Informed Adherence: The Need for Shared Medical Decision Making

William J. Sieber, PhD and Robert M. Kaplan, PhD
Health Outcomes Assessment Program, Department of Family and Preventive Medicine, University of California, San Diego, California

ABSTRACT: Screening tests for colon cancer, breast cancer, and elevated serum cholesterol are widely advocated and included in some practice guidelines. Failure to complete these tests is regarded as patient noncompliance. The purpose of this paper is to review noncompliance with screening tests from the perspective of a traditional biomedical model and an outcomes model. According to the traditional biomedical model, compliance with screening tests is necessary to identify disease at an early stage and to initiate treatment. In contrast, the outcomes model argues that patients and providers should engage in activities that maximize benefit from the patient’s perspective. Screening may lead to significant increases in case identification and in treatment rates. However, screening might also identify “pseudodisease,” defined as disease that is not clinically important. For some diagnostic tests, cases of pseudodisease far exceed cases of clinically meaningful disease. The outcomes model suggests shared medical decision making in which uncertainties surrounding tests are disclosed to patients. Using information about the risks and expected health outcomes of screening and treatment, patients can participate in decisions about their own health care. Control Clin Trials 2000;21:233S–240S © Elsevier Science Inc. 2000

KEY WORDS: Outcomes model, cancer screening, mammography, PSA, shared decision making

INTRODUCTION

Nearly all medical encounters end with advice and recommendations. Patients are advised to fill a prescription, take a medication, stay on a prescribed diet or give up cigarettes. Adherence is defined as the degree to which the patient follows this advice. The intent of attempts to increase adherence is to increase the likelihood that a goal (i.e., health) is achieved. We will argue that blind adherence to medical advice may help medical research answer an isolated question, but that understanding patients’ needs and respecting their ability to make informed decisions should be considered in clinical care as well as in controlled clinical trials.

Published figures suggest that nonadherence rates vary between 15 and 93%, depending on the patient population and definition of adherence. Most
studies suggest that at least a third of patients fail to adhere to treatment recommendations, while rates tend to be higher for patients with chronic conditions [1,2]. A large amount of literature suggests nonadherence results in adverse consequences for consumers of health care [1, 3, 4]. However, experimental studies in different areas of behavioral intervention and medical care show positive direct effects of adherence; that is, patients who adhere with treatment have better health outcomes regardless of whether they are on active treatment or placebo [5]. Patient active participation in choice of and commitment to treatment may be a crucial component of treatment efficacy [6, 7]. Incorporating the idea of increasing patient choice with the assumption that controlled clinical trials are the optimum vehicle for understanding the effects of a treatment presents quite a conundrum.

In this paper, we challenge the idea that nonadherence results in poorer patient outcomes. Using an outcomes model to better quantify the consequences of nonadherence is a first step in better understanding the role adherence plays in both clinical medicine and clinical research. If adherence is not shown to consistently produce better outcomes than nonadherence, continuing to exclude the patient in treatment decisions would be inexcusable. Shared decision making involving both patient and provider is the logical extension of patient-oriented health care and should be incorporated into both research and healthcare interventions.

BIOMEDICAL AND OUTCOMES MODELS

Health care has been dominated by a traditional biomedical model. According to this model, human pain and suffering are caused by disease processes. Disease activity is measured by judgments of trained physicians and by physiological measures, including blood chemistry and radiographic evidence of pathology. The purpose of medicine is to find disease pathology and to fix it. For problems such as high blood pressure, for example, the physician’s task is to diagnose the problem and administer a medicine that will make blood pressure normal. The measure of success is a blood pressure reading that falls within a defined range of normality. Treatment decisions involve the selection of the alternative that is indicated, and patient participation in decision making is regarded as unnecessary. Patients who do not use the indicated treatment are nonadherent. When the treatment fails to obtain the expected benefit, a common explanation is patient nonadherence.

An alternative model for health care known as the outcomes model is similar to the traditional biomedical model. However, the ultimate outcome is not a measure of disease process; the outcomes model emphasizes quality of life and life duration instead of clinical measures of disease process. For example, the outcomes model considers quality-adjusted life years (QALYs). An example of a measure of disease process might be tumor grade. Although tumor grade may affect QALYs, the relationship is far from perfect. There may be circumstances in which changes in tumor grade have no effect on life expectancy or quality of life. According to the outcomes model, little change has occurred. A clinical model would attend to changes in tumor characteristics. This approach takes a more holistic perspective toward health; improving the patient’s ability to function and live free of symptoms is more important than a biochemical
marker of disease. The implication for clinical trials is that general outcomes must be included in evaluations. In the outcomes model, patient preferences for health outcomes are central to the decision-making process. Instead of being labeled nonadherent, patients who do not use treatments as prescribed may be considered to have greater preference for alternative treatment or for no treatment at all. These preferences make sense if a treatment results in undesirable side effects or consequences.

