

Patients' Assessment of Outcomes: An Important Aspect of Pharmaceutical Care

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Talking to people who are taking medications to improve their lives is a basic aspect of pharmaceutical care. Is it necessary to ascertain the patient's perceptions of the effects of a medication? Can reliable and valid information about therapeutic efficacy be obtained from laymen? What questions should be asked? The following thoughts on and experiences with these issues stem from my interactions with individuals suffering from heart failure.

Providers of pharmaceutical care, policymakers, third-party payers, and others have become increasingly more concerned with treatment outcomes for various reasons (1). Patients have always been concerned about outcomes. Indeed, the premise for taking a medication for a chronic illness is to improve one's daily life or to reduce deterioration and the frequency of adverse events such as acute decompensations and premature death. Ergo, a medication is therapeutically effective only if the patient can perceive a benefit in his or her daily life or if the treatment reduces the chances of deterioration and adverse events. How can therapeutic efficacy be assessed?

Medical science favors the use of objective outcome measures and discounts somewhat the patient's perceptions. The perspectives of health care providers are predicated on physiological models of

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heart failure that define the problem as cardiac output insufficient to meet metabolic needs or as exercise intolerance, particularly during physical activities. These medical conceptualizations of heart failure lead to the use of hemodynamic measures and exercise tolerance tests to evaluate the effects of therapy. The complexity of this syndrome offers many other pathophysiological targets and potential measures of efficacy. However, care givers must establish predictive relationships between objective measures and outcomes important to the patient, such as fewer limitations due to dyspnea and fatigue, fewer hospitalizations, and prolonged life (2). How much must the cardiac output, pulmonary capillary wedge pressure, left ventricular ejection fraction, exercise tolerance time, etc., improve before a patient with heart failure truly benefits from a medication? Numerous physiological variables have been associated with increased mortality and poor exercise tolerance (3, 4). However, physiological measures that accurately reflect the individual effects of medications for heart failure on outcomes important to the patients have not been established (5, 6). Furthermore, left ventricular dysfunction causes complex pathophysiological responses, making it unlikely that simple and strong predictive relationships will be found. The patient-oriented outcomes must, therefore, be assessed directly by subjective methods rather than inferred from disease-oriented objective measures.

Few, if any, medications are consistently and uniformly effective. Can responders and nonresponders be differentiated in terms of therapeutic outcomes? Health care providers must rely on controlled clinical trials to evaluate the chances that therapy will benefit an individual by preventing adverse outcomes. For example, in several studies of patients with heart failure, enalapril has been shown to reduce the mortality rate (7-9). However, the direct benefit to an individual who takes enalapril, with regard to the prevention of premature death, cannot be ascertained. Studies have also shown that the ejection fraction is one of the best predictors of mortality in patients with heart failure. Whether or not a change in the ejection fraction or any other prognostic variable is related to a change in the likelihood of survival also is not known. Enalapril does not increase the ejection fraction enough to expect an improvement in survival!

In the absence of surrogate measures that accurately predict the prevention of adverse outcomes, patients should be informed about the potential benefits that they cannot perceive. The goal(s) of a preventive therapy should be discussed. Each benefit must be summarized so patients can make personal cost-benefit evaluations. For example, saying that enalapril significantly increases the chances of survival is not very informative. A more useful summary would be that enalapril increases the chances of living for 2 years from 75% to 80%. Suggest that patients take the medication as long as the potential for benefit outweighs the negative aspects of taking the medication. Ask the patients about their views of the tradeoffs they are making by taking a medication.

How much a treatment improves an individual's daily life can be assessed, although historically this outcome has not been adequately addressed in studies of chronic heart failure. Many past efforts to involve the patient have attempted to use patient perceptions as substitutes for objective measures. The result was often poor congruence, much like physicians' subjective assessments, and a pessimistic view of subjective measures (10-13). Nevertheless, as previously stated, subjective measures are necessary to evaluate therapeutic efficacy. Health care providers seem to rely on their own subjective assessments. Patient beliefs and preferences are key determinants of patients' medication use and should be discussed (14).

