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Disease-free survival with the absence of morbidity is the fundamental goal of therapy. Therefore, it is necessary to employ measures that reflect the achievement, or lack thereof, of such a fundamental goal.

While the literature abounds with information on the frequency and severity of single morbid sequelae (neuropsychologic, cardiac, endocrinologic, etc.), there has been very little attention paid to the occurrence of multiple morbidities in individual subjects. Consequently, an obvious challenge is to develop a system for the measurement of global morbidity burden or comprehensive health status. In constructing an instrument for the measurement of health-related quality of life (HRQL)- what health status is worth – there is an apparent logic to assembling an array of holistic health state descriptions to encompass all possible outcomes. This approach is constrained by the pragmatic problem of the sheer number of such possibilities and the enormity of the task of describing each separately and completely. Rather, we have adopted a different approach based on the identification of the most important core attributes (domains of health), each of which can be manifest as a wide range of functions (from normal to grossly morbid).

Within the realm of oncology, there is a rapidly growing recognition of the need to engage in such studies.[1] Indeed, the National Cancer Institute of Canada has mandated the inclusion of measurement of HRQL in all phase III clinical trials for which it provides funding support.[2] In the present review, the focus on cancer in children is justified by several considerations, although (at least in the industrialized world) fewer than 1% of cases of malignant disease occur in childhood and only one child in 450 will develop cancer before his or her fifteenth birthday. These issues have been identified by Bleyer[3] as follows:

1. Cancers in childhood rank third in terms of years of life affected (after cancer of the lung and breast).
2. Cancers in childhood rank second (behind breast cancer) in terms of years of potential life saved.
3. Children contribute approximately 20% of all cases to clinical trials in oncology in the U.S.
4. By one estimate, survivors of cancer in childhood may account for as many as 1 in 600 young adults by the year 2010, at least in North America.

The ever-increasing survival rates in pediatric oncology, presently approximating 70% overall, demand that close attention be paid to the medical price of cure, namely short-term toxicity and the late effects of therapy. Particular concern is devoted to the chronic outcome for long-term survivors, as reflected in measures of HRQL. Combination of economic and clinical data allows the derivation of information on cost-effectiveness that can be refined to a measurement of cost-utility by the further adjustment for quality of survival.

There are a variety of approaches to the assessment of HRQL.[4-6] These include both generic approaches (measures that apply to virtually any population and so cover a broad range of dimensions of health status) and specific approaches (measures that apply only to a particular disease; these measures include only the dimensions of health status relevant to that group of patients). This article focuses on a subcategory of the generic approaches to HRQL; the preference or utility-based measures. Preference-based measures have two major components: a) Detailed, descriptions of important health states being measured, and b) Preference scores of the decision-maker for each of the possible health states.

Health status measures typically are used for one of two major purposes: discrimination of the burden of illness within a population, or evaluation of change over time.[4,7] For discriminative purposes, desirable characteristics of a health status measure include reliability (stable scores in stable subjects) and validity (the measure captures what it is supposed to capture). For evaluative purposes, the desirable characteristics are reproducibility (the analog of reliability, stable scores among those whose health status has not changed), responsiveness (sensitivity to meaningful changes in health status), and validity. Among these, establishment of validity has proved to be a major challenge. In the main, validity may be classified into two forms.[8] Criterion validity is the comparability of the new measure with an existing measure that is considered to be a gold standard. Construct validity is the comparability of the new measure with an existing measure that is widely accepted, or the association of two new measures that were predicted to behave similarly. The functional elements of construct validity are convergence (between independent measures

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of the same trait) and discrimination (between measures of different traits). The term face validity is applied to informal assessment of a new measure as intuitively valid without appraisal of comparability with existing measures.

An important distinction to consider is whether a measure of health status is based on the construct of functional capacity or on the construct of performance (the level at which an individual chooses to function).[9] Measurement of functional status has been considered in detail by Nelson and colleagues.[10] These investigators consider that such measures should:

a. Produce reliable and valid data on a core set of functional dimensions e.g., physical, mental, and role function.
b. Fit easily into routine data collection activities normally performed in clinical practice.
c. Be applicable to a wide range of problems and diagnoses.
d. Possess a high degree of face validity and be judged by patients and clinicians as acceptable.
e. Yield easily interpretable scores.
f. Provide the practitioner with clinically useful information regarding patient, functional status.

Yet another critical construct consideration is whether measurement of health status is by self-assessment or is undertaken by another party. Systematic differences between measurements recorded by different observers are well described.[11] For purposes of measuring HRQL, it is the subject's own perception of health status that is most important. Accordingly, it is certainly worthwhile to attempt to obtain such self-assessments, recognizing that, for children, proxy assessments (by parents, health care professionals, etc.) may be more appropriate.

These and related issues formed the basis of an international workshop held in Canada in 1998 on the subject of health-related quality of life in children with cancer.[12] In this presentation, the results of studies conducted in children with cancer will be reviewed, with a focus on the use of the Health Utilities Index, a family of preference-based, generic measures of HRQL developed at McMaster University.[13]

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