COMPLIANCE, ADHERENCE, COOPERATION

The progression of terminology about adherence used over the past two decades reflects a growing trend toward more active involvement of patients in health-care decisions. While the term "adherence" is somewhat more neutral than "compliance," it still implies that the patients should stick with the advice given to them by a professional provider. Use of the term "cooperation" implies that the doctor and the patient are working together as a team. Positive consequences result from such cooperation in health care, supported by evidence from outcomes research. No matter the term used, a patient would be ill-advised to follow advice that leads to negative consequences.

There are many reasons why a patient may not adhere to a recommendation offered by a provider. For example, consumers have been led to believe that those with elevated cholesterol are likely to die of heart disease, while those with normal levels survive. However, there are many difficulties in the study results on which these beliefs are based. First, the national cholesterol education program recommended cholesterol screening tests for all Americans regardless of age, gender, or ethnicity. However, at the time the guidelines were issued the clinical trials supporting the policies were based exclusively on men and there was no specific evidence for women or children [8, 9]. A second concern is that many clinical trials consistently fail to demonstrate that any improvement in life expectancy results from cholesterol lowering; reductions of deaths from heart disease are offset by increases in deaths from other causes [8, 10, 11]. Although some recent secondary prevention trials have found a benefit for all cause mortality, meta-analysis of primary prevention trials do not show statistically significant benefits of statin drugs when all causes of death are considered [12].

A patient should be cautious about following recommendations from a provider if such recommendations vary widely between providers and this variability does not affect outcomes important to the patient. That is, one assumption of the traditional biomedical model is that medical technology enhances health outcomes and that a patient who does not use these technologies is engaging in self-punishment. A second assumption is that there is general agreement and consistency among providers about diagnosis and treatment, given the same data for a particular case. However, there is substantial evidence that reliability of diagnosis is low and that there is substantial variation by geographic region in the rate at which problems are diagnosed [13–17]. The Wennberg studies show that the treatments with which patients are asked to comply vary greatly across geographic areas. The rates of diagnosis, application of procedures, and rates of hospitalization by diagnosis vary remarkably across geographic locations. The treatments patients are asked to comply with vary
substantially depending on where the patient lives [18, 19]. In some areas patients are several times more likely to get treatment. Yet there is little evidence that those who receive more care achieve better patient outcomes. This contradicts the hypothesis that adherence ("Do what the doctor orders!") will result in better patient outcomes. This argument extends to the use of diagnostic tests [20].

According to the American Cancer Society, it is necessary to screen for cancers so that they can be detected early [21]. It is believed that there is a reservoir of undetected disease that might be eliminated through more aggressive and earlier interventions. Screening guidelines have been proposed, and those who fail to follow these guidelines are regarded as nonadherent. However, it is necessary to understand all possible consequences of screening. While finding out that a person has disease is likely viewed as a positive consequence, if there is no clear remedy for the disease or if the disease would be unlikely to shorten life or reduce quality of life, knowing one has disease will likely lead only to increased disruption in one's life (e.g., more medical procedures, increased anxiety). There can also be negative consequences if screening itself is painful or has other expenses and is likely to result in negative findings.

There are two examples that demonstrate these circumstances. Evidence suggests that women between the ages of 40 and 50 who begin getting regular mammograms have no greater likelihood of avoiding death from breast cancer than women who do not get screened regularly. However, as suggested by recent evidence, about one-third of these women will have a false positive test that requires additional evaluation. Among women who do not have breast cancer, an estimated 18.6% will undergo biopsy after ten mammograms and about 6% of those who have regular clinical breast exams, but do not have cancer, will undergo unnecessary and painful biopsies [22].

In the case of prostate cancer, a prostate specific antigen blood test is more likely to detect prostate cancer than a traditional digital rectal exam, thus increasing the likelihood of a biopsy and the identification of prostate cancer. Diagnosis of prostate cancer often leads to a radical prostatectomy (surgical removal of the prostate gland). However, the "fix" has consequences, often leaving the man incontinent and/or impotent [23]. Using patients' preferences for the possible outcomes of various treatments, little if anything is gained in regard to QALYs if prostatectomy is chosen [24]. These and similar results have led the American College of Physicians and other groups to suggest that patients be informed about the risks and benefits of cancer screening and make their own decisions [25, 26]. Adherence according to these recommendations is no longer a relevant term.

**SHARED DECISION MAKING**

The argument made in the previous section is that decisions about screening and options for treatment are very difficult and that if positive outcomes are uncertain or unlikely, the patient's preferences should be included in the decision-making process. Russell, in her controversial book *Educated Guesses*, concludes that educated guesses support current policy to screen for high cholesterol, prostate cancer, and cervical cancer [9]. However, there is equally good evidence supporting educated guesses that the tests should not be used or that
they should be used less often. Nevertheless, we refer to patients who elect not to use these tests as nonadherent.