Assessing therapeutic efficacy by asking the patients questions requires a systematic and unbiased effort. Specifically what should be asked? One definition of quality of life refers to living as one desires without limitations (15, 16). Questions may focus on limitations in general, only those caused by a chronic disease, or those that might be responsive to or caused by the effects of a particular medication. Evaluation efforts may rely on generic questions about quality of life or health status in an effort to allow comparisons across many aspects of care and multiple disease states (17). These broad comparisons can be made by using a standard response format and more specific questions (18). Less specific inquiries are susceptible to variations induced by factors unrelated to the disease or drug of interest. These other factors may mask the effects of pharmaceutical care. Questions should be specific and not be open

to numerous interpretations by the respondents. An inquiry such as, "Did this medication help you?" is not the best approach to find out if a particular medication is helping to control a particular disease. It is biased toward beneficial effects. The areas of an individual's life—physical activities, emotional concerns, social and recreational activities, costs, side effects, symptoms—that are potentially affected by the pharmaceutical care being evaluated should be considered. Multiple questions promote a more comprehensive and reliable evaluation. As providers of pharmaceutical care, pharmacists must decide what questions to ask and how to phrase neutral questions. Talking to patients about how an illness or medication has affected their lives can be very helpful. Specific goals of therapy should be identified for each patient, and these determinations should be followed up by asking about patient-oriented outcomes.

Merely asking if a beneficial or adverse effect has occurred is insufficient. The importance of each outcome to the patient must be ascertained. We often find that a salt-restricted diet is more bothersome to the patient with heart failure than any degree of peripheral edema. A hospitalization for pulmonary edema may or may not be more detrimental to an individual's quality of life than a low-salt diet. Many commonly used questions only establish the presence or absence of an effect without any values. Alternatively, the degree of a symptom is rated by the health care provider (e.g., mild, moderate, or severe) according to his or her values rather than the patient's (e.g., the New York Heart Association Classification of Physical Limitations). Care givers and patients often have different perspectives about the severity of the problem and the degree of improvement with therapy because professionals are more disease-oriented and are not as focused on the effects of the disease on patients' lives (19, 20).

Some questionnaires such as the Sickness Impact Profile have assigned values to limitations based on a consensus opinion of a group of patients (21). This questionnaire considers health-related work disability as the most valuable item, yet many patients with heart failure who are older and have limited chances of survival for five years place more value on other outcomes. Patients' values are variable, and it should not be assumed that patients' values agree

with professional values. Knowing a patient's values or preferences fosters an understanding of how the person taking the medication weighs various side effects, costs, and benefits associated with a medication. Talking about preferences also encourages a more balanced discussion about outcomes, without concentration on the most notable good or bad outcome.

Can patients provide reliable outcome assessments? Reliability refers to the consistency of responses when a condition is stable. As questionnaires are developed, the reliability of the scores is tested in various ways (22). For example, the Minnesota Living with Heart Failure Questionnaire has been shown to have high correlations between repeated baseline assessments in several multicenter studies of patients with stable heart failure (18). A high correlation indicates that the random noise and the effects of other variables are sufficiently low for the questionnaire to serve as an outcome measure for treatments of heart failure. Questions that are easy to understand and that focus the respondent's thoughts enhance the reliability of the information. Standardized instructions about what to consider and a specific time frame serve to put the respondent in a consistent frame of mind. Steps to limit the effects of the care giver and the environment on the patient's responses are necessary (23). Patients should be asked for their opinions before they are informed of professional evaluations or the results of tests. Patients should be told that their opinions are important. The care giver should avoid interruptions, which make many people reluctant to talk. Technical language can confuse people. Responses should not be suggested via wording, voice tone, or non-verbal communications. Most patients can provide reliable outcome assessments when they are given the opportunity and the information is gathered in a skilled manner.

The most formidable barrier to using patient assessments as outcome measures is lack of time. Care givers are very busy and do not like to enter into lengthy conversations with patients. Patients do not like to answer long questionnaires, especially if the questions do not seem relevant to the patients' interests. Performance of medical tests often consumes time. However, most patients with chronic diseases are very interested in discussing the rationale and effects of their care. If the care giver and the patient schedule time

during the initial visit to identify the patient's problems (as perceived by the patient) and the goals of therapy, then time and money can be saved by focusing the treatment and follow-up on mutually understood objectives. Brief questions that are specific and easy to interpret save time. A written questionnaire can be completed while patients wait. Perhaps some of the routine physiological assessments that are not particularly useful in deciding whether a treatment is warranted or deleterious could be replaced by standard questions. Important information is missed when care givers and patients do not take time to talk and to establish an open relationship. Through conversations with patients, providers of pharmaceutical care can recognize, and perhaps prevent, problems related to medications that consume time and money as undesirable outcomes.

Elicitation of patients' assessments of the outcomes related to pharmaceutical care is a necessary aspect of providing good care and of evaluating care. Patients' perceptions can contribute to the assessment of therapeutic efficacy when those perceptions are systematically collected with care to avoid biases. Providers of care should take the time to develop and apply skills and tools for making subjective assessments. The assessment of therapeutic efficacy remains in the realm of the art of pharmaceutical care.

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