Eur J Cancer, Vol. 29A, Suppl. 7, pp. S3–S5, 1993.
Printed in Great Britain

0959–8049/93 $6.00 + 0.00

Cost-Effectiveness Analyses in a Changing Health Care Environment: New Issues and Challenges

M.J. Strauss, G.C. Bleecker and A.B. Steinwald

Rising health care expenditures have fuelled the demand for more information about the impact of new medical products on costs and outcomes. Cost-effectiveness analysis (CEA) is one technique used to inform health care decisionmakers. Recent analyses show an increase in the number of CEAs published annually, although study quality remains unchanged. While advances in CEA methodology have been significant, further progress will require changes in the way we conduct basic clinical research, and a greater commitment by groups not usually involved in these analyses.
Eur J Cancer, Vol. 29A, Suppl. 7, pp. S3–S5, 1993.

INTRODUCTION

MEDICAL PRODUCT innovation during the last decade has created a dilemma for governments worldwide. New technologies have fostered important gains in patient care but, generally, have led to increased overall health care costs. As a result, there is now a greater focus on evaluating the impact of new technologies on costs and patient outcomes. Several national health financing agencies have incorporated cost-effectiveness analysis (CEA) into their basic determinations of whether new technologies warrant reimbursement. A variety of techniques have been used to evaluate, in combination, the economic and clinical impacts of medical technologies. This paper uses the term CEA as a general description for such methodologies.

These government agencies, as well as private insurers, use CEA in an attempt to obtain greater value for health care expenditures. This can be achieved not only by controlling costs but also be enhancing patient outcomes. Quality of life assessment, for example, can be used to evaluate the ultimate impact of new technologies on patient welfare, and to supplement traditional clinical efficacy and safety data. This necessarily includes subjective self-assessments of factors that are important to the patient, such as functional ability and overall well-being. Most agencies and payers agree that preserving life at great monetary expense with little benefit in quality of life is an inappropriate objective of medical technology development.

Combining the concepts of cost and outcome into a meaningful analysis that is useful for allocating health resources has proven to be a difficult undertaking. Although there is a consensus that more CEA research is needed, there has been limited focus on how to fund, develop and implement such studies, and how best to communicate results widely. Traditionally, medical products have been brought to the market through a collaborative relationship between manufacturers and academic researchers, who together evaluate product safety and efficacy. Government regulators and payers have relied heavily on this process, and on publication of results in peer-reviewed journals. The mandate to expand studies to include CEA, however, now demands that there be changes in this dynamic. New research methods, new academic disciplines and new and expanded journals must now be involved in the process. This paper briefly examines these issues.

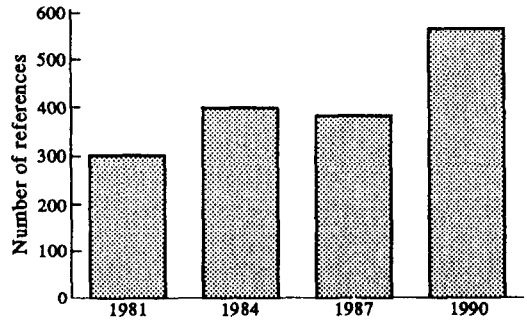

Fig. 1. Number of CEA and CBA references in the literature.

THE CURRENT STATE OF CEA— HOW WELL ARE WE DOING?

CEA is a focus of intense interest within both the medical and health policy communities. All the evidence suggests that the discipline is experiencing a period of rapid growth. Our review of the medical literature indicates that, in the past decade, the number of CEA and cost–benefit analysis (CBA) studies published annually has almost doubled, increasing from 313 in 1981 to 565 in 1990 (Fig. 1). Part of that increase reflects a rapid growth in the number of cost studies performed in conjunction with clinical trials (Fig. 2), a research area that many believe should grow even faster [1]. Interestingly, however, a detailed

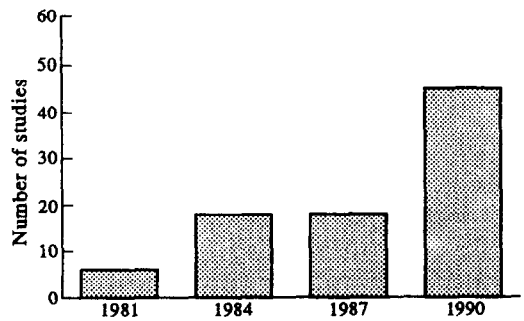

Fig. 2. Number of CEA and CBA studies cross-referenced with clinical trails.