Recommendations to increase patient involvement in treatment decisions have come from several groups. For example, Winawer et al. reviewed the literature on screening for colon and rectal cancer [27]. They found evidence suggesting that screening provides a small benefit for men and women older than 50 years of age. However, their review of the evidence did not support any specific approach to screening. They considered flexible sigmoidoscopy, fecal occult blood tests, colonoscopy, and barium enemas. Each of these approaches is associated with a profile of risks and benefits. Ultimately, the group recommended that the decision be made by an informed patient in collaboration with an impartial provider.

So what characteristics might need to be understood before we identify a particular instance of nonadherence as informed? First, the patient must have an adequate fund of information about the different treatment options including both the positive and negative possibilities of each option. Second, the patient must possess enough self-knowledge to know what he or she is capable of performing. This self-knowledge would include self-efficacy specific to the protocol, cultural beliefs about the cause and cure of the condition, as well as health beliefs that would lead to positive or negative attitudes toward the treatment procedures. Third, the patient must possess a certain scientific mindedness that would include the ability to perform hypothesis testing. That is, a patient must be able to organize a rational or reasonable approach to discover what is working and what is not working. Fourth, a patient must be fully informed and aware of the consequences of being wrong. Not following a specific medication regimen may be more fatal and dangerous than nonadherence to other medications with greater windows of nonfatal or serious error.

**CLINICAL TRIALS**

In clinical trials research, some issues should be considered. First, clinical trials should include generalized measures of health-related quality of life. Not only do we need to know whether or not medicines affect a biological process, we must also understand how medicines affect patients. Randomized trials can be used to estimate whether there is an overall treatment effect. Beyond this, we need to learn whether adherence to treatment (or to placebo) produces benefit from the patient's perspective.

A second important issue concerns preference for treatment. One problem in contemporary clinical trials is that many patients do not want to be randomized to experimental conditions. Some evidence suggests that choice may play an important role in patient outcomes [5]. Considerable evidence indicates that the choice to comply is associated with better patient outcomes independent of whether the choice was to take active drug or placebo [5]. The role of choice in determining patient outcomes is difficult to evaluate. The best information about treatment effects comes from randomized clinical trials. Innovative experimental designs might help address this issue. In these designs, patients might be asked to choose between three options: (1) treatment, (2) waiting, or (3) being randomized to treatment or waiting. In these trials, the randomized arms of the trial estimate the pure treatment effect. The observational arms in the
trial provide estimates of the impact of preference. These designs may help us learn more about the role of preference in patient outcomes [28].

While the need for patient involvement is clear when providers are uncertain about the appropriate options in clinical care, how might this affect the conduct of clinical trials? When it comes to clinical care, involving the patient in ongoing treatment modifications seems reasonable, but what about a clinician-researcher who has been trained to strictly follow a treatment regimen to answer a hypothesis about the effectiveness of a medication, as in a randomized controlled trial? There may be certain things to keep in mind:

- Is the goal of the trial to assess efficacy or effectiveness? If the safety and efficacy of the medication has been established, is it not more important to better understand how patients actually implement the physician's recommendations and the implications of this, especially if it is less than perfect?
- Adequately measuring a reality that a clinical trial researcher cannot control (i.e., nonadherence) is preferable to ignoring the potential impact of nonadherence on the results obtained. Understanding the difficulties in administering a treatment is valuable, whereas underestimating the effects of a medication due to the assumption that adherence was 100% may have significant, negative consequences for any individual patient. In other words, a fully adherent patient may be taking more medication than the average patient in the clinical trials on which dosing recommendations are based.
- What are some ways we can improve the validity of assessing what the subject is actually doing? First, we must work on decreasing the social desirability of the measures we use. Second, we can train research staff to be sensitive to patients' communications that may suggest adherence problems. Third, we must convey an attitude of assessing the individual rather than a rigid preoccupation with sticking to a demanding research protocol.

CONCLUSION

Fully understanding nonadherence requires the ability to explore different perspectives. While previous opinions about nonadherence have assumed a medical model that views the patient as a passive recipient of medical technology, we prefer an outcomes-oriented model that includes the patient's values and preferences in the treatment decision-making process. There are typically several therapeutic options, and patients must participate in decisions that will maximize benefits for themselves. Shared decisions involving both patients and providers may have the highest likelihood of improving health outcomes.

Several examples have hopefully clarified why some patients should be cautious in blindly adhering to medical advice, and we have tried to identify why "informed adherence" may occur. Greater emphasis should be placed on analyzing outcomes based on level of adherence, as well as conducting trials that test the impact of patient preference on both adherence and outcomes.

This work was supported by a grant to Dr. Kaplan from the American Cancer Society and by
grant R01 HS 09170 from the Agency for Health Care Policy and Research. Funding for the conference from which this paper evolved and for postconference publication was provided by Merck & Co., Inc., National Institute on Aging (IR13AG16229-01), NIH Office of Behavioral and Social Science Research, and Wyeth-Ayerst Laboratories.

REFERENCES


