COMMENTARY

Efficacy and Cost-Effectiveness of Cancer Treatment: Rational Allocation of Resources Based on Decision Analysis

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Cancer care direct costs comprised 10.7% of all medical care costs in 1985 (*1*) and were estimated at $35 billion in 1990 (*2*). Oncology physician charges increased by approximately 17% annually in the 1980s (*3*), while the medical consumer price index as a whole increased annually by at least 10% (*4*). Although some costs have been reduced because of trends such as shorter hospital stays (*5*), cost has become a major issue in decisions regarding reimbursement for high-technology and high-cost procedures (*6*). The combination of shrinking resources and an aging population may force major changes in the delivery of routine cancer treatment (*7*) because the young, the elderly, the curable, and the incurable must compete for the same limited resources.

Many cancer treatments are, unfortunately, ineffective. Cancer mortality rates have not changed substantially in the past decade, despite a "War on Cancer" (*8,9*). Major strides have been made in the recognition of the need for effective palliative care (*10*), in the treatment of some cancers (e.g., cisplatin for testicular cancer and adjuvant therapy for early breast and colon cancers), and in the cure of leukemia with allogeneic bone marrow transplantation (*11*). Yet, chemotherapy results in durable response in only 4% of patients and substantially prolongs the life of only an additional 3% of patients with advanced cancers (*12*). Until now, the efficacy and cost-effectiveness of routine cancer treatment has not been a central issue. The number of medical research articles on cost or cost-effectiveness has risen exponentially in the past 5 years, but only a handful have addressed oncology-related issues (*13,14*). Oncologists have concentrated on the question: "Does the treatment cause a response?" Now, society asks, "Does it increase survival or quality of life enough to justify the dollars spent compared with alternative uses of the same money, such as treatment for hypertension, mammography screening, or smoking cessation?" (*15*). Currently, the lack of meaningful economic information about routine oncology treatment limits the debate to opinions without facts.

Health care rationing exists already, albeit informally, by ability to pay. Most parties agree that some form of ranking of procedures on the basis of effectiveness is necessary (*16*), and this ranking should be performed openly and in explicit detail (*17*). The national mandate to evaluate the effectiveness of health care (*18*), including cancer care, now incorporates cost. The Agency for Health Care Policy and Research established by Congress to evaluate medical care is establishing practice guidelines that include cost considerations for pneumonia, prostate hypertrophy, and cancer pain control.

Our goals were (*a*) to determine the number and quality of economic analysis and decision-analysis studies that assessed the cost-effectiveness of cancer treatment and (*b*) to determine the possibility of using such studies to allocate health care resources. We searched medical (MEDLINE, CANCERLIT, and PDQ) and health administration databases for studies of efficacy and cost-effectiveness of treatment. In this commentary, we describe the terms used in research on efficacy and cost-effectiveness (i.e., outcome analysis). We also provide an overview of available studies, review some selected studies in detail, list the critical elements for accurate decision analysis, and discuss what oncologists must know about this field and why.

Definition of Terms

Most of the terms used in effectiveness and cost-effectiveness research are not taught in medical school; they come instead from the fields of management and economics. All are designed to help us define a common term of comparison (e.g., dollars per year of life saved by an intervention), so that alternative uses of the same resources can be compared. We have tried to minimize jargon and offer working explanations of commonly used terms and examples in Table 1. The interested reader is referred to recent reviews of technology assessment (*19*), outcome

*See "Notes" section following "References."