Correspondence to M.J. Strauss.
The authors are at the Health Technology Associates, Inc., 555 Thirteenth Street N.W., Suite 700E, Washington, DC 20004, U.S.A.

review of a randomly selected sample of CEA studies found that the increase in the number of cost studies linked to clinical trials is due mostly to an increase in the number of clinical trials being performed [2]. Over the past two decades, the portion of trials that included an economic component stayed relatively constant, between 0.22 and 0.28%. In other words, we are undertaking more cost studies linked to clinical trials because we are undertaking more clinical trials.

While the number of CEAs published each year is increasing, their overall quality has remained relatively constant. Research from two different groups suggests that CEAs are of reasonable quality, but improvement is clearly warranted. Adams *et al.* found that study quality is highest in clinical trials initially designed to include a cost component as compared to those in which a cost analysis is added later [2]. Common problems that they found throughout the literature were that most studies fail to aggregate cost and outcome in a single analysis (that is, perform a true CEA), and most papers do not report sensitivity analyses. Similarly, Udvarhelyi *et al.* found that the quality of studies is fair, and that recent studies are no better than ones performed in the past [3]. They did find a clear difference across journals, however. The best studies are published in general medical journals. The quality is less impressive in the medical specialty journals, and worst in general surgery journals.

IMPLICATIONS OF THE NEW COST-EFFECTIVENESS MANDATE

Rising health care expenditures have helped fuel the demand for more information about the impact of new medical products on costs and outcomes. In response, researchers have expanded dramatically both the methodology and the focus of CEA. Yet for fundamental advances to occur, with global attention paid to the cost-effectiveness of virtually all medical products, we believe that there must be basic changes in the way clinical research is financed and conducted, and how results are communicated by journals and used by health care payers.

There must be greater acceptance of CEA among those who finance and conduct clinical research.

Medical product manufacturers and government agencies finance the majority of research on new medical products. While both groups clearly have begun to devote additional resources to CEA, funding remains inadequate. More studies are needed to fill the voids in cost and outcome information. One obvious explanation for the inadequacy lies in how manufacturers have harnessed resources to address CEA. Most corporate clinical research departments consider CEA to be an independent marketing responsibility. Only with great reluctance will most clinical research departments cooperate with marketing to incorporate CEA into their investigational efforts, making such research a "poor cousin" to basic clinical research.

Regulatory and reimbursement concerns have forced the medical product industry worldwide to invest in CEA. In contrast, most basic clinical researchers in medical specialty and subspecialty fields, particularly in the U.S.A., have failed to grasp the importance of CEA. They are often indifferent or even resistant to efforts to link CEA to clinical research, expressing concerns about the quality of research and patient care, and their belief that any such efforts would compromise that quality. In part, this is attributable to the fact that many clinical researchers have no real incentive to be concerned about CEA. Neither the National Institute of Health, the principal sponsor of U.S.A. clinical studies, nor most academic clinical departments, have made much commitment to this type of research.

Clinical study designs must be modified, as appropriate, to accommodate CEA studies.

CEAs are fundamentally different from clinical studies, and demand that researchers consider additional design and implementation issues. CEAs can be conducted using a variety of research designs. The most intensive studies collect data on all health care resource utilisation; other studies model the economic impact of a product. Similarly, outcome assessments can focus on specific treatment effects or more broadly examine a wide range of outcomes over a long period.

