What Have You Done For Me Lately? The Value Added by Health-Related Quality of Life Data in Clinical Trials

Abstract:
Over the past several decades there has been increased interest in assessing patients’ health-related quality of life (HRQL) to better understand the burden of disease, to screen and stratify patients for treatment, to demonstrate treatment efficacy, to facilitate regulatory approval of new therapies, to substantiate marketing claims, to raise consumer and clinician awareness, and to improve the quality of care in daily clinical practice. The use of HRQL outcomes is perhaps nowhere so potentially important as in the arena of clinical trials. In this setting, HRQL data can extend the evaluation of novel medical treatments and health care interventions to include not only cardinal clinical outcomes such as disease control and survival, but also the effects of treatment on patients’ self-reported physical and psychosocial symptoms and functioning. HRQL data can be particularly useful in clinical trials in which: (1) treatments for chronic diseases are being evaluated, where symptom control and maintenance or restoration of functioning are at issue, rather than cure; (2) the disease site is associated with a poor prognosis; (3) different treatment modalities are being compared, (4) treatments of differing intensity or duration are being compared; and (5) survival is expected to be equivalent (or only marginally superior), but HRQL outcomes are expected to differ significantly.

Many clinical trial-based HRQL studies add to our knowledge base by confirming common sense notions, and clinical experience and wisdom. Yet, if this was the only value added by HRQL investigations, the half-life of such efforts would probably be quite short. Importantly, HRQL studies can also make explicit the trade-offs associated with competing treatments or can even yield counterintuitive results. In this paper a number of examples drawn from the oncology field will be used to illustrate how HRQL data can contribute significantly to the evaluation of treatment efficacy, and in some cases add a unique perspective to such evaluations.