Patients in Research: Not Just Subjects, But Partners

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Increasing attention is being paid to the issue of how to ensure that women have the same opportunity to be included in clinical research trials as do men (*1*). This issue is important in regard not only to the care that women who enter the trials receive but also to our ability to use the knowledge we gain from clinical trials to care for other women (*2*).

Yet, inclusion of women in clinical research trials is only one of the ways in which increased participation in research by patients is needed. It is time to reexamine the role of patients at each stage in the research process. Patients and researchers have different and complementary areas of expertise (*3*), and increased patient participation could lead to improvements in the relevance of research questions and in the appropriateness of recommendations arising from studies.

Patient Participation in Clinical Care

In the past, patient participation in research development has been limited. Clinical research, like medical care, has been based on the model of experts determining the best course of action with little input from those served. Though some may think that a meaningful partnership with patients in the research process is impossible, a great deal can be learned from the experience of patient participation in clinical care.

Over the past 15 years, the value of increasing the participation of patients in clinical care has been shown repeatedly. Across a range of diseases and chronic conditions, better patient-doctor communication has been shown to lead to better health outcomes for patients. For example, patients who receive more information about their care require less pain medication and spend fewer days in the hospital (*4*). Giving patients increased control of their treatment has led to further improvements in patient health, whereas the exercise of more control by doctors has had adverse affects (*5*, *6*). Specifically, teaching patients to understand their medical records and preparing them to become more involved in their own care contributes significantly to recovery (*7*). These improvements can be measured in laboratory tests as well as through patient reporting of decreased symptoms (*8*).

By decreasing the duration of hospitalization, patient involvement can lead to reduced inpatient costs as well as higher quality care. Costs can also be cut by involving patients in outpatient care. Outside the hospital, high blood pressure and high blood sugar are only two examples of conditions that when poorly controlled can lead to poor health and the need for expensive care. Both hypertension and diabetes are better controlled after brief interventions to increase the role individuals play in their medical care beyond that of a compliant patient (*9*). Furthermore, enlarging the role patients play has been shown to lower indirect costs, such as the cost of work days lost (*10*).

What Increased Patient Participation in Research Could Look Like

Like clinical care, medical research would be better able to help serve patients if patients were more involved in the research process, both at early stages when research questions are being formulated and at later stages when recommendations are made on the basis of research results.

In the past, patients or nonhealth professionals have been asked to represent patient views on institutional review boards. Although these initiatives are valuable, they have been limited in two important ways. First, patients often are asked to give a generalist's perspective. They review research about many health conditions they have not experienced. Second, patients have often been asked to approve or disapprove the use of human research subjects or the quality of informed consent for research projects that have already been designed. Patients' expertise has not been regularly called on at the stage when research questions are designed nor when the implications of completed research are reviewed.

At the earliest stages: determining what issues will be studied. As researchers, we could all improve the quality and relevance of our research by spending more time in the early stages of study design in talking with the people we hope our research will help. Often laboratory studies, social science surveys, or clinical trials are designed with the input of researchers only. This is because we recognize the expertise that researchers have. What we have failed to recognize is that patients have complementary expertise.

Researchers know more than most patients about how to design a sound study to answer a particular question. Researchers also build on their knowledge of physiology, anatomy, pathophysiology, biochemistry, and other sciences to develop proposals. At the same time, patients can draw on their experience of their illness and of past treatments to think of questions that need to be answered—questions that may not occur to most researchers.

Research would change in a number of ways if we spent more time listening to patients while we were designing studies. If patients' concerns were addressed, there would be more research on the side effects of treatments. In general, physicians underestimate patients' functional disabilities (*11*). Yet 98% of patients want to know what side effects a treatment will have (*12*). Patients' desire to know how treatments will affect the quality of their lives should be addressed in research. Reliable and valid measures of patients' experiences of physical, mental, emotional, and social functioning have been developed (*13*).

If patients were involved in the early stages of research design, the outcomes studied would be different. Physicians developing in vitro fertilization have studied the question of how many patients who made it to the stage of embryo transplants went on to have positive pregnancy tests. Although answering this question is valuable, it will not enable researchers to answer the question couples who are deciding whether to have in vitro fertilization often pose: If we try in vitro fertilization, what are our chances of giving birth to a baby? An embryo transplant depends on an embryo harvest, and that is not possible in all cases. Not everyone who has a positive pregnancy test is able to carry the fetus to term and give birth to a live baby. To answer those patients who want to know their chances of giving birth, a study would need, at the same time, to measure the probability that couples who enter in vitro fertilization programs will get pregnant and give birth to a live child.

