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Measuring Outcomes in Oncology Treatment: The Importance of Patient-Centered Outcomes

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The quality of medical care long has been assessed in terms of patient outcomes. Traditional measures such as mortality and morbidity assess the unintended consequences of treatment in a very concrete, measurable way, while survival and recurrence rates similarly depict whether the treatment is achieving its intended goal. Practicing clinicians, however, know that the quality of the care received goes beyond whether the patient lives or dies and whether the cancer recurs or not. Treatment decisions have a very real impact on many aspects of the patient's life beyond those black-and-white outcomes. The problem remains that these concepts can be difficult to define and even more difficult to measure.

In "Crossing the Quality Chasm," the Institute of Medicine defined the six aims of quality healthcare. Care should be safe, effective, patient-centered, timely, efficient, and equitable.¹ This definition clearly illustrates the important role that the patient, including his or her preferences, values, and beliefs, must play in modern health care. The emergence of patient-centered outcomes as a distinct entity further highlights this important paradigm shift. It is no longer adequate to rely on objective data that reflect the providers' point of view. One also must develop concepts and measurement tools that more broadly and reliably capture factors that influence outcome and define health from the patient perspective. Patient-centered outcomes research is the study of the relationship of an observational event (outcome) and the treatment provided in the context of the patient.²

The paradigm shift is illustrated by the inclusion of patient-centered outcomes in many cooperative group trials.³ Measures such as quality of life and patient satisfaction are going

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to play an ever-increasingly prominent role in the assessment of the quality of health care that surgical oncologists provide. Familiarity with patient centered outcomes is imperative. Most surgical oncologists are familiar with the concept of quality of life; however, there are other types of patient-centered outcomes that can be addressed such as patient preference, decision regret, and patient satisfaction. The goal of this article is to provide a broad overview of patient-centered outcomes in oncologic research. It starts with an introduction to these different types of patient-centered outcomes. It then offers an overview of instrument selection and design. Finally, it provides examples of existing approaches to measurement and previously validated instruments for each type of patient-centered outcome.

TYPES OF PATIENT-CENTERED OUTCOMES

Patient Satisfaction

Patient satisfaction is the emotional or cognitive evaluation of a health encounter by a patient as defined by a set of events or experiences. This may be defined by the essential components of the encounter itself (ie, services rendered), or the manner or context in which they are delivered. These definitions highlight the fact that satisfaction is a highly subjective outcome to measure, but important nonetheless. In health care, too often it is forgotten that the patient is a consumer and his or her satisfaction with the health care system and treatment is important in his or her overall experience. Patient satisfaction, therefore, often is used as a tool to assess the quality of care of a health care delivery system, and develop and improve models for patient care. The basic domains assessed in patient satisfaction include: interpersonal aspects, technical quality, accessibility/availability continuity of care delivered, and patient convenience, setting, financial concerns, communication, efficacy, respect, time spent, and concern experienced during the health care encounter.^{4,5}

The interpersonal aspects of a health encounter encompass the direct contact between provider and patient. It may include issues of communication, sense of mutual respect, investment of time, or conveyance of personal concern for the patient on the part of the provider. Technical quality primarily refers to the specific proficiency in skill required in a practitioner and intelligence and qualifications of that provider as perceived by the patient. The measures of accessibility and its related endpoints of availability and convenience assess such issues as location (eg, distance traveled for most patients, relationship to public transportation), hours of operation, scheduling (eg, waiting times for an appointment date), accommodations for special needs (eg, teletypewriter [TTY], wheelchair accessible), and in-office waiting time. Continuity includes intraprovider consistency of care and the transfer of information from one provider to another. The setting refers to the physical design of the location of patient care, which can refer to aesthetics and accessibility.

