Competing Outcomes

Clinical practice guidelines must be outcome driven. Indeed, the validity of a guideline rests on showing that following recommendations will lead to projected health and cost outcomes.¹ The primary outcomes proposed for guideline use include survival, toxicity, health-related quality of life (QOL), and cost effectiveness.² Patient preferences have also been recognized as factor that must be considered in making guideline recommendations.³

This prescription seems fairly straightforward, and in many instances, determining which outcomes to apply is as well. Recommendations for adjuvant regimens typically use overall or disease-free survival, acceptable toxicity profiles, and costs falling into the accepted range as a constellation of outcomes used to judge whether a regimen should be included.

For many guideline users, treatment effectiveness—its impact on a major clinical outcome such as survival—is assumed to trump all other outcomes. In guidelines development, however, this is only true if that treatment's impact on other outcomes is believed to be minimal or inconsequential. In situations in which evaluating multiple outcomes points to opposing views of patient benefit, creating and following guideline recommendations can be problematic. From a purely clinical perspective, balancing effectiveness with toxicity can present difficult value judgments. For example, should adjuvant chemotherapy be recommended to elderly node-negative breast cancer patients; should allogeneic bone marrow procedures be recommended as first-line treatment in several diseases?

The NCCN Myeloid Growth Factor Guidelines present a vivid example of the difficulties of attempting to assess “competing” outcomes. The first question that arises for this supportive care algorithm is what is the appropriate effectiveness outcome for the colony stimulating factors? Experts generally agree that effectiveness must be measured in terms of patient rather than physiologic outcomes; therefore, restoring a normal white count is not an appropriate outcome. But the question still remains: which clinical measure? Must use of these agents improve overall survival, or are secondary clinical outcomes such as decreased incidents of febrile neutropenia or hospitalizations appropriate? (Although secondary from an effectiveness perspective,⁴ these may directly contribute to and cost outcomes QOL). Secondly, how is cost as an outcome factored into the balance?⁵ What cost level is acceptable? Must the agent save health care dollars to be justified?

The first step in answering these questions must be collecting outcomes data, which must be analyzed to gain an accurate picture of the outcomes of using these agents, and the impact of these outcomes on patient care. Lyman's article in this issue supplies essential data for understanding the NCCN myeloid growth factor guidelines recommendations. Notably, the review evaluates the economic and QOL outcomes as well as primary and secondary clinical outcomes. This article should be a valuable aid to guidelines users in understanding how usage recommendations were made.

References

1. Field MJ, Lohr KN, eds, for the Institute of Medicine. Clinical Practice Guidelines. Directions of a new program. Washington DC: National Academy Press;1990:58.
2. American Society of Oncology. Outcomes of cancer treatment for technology assessment and cancer treatment guidelines. [J Clin Oncol 1996;14:671-679.](#)
3. Nease RF, Kneeland T, O'Connor GT, et al. Variation in patient utilities for outcomes of the management of chronic stable angina. Implications for clinical practice guidelines. Ischemic Heart Disease Patient Outcomes Research Team. [JAMA 1995;273:1185-1190.](#)
4. Lee SJ, Earle C, Weeks JC. Outcomes research in oncology: history, conceptual framework, and trends in the literature. [J Natl Cancer Inst 2000;92:195-204.](#)
5. Eccles M, Mason J. How to develop cost-conscious guidelines. [Health Tech Assess 2001;5:16.](#)

By Rodger J. Winn, MD

Rodger J. Winn is the Editor-in-Chief of *JNCCN*. He is Clinical Consultant at the National Quality Forum, and his past positions include Associate Professor of Clinical Medicine at the University of Texas M. D. Anderson Cancer Center. Dr. Winn received his medical degree from Jefferson Medical College of Philadelphia. His post-graduate training includes an internship and residency at Jefferson Medical College, and he also completed a medical oncology fellowship at Memorial Sloan-Kettering Cancer Center in New York. He is board certified in internal medicine and holds subspecialty certification in oncology.