While many methodological issues have been discussed extensively in the literature [4], other basic and practical issues remain neglected, including: (a) the typical forms used to obtain patient consent to participate in a clinical trial are, in many cases, inadequate to collect cost information and must be modified, (b) sample size and power calculations must address the specific needs of the CEA design, (c) cost data are often skewed with high variance, dictating the need for large sample sizes when intensive data collection is planned, and (d) cost outcomes are rarely listed as primary or secondary endpoints in a trial.

CEAs employing decision analysis techniques can often be conducted with a subset of the patients enrolled in a clinical trial. For example, if a clinical study is looking at the effect of a treatment on the incidence of an undesirable and infrequent outcome, the sample size will be based on the need to demonstrate a difference between the two groups in the proportion experiencing the outcome. However, a cost study linked to the clinical trial may only need to prove a difference in mean costs between the two groups, which may require a smaller sample size.

In cancer research, linking cost studies to trials presents a huge opportunity for economic researchers. Because oncology probably leads all medical specialties in its ability to use randomised controlled trials to advance the field, economic researchers can test hypotheses that would otherwise require a modelling approach. Of course, incorporating economic endpoints into a cancer trial presents serious challenges as well. A trial may dictate that patients undergo testing and procedures that would otherwise not be performed during normal clinical practice, thereby leading to erroneous cost calculations. Similarly, seeking cost information during a trial may preclude one from pursuing some research designs. For example, crossover studies and other designs that use patients as their own controls generally prove to be inappropriate for CEAs.

Insurers and governments that demand CEA information must become partners in the process of developing that information.

Much of the motivation to perform CEAs stems from the need to meet the increasing scrutiny of insurers and government agencies that finance health care. Yet, to date, these groups have done little to clarify their needs and demands. For example, Medicare, the U.S. government program that finances care for the elderly and disabled, has indicated its intent to consider cost-effectiveness as a criterion for payment, but has provided only limited details about this initiative [5]. Additonally, both the Canadian province of Ontario and the national government of Australia are requiring CEAs before reimbursing new products, but neither government has provided enough guidance to the medical industry as to how CEA data will be analysed or how coverage decisions will be made.

The failure by payers to invest staff and resources to support CEA, and provide guidance to researchers is a major hinderance to further development of this research area. In the U.S.A., a

manufacturer planning a clinical study for product registration can meet with clinical, research design and statistical experts at the Food and Drug Administration to discuss study design issues and the factors needed to achieve regulatory approval. Unfortunately, analogous meetings with payers to discuss CEA issues are not possible because insurers and government agencies generally lack the expertise to provide consultation or guidance.

Journals must play an active, leadership role in helping to assess the cost impacts of medical technology.

For CEA to gain the credibility and exposure necessary for maximal impact on policy, peer-reviewed journals must increase the rate at which they publish CEA studies. Fortunately, the trend is in the right direction. Also encouraging is the fact that many of these studies (approximately 40%) now appear in medical specialty and subspecialty journals (i.e. not just health policy and general medicine journals), although there are very few such studies in surgical journals. To expand further the publication of CEA studies, journals must develop their own expertise in the field, seek appropriate referees for submitted articles, and encourage submission of both positive and negative studies, all while maintaining the usual high standards for publication.

CONCLUSIONS

Worldwide constraints in the financing of medical care have focused attention on the costs and effectiveness of medical technologies. Achieving a greater understanding of those concepts demands changes in the way we conduct basic clinical research and a greater commitment by groups not usually involved in cost-effectiveness analysis.

1. Drummond MF, Davis L. Economic analysis alongside clinical trials. *Int J Technol Assess Health Care* 1991, 7, 561–573.
2. Adams ME, McCall NT, Gray DT, Orza MJ, Chalmers TC. Economic analysis in randomized control trials. *Med Care* 1992, **30**, 231–243.
3. Udvarhelyi S, Colditz GA, Rai A, Epstein AM. *Ann Int Med* 1992, **116**, 238–244.
4. Bulpitt CJ, Astrid EF. Measuring costs and financial benefits in randomized controlled trials. *Am Heart J* 1990, **119**, 766–771.
5. U.S. Federal Register 1989, **54**, 4388–4389.