EXECUTIVE SUMMARY

Background

At some point in our lives, each of us will need medical care to treat an acute or chronic health condition. As patients, we frequently face difficult treatment decisions, needing to assess tradeoffs between various treatment options—such as whether to “do nothing” (i.e., watchful waiting), choose medication therapy, or have surgery. Patients increasingly are being encouraged to become more active participants in their care, including making decisions about treatment choices. However, for patients to be able to weigh tradeoffs and participate in decisions about their care, they must have access to information on the consequences or outcomes of different treatments. Information on the consequences or outcomes of various treatment options is generally not available to support informed decision-making by patients and their physicians.

Clinical trials often do not assess and compare the efficacy of various treatments for the same medical condition. And where clinical-trial data do exist, it is frequently difficult to use the results to help patients understand the outcomes they might actually experience. The reasons include the homogeneity of patients enrolled in clinical trials, the lack of consistent outcome measures, the failure to focus on outcomes of interest to patients, and results derived from interventions that do not reflect current or actual practice in most settings. When “outcomes” information is available, it usually is not readily accessible to patients, in a format that is easily understood, or tailored to address the unique needs of patients like themselves—namely, to address the question, what happens to “People Like Me” who choose this treatment?

The purpose of this research was to provide the Agency for Healthcare Research and Quality (AHRQ) with information that would contribute to an understanding of the need for and feasibility of establishing operational national outcomes data bases that could be used by patients and providers to make more informed treatment decisions. The “People Like Me” concept involves capturing longitudinal outcomes information on a wide range of patients undergoing various treatments for a select clinical condition. This information would then be packaged and made available for use by patients and physicians to understand what happens to People Like Me who choose a particular treatment and/or to compare treatments.
Study Objectives

This report summarizes the findings of RAND’s assessment of the need for and issues related to the feasibility of establishing two separate outcomes data bases—one for prostate cancer and another for surgical treatment for patients with osteoarthritis. The two conditions were selected to illustrate the range of challenges and opportunities that would be encountered in establishing national outcomes data bases for various clinical conditions.

The study focused on the following:

- Examining the need for outcomes data bases, as determined by a review of the research literature on shared decision-making and through structured discussions with patients who have the clinical condition and physicians who treat those patients
- Defining the set of questions patients and doctors want answered
- Identifying existing data bases that collect longitudinal outcomes data on patients who have the two conditions
- Assessing whether existing data bases address, either partly or fully, the questions of interest, and whether they provide a ready platform for use by patients and physicians in a People Like Me context
- Examining the lessons learned from those who have developed outcomes data bases, such as exploring recruitment of patients and physicians, selection bias, development and maintenance issues, and costs
- Defining key parameters that should be addressed in constructing the data base (e.g., number of patients required, how the data need to be stratified to answer questions more closely tailored to specific patients)
- Defining next steps and recommendations for AHRQ regarding the development of patient-centered national outcomes data bases.

Findings

Based on our review of prostate cancer and osteoarthritis as two conditions that illustrate the complexity of treatment choices faced by patients, we found a very strong desire among patients to have access to an information source that would not only explain what their condition is but would also help them understand what the various treatment options are and what outcomes (i.e., survival, functioning, side effects) people like themselves could expect from each of the various treatment options. Overwhelmingly, patients observed that information was difficult to obtain, was not synthesized and presented in a way to allow them to understand the trade-offs, and
rarely offered insights as to what their own experience might be given their unique characteristics (e.g., age, race/ethnicity, gender, health status, stage of disease). Patients with prostate cancer and osteoarthritis also described relying heavily on other patients for information in the absence of information that was presented from the patient’s perspective as to what they might experience—as well as on the Internet, where they acknowledged it was difficult to assess the accuracy of the information presented.