Decision Regret

Regret has been defined as “the negative, cognitively-based emotion that we experience when realizing or imagining that our present situation would have been better had we acted differently.”⁶ Some scholars further divide regret into dissatisfaction with a decision and its associated outcome on one hand, and disappointment in an outcome caused by a feeling of

responsibility for the decision made on the other.⁷ The regret is seen as an emotional state of disappointment or sorrow in the former. Self-recrimination, exemplified in the latter, is experienced as the component of regret that describes the degree of responsibility the individual feels for the negative outcome experienced. Decision regret is important in patient-centered outcomes research, in that it can indicate a flaw in information transfer (ie, informed consent and patient education) and treatment decision in the health encounter. Moreover, regret at one point in the treatment encounter can affect future treatment decisions, and the perception of overall quality of life. This construct has been studied extensively in the context of consumer research but has been difficult to capture in health care outcomes, and especially difficult in oncology research in particular. As more medical and surgical treatment options become available and patient-centered healthcare becomes a priority, however, the role of the patient in the treatment decision process increases. This increased patient participation proportionally increases the opportunity for decision regret. In oncologic treatment, very few interventions are curative, and many can have short-term or lifelong morbidity associated with little or no change in overall mortality. One can measure the decision anxiety, degree of responsibility or patient participation, and differences pre- and post outcome in the level of measured regret after an intervention or treatment.⁷ Using decision regret measures in conjunction with generic quality-of-life or functional measures may provide a more robust description of the context of decision regret as it relates to outcomes and patient experience.

Patient Preference

Largely discussed in the context of multiple treatment options with equivalent risk–benefit profiles, patient preference is related closely to patient satisfaction and decision regret in outcomes research. It depends upon the assumption of full disclosure of all reasonable treatment options for a given patient in a comprehensible, meaningful way. Only if the patient truly understands his or her treatment options, can she or he form an informed preference for one treatment over other options. Therefore, the measurement of patient preference often is linked tightly to patient education, decision-making analysis including decision regret, and quality-of-life measures. One also can measure patient preference as it pertains to perceived quality of life while undergoing a particular treatment regimen.

Health-Related Quality of Life

Health-related quality of life (HRQOL) describes the experience of different domains of health as modified by both disease and treatment processes.⁸ HRQOL is conceptualized as the five different ways in which disease and treatment can impact well-being and quality of life: impairment, functional status, perception of health, social interactions, and duration of life. These constructs are general, abstract concepts that provide a framework for thinking about HRQOL. In order to actually measure HRQOL, more specific, concrete domains of functioning have been defined. These will be discussed under generic measures of HRQOL.

MEASURES

Patient-centered outcomes can be classified into two major categories: generic or disease-specific measures. There are advantages and disadvantages to each type of measure that

must be considered. Generic measures are comprehensive and assess the overall impact, independent of specific disease type, treatment, or patient population. Condition-specific measures tend to be more sensitive tools that are designed to capture symptoms that are specific to a given medical condition and the direct effect of a condition on an individual's quality of life. Although it is often ideal to include components of both a generic and disease-specific tool, complexity and cumbersome administration can limit the practicality of this approach. One can modify a generic measure for a specific condition, or add a condition-specific supplement to simplify the tool and expedite administration.

Once one determines the appropriate measurement tool for a particular question, secondary risk adjustment may be required. This includes predisease functional status, baseline functional status at the beginning of treatment, and comorbidities not directly related to the disease in question but which affect overall health and response to treatment. One also may measure disease severity itself for the diagnosis in question. Finally, demographics and psychosocial factors often are included, because they may impact the individual's response to disease or intervention, preference and decision-making, or perception of health.

Generic Versus Condition-Specific Measures

Generic measures are broad measures designed to capture the physical, psychological, and social aspects of health. Their design should allow researchers to assess common denominators that define the effect of treatments on health status and quality of life. Quantity of health refers to the objective measures historically used in outcomes research, which include mortality, morbidity, and survival. The quality of health can be captured by the seven domains of health, which have been defined to measure HRQOL. The seven domains of health are: physical, social, emotional, and cognitive functioning, pain, vitality, and overall well being.⁹ These measures are designed to be generic measures of quality of life as it pertains to health; however there are disease-specific questionnaires that may focus on more unique aspects of the domains as they relate to the specific disease process or treatment. As generic measures, however, there are several aspects of each that can be assessed broadly by most measurement tools. Physical functioning primarily refers to physical mobility and independence. Common aspects measured include fitness or physiologic health by clinical or objective measures, basic self-care activities (activities of daily living, ADLs), or more complex self-care activities (instrumental ADLs, IADLs). Social functioning measures often include social role functioning, community involvement, quality of interpersonal relationships, and coping capacity. Emotional functioning describes the range of affective well being, specifically positive and negative emotions and emotional stability. Cognitive functioning measures include memory, reasoning, and orientation to describe range of intellectual ability. Pain often is defined as the degree of debilitating physical discomfort as a sensation, not as a degree of physical functioning. Its measures often capture intensity, frequency, and duration. Vitality simply refers to the patient's general state of well being; however, unlike emotional functioning, it measures sleep quality and quantity, degree of restful sleep, and general energy level. Overall well being is distinct from vitality, social, or emotional functioning, in that it represents the patient's perception of the net balance of all aspects of health-related quality of life. This global measure often refers to the individual's sense of contentment, happiness, or general health status. All of

these domains combined generate the overall health-related quality of life. There are several well-validated tools used to assess part or all of these domains in general or in disease-specific terms.