More long-term research is needed to address patient concerns. Industrialized countries are now countries of chronic diseases. Major killers of the past, including infant mortality and acute infections, now kill far fewer in the United States than they did at the turn of the century. The major killers of today—heart disease and cancer—are chronic diseases, as are major causes of morbidity such as asthma and epilepsy. Not only has the decline in acute illnesses left chronic diseases as the major causes of death and

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disability, but the increased longevity of the population means that more people are living lengthy periods with chronic nonfatal ailments. Yet much research still focuses on short-term benefits and side effects of treatments. Research funding and institutions are not structured in a way that permits long-term research. Investigators usually need to show final results in 3 years, not 30, and funding agencies rarely support long-term investigations. Patients' lives work on a different time frame. Patients with chronic conditions often know that they will be living with a particular disease and its symptoms, treatment, and side effects for the rest of their lives. They need information about both immediate treatment choices and long-term consequences. Hearing patients' concerns about what questions need to be answered would help to balance the need for short- and long-term research.

At the end of research: how patients' perspectives may affect recommendations. Similarly, patient input at the final stages of research could significantly improve its usefulness. Recommendations based on study results are typically formulated by researchers and clinicians in discussions from which patients are absent. The absence of patients during the formulation of recommendations is particularly striking given the body of research that demonstrates that the value of a treatment often depends on the patient. Using analytic methods, researchers have demonstrated that in many instances there is no one treatment that is objectively better for all patients; the best treatment depends on the utility to the patient of different outcomes, the patient's values, and how the patient weighs the risks and benefits (*14*). In presenting treatment recommendations without incorporating the values of patients, researchers' values are inherently used to balance the risks and benefits of any treatment. In presenting uniform recommendations as opposed to information to assist patients in making decisions, investigators often inadvertently obscure the fact that the best treatment choice depends on the individual patient's values and preferences.

Although research results are generally presented in the absence of the patients they affect, there are exceptions. One exception is in the field of acquired immunodeficiency syndrome (AIDS) research. Unusual cases of unfair or inhumane treatment of researchers by protesters may have obscured the wide benefits of having patients present at many AIDS conferences. The presence of people living with AIDS at meetings discussing research leaves an indelible stamp on what takes place. There is a constant reminder of how the proposed research affects the lives of individuals. Their presence affects what subjects are proposed for study, the design of research proposals, interpretations, and policy recommendations made on the basis of the results.

In contrast, much recent tuberculosis research has been done without patient input. At one meeting, investigators and policymakers recommended requiring all people diagnosed with tuberculosis to have a professional present for each pill taken in treatment [directly observed therapy (DOT)]. The rationale was that the imposition on infected individuals would be trivial and the public health gains great. Yet, the details of the program were not spelled out. DOT can mean anything from a health care worker coming to the home of the patient twice a week to the person with tuberculosis being required to go to the doctor's office or clinic daily. For a mother with three children with no one to share their care, who lives an hour by bus from the clinic, the added burden of DOT could be quite substantial as well as unnecessary, given that most patients with tuberculosis do take their medications as instructed. DOT continues to be widely recommended as cost-effective, with little attention being paid to the costs borne by patients.

Patient participation at every stage. The beginning and end of research are only two examples of times when patient participation would be valuable. There are roles that patients can play at every stage of research. Clinical trials frequently need to address problems that lead to patients being unwilling to sign up for a study or to continue in a study once they have begun. Carey and Smith (*15*) have reported on an innovative and valuable use of patients in focus groups and a participatory advisory panel. Patients provided information that was important to the researchers' ability both to recruit study subjects and to obtain unbiased information.

Learning from Patients

Including patients as subjects in clinical trials should be only one of the ways in which patients have a chance to participate (*16*). Patients have a great deal to contribute to each stage of research, and their knowledge would complement researchers'. Together we could better define which research questions should be studied, determine what outcomes should be measured, encourage patient participation in adherence to protocols, and interpret what research results mean for the daily lives of patients. In the process of eliciting patients' viewpoints, it is important that no one person be asked to represent all patients. Patients' experiences and viewpoints vary as much from each other's as from researchers'. Patients' viewpoints can be elicited on a one-time basis in focus groups and on an ongoing basis through advisory panels.

Much can be learned from experiences over the past 15 years in clinical medicine. Patients have welcomed the opportunity to become more involved in their own clinical care. The quality of research would be greatly enriched and patients better served if they were welcomed as partners, not just subjects, in research.

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