Perhaps because of their own difficulties in finding information, patients indicated they would be very willing to participate in a longitudinal outcomes database, particularly if they understood that it would benefit future patients in their quest for information. While patients wanted to ensure that their own personal data were protected, they did not see privacy issues as an obstacle to their participation. Patients expressed a preference for the federal government—such as the National Institutes of Health—or a nationally respected and trusted organization (e.g., American Cancer Society) to operate such a system because those groups would have no vested interest in any particular form of treatment and were more likely to present accurate information. Patients noted they would have reservations about participating if the project were sponsored and operated by a pharmaceutical company or medical device manufacturer that produces a treatment. Those firms might be less willing to provide objective information and/or might try to market products directly to patients participating in the data base project.

Providers also expressed considerable support for the People Like Me data base concept, particularly as it pertained to obtaining information on the clinical benefit of various therapies for different types of patients. They also seemed interested in finding ways to better organize and present information to help patients understand treatment options because they currently struggle with how best to communicate with patients given a wide range of patient preferences for information—from “you make the decision, doctor” to “I want to know all my choices and what is going to happen as a result of each choice.” Both providers and patients agreed that a national outcomes data base would provide an important tool for shared decision-making between patients and providers, something that is currently lacking.

Providers did express concern about the validity of the data base depending on how the sample of patients whose data make up the data base were chosen. They underscored the complexity of gaining representative participation by providers and patients in a voluntary data base that reflected a sample of patients with the condition—which could potentially lead to a biased sample. Physicians expressed some reservations about patients being able to view the outcomes information by themselves.
outside of the doctor-patient consultation; however, they acknowledged that the Internet has greatly transformed the discussions that doctors have with their patients, so that patients often come to the doctor armed with information they want to discuss. Physicians’ primary concern regarding the construction of the data base seemed to be with the accuracy and validity of the information in the system.

With respect to the feasibility of developing national outcomes data bases, it was clear from our research that efforts have already been made—both in the United States and abroad—to develop data collection systems that longitudinally track patient outcomes. Outside the United States, these more frequently consist of disease registries that capture 100 percent of the patients with a particular condition or treatment—typically within national health systems. The information derived from the longitudinal data base systems that we reviewed was viewed as extremely valuable for research purposes to understand how patients fare under different forms of treatment—especially as treatments evolve after the clinical trial stage. While not yet fully realizing their potential in this regard, the data bases were seen as a valuable tool to support clinicians in their interactions with patients. None of the projects that we reviewed had taken the next step of making the information available for direct use by patients, although the developers noted that this was an important audience for the information, and they were interested in finding ways to translate existing data for use by consumers and exploring how they might modify their data base efforts in the future to support greater shared decision-making between physicians and patients.

The existing longitudinal data base efforts demonstrate that establishing an outcomes data base is technically feasible and is valued by end users, but that substantial resources are required to design and operate them. The amount of resources required largely depends on the number of patients and providers who participate in the data base to produce statistically reliable results by different profiles of interest to patients (how many People Like Me demographic and clinical characteristics are accounted for), the amount of data required to be captured (how many data elements), the frequency and intensity of follow up efforts to track patients over time, the scope of the effort (how many conditions and interventions are being monitored), and the investment made to translate and present the results in a way that patients can understand and use to make informed choices. To establish and operate a national outcomes data base, the investment is likely to range from $5 to $25 million annually—with the costs determined by the factors noted above.

It is clear from our work that there is keen interest among patients to have a People Like Me resource, that providers support this activity, and that smaller-scale
efforts have been successfully designed and implemented. Prior to undertaking the establishment of a national outcomes data base for any medical condition, we recommend conducting meetings with patient and provider representatives to solicit their support for and input into the design of the system. While various health conditions share similar features, we did identify through the patient-provider discussions some unique factors across different conditions that would affect the design and implementation of the data base. The patient and provider meetings will be an essential step to firmly define the scope of the project, the goals of the data base, how data will be captured (which outcomes are of interest and how are they measured), how the data are analyzed, and how and what data will be shared with patients and providers.