The design of a generic measure of HRQOL requires several specific considerations.⁹ First, one must choose which domains of health to measure. This decision will affect the range of measure or the scope and spectrum of performance and change in health status. The measures must have clinical relevance, which provides a context and logic for measurement design and score interpretation. Each domain can be given more or less weight within the overall measurement tool, based on the desired emphasis of the tool. The goal is to design a sensitive (one that is able to capture differences in health status), reliable (one that is consistent and reproducible), and valid (one that measures the constructs it was designed to measure) tool. Finally, practical consideration requires design that allows the investigator to achieve his or her measurement goals while limiting the burden of administration for participants. If the tool is too brief, or administration is too burdensome, it can impact the quality and quantity of information obtained adversely.

Disease-specific measures are designed to focus on the most relevant domains for a particular condition. These measures are either objective (clinical) or subjective (experiential). Unlike generic measures, which look at global changes in health status, disease-specific measures are designed to measure responsiveness or small treatment effects on a particular condition. These tools usually are designed to maximize sensitivity and specificity and focus on signs, symptoms, function, and test results.

Existing Tools Versus Instrument Design

Several generic and condition-specific instruments have been designed and validated previously. It is usually preferable to use a previously validated tool when possible. If such a tool does not exist, it is important to carefully design and validate a new instrument through pilot testing before using it in a large study.

Existing Instruments to Measure Patient-Centered Outcomes

Many of the concepts defined previously naturally interrelate. It is not uncommon for studies that investigate the impact of an intervention on health-related quality of life to also include measures of patient satisfaction, decision regret, and patient preference. A description of the existing approach to measure each patient-centered outcome follows.

Patient satisfaction—The European Organization for Research and Treatment of Cancer (EORTC), an international research organization based in Brussels, Belgium, has served as the epicenter of clinical randomized controlled trials in Europe for over 40 years. The EORTC also has been influential in establishing widely validated quality-of-life measurement tools and tools for patient satisfaction.

The QLQ-INPATSAT32 questionnaire was developed by the EORTC to measure patient satisfaction with inpatient care.¹⁰ This tool was designed to measure the patient satisfaction with the following dimensions of the care experience: provider/support staff technical and interpersonal skills, information availability and provision, information exchange between

providers and patient, hospital access, waiting time, general comfort, and satisfaction. This tool was found to have excellent reliability and internal consistency, and was convenient for patients to complete (approximately 15 minutes). Results from the QLQ-PATSAT32 measuring patient satisfaction were compared with the QLQ-C30, a validated quality-of-life tool also developed by the EORTC for patients who have gastric and esophageal cancer. Patient satisfaction and quality of life were found to be independent patient-centered outcomes measuring different components of disease and healthcare. Patients were clearly able to separate their experience of the treatment encounter from their assessment of their quality of life.¹¹

Decision regret—Decision regret is rarely the sole outcome measured in oncology. It is often a component of patient satisfaction and decision-making assessments. A simple five-item decision regret scale was developed and has been validated for several treatment modalities including hormone replacement therapy, breast cancer surgery, adjuvant therapy for breast cancer, or treatment for prostate cancer.^{7,12} Decision regret was correlated with dissatisfaction with the decision, but it did not measure patient satisfaction with outcome. Waljee and colleagues¹³ recently published their study on patient satisfaction and provider trust after breast-conserving therapy. They utilized a shorter six-item patient satisfaction scale, along with the five-item decision regret scale described, a 15-item decisional conflict scale, and an 11-item physician trust scale. As in other studies, the previously mentioned areas were measured in the context of more traditional morbidity measures including postoperative complications, re-excisions, need for completion mastectomy, or poor cosmesis. In this study, dissatisfaction and decision regret correlated strongly with postoperative complications and patient distrust of surgeons. Their study also indicated that decision regret may be linked to preoperative patient education informing patient expectations and investment in the decision-making process. Patients who felt they had shared responsibility in the treatment decision also had outcomes that met with their range of expectations. Davidson and Goldenberg¹⁴ recently published their study investigating decision regret and quality of life in early prostate cancer. Their study also used a combination of quality of life measures, both generic and disease-specific (QLQ-C30, EORTC-PC), the previously described decision regret scale, and a five-item tool to measure the patient's assumed role in decision making. This study revealed that when tested at the time of diagnosis and then again at a significant time after completion of treatment (4 months in this study), decision regret was minimal and did not correlate with adverse outcome. This finding has been consistent with other similar studies in prostate cancer that measured regret up to 2 years after treatment.¹⁵ These studies underline the inter-related nature of patient-centered outcomes and the need for multiple measures to assess for similar but not necessarily associated endpoints.

