute myocardial infarction, diabetes, prostate disease). The PORTs were to address the following questions: what works and at what cost; for which patients or subgroups of patients; when; why is there variation in the use of treatments, and what can be done to reduce variation (Freund et al., 1999). The PORTs were to use readily available data—observational in nature—to advance our understanding of outcomes of care as applied to patients in everyday practice. The PORT approach stood in contrast to the traditional approach of conducting randomized clinical trials (RCTs) to answer questions regarding whether one treatment is more effective than another. The use of observational rather than experimental data to advance our understanding of differences in patient outcomes from medical care, however, was not without controversy.

Peto and Baigent (1998) expressed concern regarding the limitations of using observational data for outcomes assessment, stating that the strength of RCTs rests in their ability to avoid the confounding that is associated with unmeasured initial differences among treatment cohorts. Randomization is designed to produce patient groups that are truly comparable with respect to known and unknown prognostic factors at baseline, whereas observational studies run the risk of not controlling for unrecognized confounding factors that may bias the results. Yet, a review by Benson and Hartz (2000), designed to compare differences between observational studies and RCTs, found little evidence that estimates of treatment effects in observational trials reported after 1984 were consistently larger than, or qualitatively different from, those obtained in RCTs.

Wennberg et al. (1993) countered that RCTs also have the potential for confounding, including a bias that occurs from failure to investigate the interdependency of therapeutic effects with patient preferences, placebo, and compliance. Not much is known about the capacity of RCTs to establish accurate outcome probabilities because the estimates obtained under randomization may not predict outcomes under open choice. It is not known whether patient preferences influence the effectiveness of treatments or outcomes—something that RCTs do not control for (McPherson et al., 1997). These researchers noted that probabilities for symptom reduction and other outcomes estimates produced under traditional RCTs (i.e., patients randomly assigned a treatment) versus an alternative study design, the Preference Clinical Trial or PCT (i.e., patients choose among all treatments after being
offered information about risk and benefits of conventional and experimental
treatments) decision-making would likely not be the same. They hypothesized that
expectations associated with active patient choice should result in better outcomes for all
treatments. The PCT is more observational in its approach to evaluating
outcomes—allowing patients to understand tradeoffs associated with all of the various
treatment options, letting them choose the treatment, and then following what happens
to people who choose different treatments. Wennberg and his colleagues emphasized
that researchers must identify all of the outcomes that matter to patients and then be
able to estimate accurately the probabilities that the various outcomes will occur, given
the treatment used and conditioned on patients’ comorbidities and severity of illness.
The proposed People Like Me approach to identifying the outcomes of interest to
patients and building data bases that help provide this information could focus attention
on which clinical trials to fund and which outcomes would need to be derived from
longitudinal tracking of patients in an observational data base.

To help patients understand the consequences of treatment, a variety of efforts
have been undertaken to develop and evaluate new approaches to involve patients in
making treatment decisions and self-management of their chronic illnesses. We
reviewed the literature on shared decision-making in the context of this project, with the
specific goal of eliciting key considerations that might affect the design of patient-
centered outcomes data bases (see Chapter 3). Shared decision-making tools remain
largely a “research” activity, and they have not been widely or systematically adopted in
the routine practice of medicine. Consumers are seeking out information to guide their
decisions, much of it from the Internet (Berland et al., 2001) and from patient support
groups that try to fill the needs of other patients like themselves. However, the locus of
most efforts of consumers to inform themselves and share health information with
others is peripheral to the physician-patient encounter, thus creating the potential for
individuals to receive conflicting information from clinicians and other sources.

At the same time that patients are tapping into new and diverse sources of health
information, many new evidence-based tools designed to inform clinicians and improve
decision-making have emerged and been absorbed into practice (e.g., Partin nomograms
for prostate cancer treatment). However, there is not yet a strategy that combines the
use of data to inform decisions made by patients and clinicians in a way that is both
relevant to patients’ experiences and preferences and clinically credible.
How to Fill the Information Gap

In 2000, AHRQ sponsored two expert meetings funded by the Kanter Family Foundation. The meetings focused on a discussion of the potential utility of developing data systems that would allow patients to share information with other patients about the decisions they had made when confronted with alternative treatment choices and the outcomes they experienced. In conceptualizing the data base, it was thought that combining the experiences of many patients could help them contribute to the development of an accurate picture of what happens to people like themselves, without compromising their own privacy. Moreover, it would promote the development of evidence-based tools and data systems to help clinicians and patients make informed decisions when faced with a similar array of valid choices.

From these meetings, the expert participants underscored that for any condition or disease, development of a system would require

- a data base of information from a large number of individuals with a given condition to permit analyses within demographic or other subcategories relevant to experiencing different outcomes (e.g., stage of disease, functional status)
- an effective user interface that would assist physicians and patients obtain answers to questions in real time.

One strategy for filling the information gaps is to develop national longitudinal outcomes data bases or registries that could be used to produce information for patients and their physicians to use in making treatment decisions. The information derived from such data bases would also help fill the information gap regarding where quality-of-care problems exist and identify opportunities for improving the safety and quality of care delivered to patients across the United States.

To generate information on outcomes from various treatments for a clinical condition, it would be necessary to collect a range of information, including immutable patient demographic characteristics, baseline prognostic factors, treatments the patient receives, and subsequent outcomes over many years from patients diagnosed with that condition. This is a considerable challenge both in terms of logistics and costs, and may be an appropriate strategy for certain clinical conditions. Recognizing that developing an outcomes data base may not be an appropriate strategy in many cases (because of feasibility and cost considerations), we start by defining a set of criteria that can be used as an initial screen to determine whether a given clinical condition is a suitable candidate for this type of investment (see Chapter 2).
Assessing the Need for and Feasibility of Establishing National Outcomes Data Bases

To understand what would be involved in establishing patient-centered outcomes data bases that could be used by patients and physicians, AHRQ contracted with RAND to assess the need for and feasibility of establishing outcomes data bases, using two clinical conditions as models, to explore the range of issues that need to be considered—size, scope, participation by patients and physicians, and cost. For the two conditions selected for review, the feasibility assessment would

• specify a framework to guide the development of data bases across a variety of diseases
• explore the need for creating outcomes data bases for two model conditions
• assess the readiness for implementation for the two model conditions
• evaluate the willingness of patients and providers to participate in a national outcomes data base and identify potential barriers to participation that would need to be addressed for the data base to succeed
• evaluate the feasibility and costs associated with initial development and ongoing maintenance of the two model data bases.

Specifically, RAND was asked to identify factors that could facilitate or impede the ongoing data collection in the process of routine patient care. This report summarizes the findings from our assessment.

The organization of the report reflects the major tasks that RAND was asked to perform. Because not all conditions may be suitable for creating a national outcomes data base, Chapter 2 presents a framework for selecting clinical conditions and describes the process used to conduct the assessment. For the purpose of this study, two conditions were selected to make concrete the challenges and opportunities that would be encountered in establishing national outcomes data bases for various clinical conditions. Chapter 3 contains a review of the literature on shared decision-making and, drawing from this work, highlights key design considerations for establishing national outcomes data bases. Chapter 4 provides a detailed assessment of the need for and feasibility of establishing a prostate cancer outcomes data base. Chapter 5 is dedicated to a similar assessment for osteoarthritis. Chapter 6 summarizes key factors that we have identified that must be considered and addressed to establish outcomes data bases, defines next steps, and makes recommendations regarding the development of patient-centered national outcomes data bases.