Patient preference—The premise of patient preference measurement is “the formal measurement of the strength of the preference of patients for a specific treatment or the outcome of such treatment.”¹⁶ In order to compare across different disease states, a value or utility usually is attached to each based on its desirability.⁹ These utilities function as an adjustment factor for quality of life. In oncology, utilities and probabilities can be combined with length of survival in a single measure of the quantity and quality of life in the

respective treatment states. The expected value of each treatment strategy thus is expressed in terms of quality-adjusted life years (QALYs). Such preference weights are important for cost-effectiveness and other decision analyses.

The two most common methods of utility measurement include standard gamble and time trade off.^{17–19} The standard gamble method allows the patient to choose between a definite outcome, and a gamble defined as the probability of the best possible outcome (ie, optimal health) versus the probability of the worst possible outcome (ie, death). Time trade off refers to choosing an intervention that may decrease overall life expectancy with a trade off of higher quality of life during that shorter life span. A key component of study design is the identification of the cohort in which preference weighting will be assessed. There is debate about whether it is better to assess preference on a population basis or from patients with firsthand experience with a given condition or treatment.^{20,21} These groups are likely to assign different weights to various health states and will impact the study results.

Health-related quality of life—Health-related quality of life is perhaps the most frequently measured patient-centered outcome in clinical oncology. Several generic measures have been developed and validated over time. Commonly used tools include the 36-item Short Form Health Survey (SF-36), Sickness Impact Profile (SIP), and the Nottingham Health Profile (NHP).

The SF-36 is perhaps the best-known method of assessing of HRQOL.²² It covers the following six domains of health: physical/emotional/social functioning, pain, vitality, and overall well being. There are several different forms related to the SF-36. The SF-36 is so named because it is a shorter version of the longer 149-question Medical Outcomes Study Functioning and Well-Being Profile, the most comprehensive measure of HRQOL. Several shorter versions of the SF-36, most notably the SF-12, have been developed and validated.²³ The SF-36 and related tools were designed for generally healthy populations.

The SIP covers physical, emotional, cognitive, and social functioning, pain, and overall well being.²⁴ It is a more detailed tool designed to detect subtle changes over time or between groups. The strengths of the SIP are its sensitivity and high reliability; however it is a long and cumbersome tool, which makes it impractical in certain settings.²⁵ The NHP is a tool that covers the domains of physical, emotional, and social functioning, pain, and vitality.²⁶ It is intermediate in length compared with the prior two and has been proven to have high reliability and validity in a wide range of health conditions and severity, but lacks some of the sensitivity of the other two measurement tools.

The EORTC QLQ-C30 is perhaps the most common tool used in the oncologic literature to measure health-related quality of life (HRQOL). A recent review article looking at HRQOL, however, found that although the QLQ-C30 predominated as a single validated tool, there were as many studies that used unique unvalidated tools.²⁷ The EORTC has begun to expand its research to develop more disease-specific tools (eg, QLQ-PAN26 for pancreatic cancer) that can be used as an adjunct to the generic QLQ-C30.²⁸

SUMMARY

Patient-centered outcomes research has become an integral part of clinical oncologic research. Since the mid 1980s, there has been a clear paradigm shift toward patient-centered definitions of what constitutes quality healthcare and successful treatment. In surgical oncology, a cure is not always possible. Patient-centered outcomes, however, provide the tools to ensure the highest quality care is provided to prolong life in a meaningful manner as judged by patients.

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