The Implications of Cost-Effectiveness Analysis of Medical Technology: Methodological Issues and Literature Review

September 1980

NTIS order #PB81-144602
Library of Congress Catalog Card Number 80-600160

For sale by the Superintendent of Documents, U.S. Government Printing Office
Washington, D.C. 20402
This volume is a background paper for OTA’S assessment, The Implications of Cost-Effectiveness Analysis of Medical Technology. That assessment analyzes the feasibility, implications, and usefulness of applying cost-effectiveness and cost-benefit analysis (CEA/CBA) in health care decisionmaking. The major, policy-oriented report of the assessment was published in August 1980. In addition to the main report, there will be five background papers: 1) the present document which addresses methodological issues and reviews the CEA/CBA literature, 2) a psychotherapy case study, 3) a diagnostic X-ray case study, 4) 17 other case studies of individual medical technologies, and 5) a review of international experience in managing medical technology. Another related report was published in September of 1979: A Review of Selected Federal Vaccine and Immunization Policies.

Background Paper #1: Methodological Issues and Literature Review, parts of which are based substantially on work done for OTA by Dr. Kenneth Warner of the University of Michigan, was prepared by OTA staff. In preparing this paper, OTA consulted with members of the advisory panel to the overall assessment; with the authors of the case studies prepared for the assessment; and with numerous other experts in economics, medicine, ethics, and health policy.

Drafts of this paper were reviewed by the advisory panel chaired by Dr. John Hogness, by the Health Program Advisory Committee chaired by Dr. Frederick Robbins, and by approximately 75 other individuals and groups representing a wide range of disciplines and perspectives. We are grateful for their assistance.

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Introduction and Background

With the desires to control costs, enhance quality, and improve access to health care has come the need to identify, and to understand what is meant by, cost-effective medical care. Two closely related evaluative techniques—cost-effectiveness analysis and cost-benefit analysis (CEA and CBA, respectively)—are being used or advocated with increasing frequency to address this need. As measured by contributions to the literature, professional interest in these techniques and in their findings grew exponentially through the past decade. A reading of this literature, combined with discussions with numerous individuals and groups, indicates considerable excitement, widespread confusion, and a growing caution about the methods, implications, and usefulness of CEA and CBA in health care.

As a result of these cost-related concerns and the growth of interest in CEA and CBA, OTA was asked by the Senate Committees on Labor and Human Resources and on Finance to examine the feasibility, usefulness, and implications of using cost-effectiveness information in decisions relating to medical technologies. The reviewing assessment, which includes this background paper on methodology, examines cost effectiveness by asking three major questions:

- What is the technical, or methodological validity of CEA and CBA when used to assess certain types of medical technologies within certain settings?
- What are the implications of using CEA or CBA? That is, what are the value and ethical, legal, political, medical, health, and economic implications?
- Can CEA and CBA be used appropriately in health care areas such as reimbursement, health planning, individual physician decisionmaking, or prepaid group practice?

The present background paper focuses on aspects of the first question. All three questions are examined in the main report of the assessment. (That main report and the four other background papers are described briefly in app. D.) This volume critically examines the methods of cost-benefit and cost-effectiveness analysis and reviews the literature on CEA and CBA in health care.

DEFINITIONS OF CEA AND CBA

The terms cost-effectiveness and cost-benefit analysis refer to formal analytical techniques for comparing the negative and positive consequences of alternative projects. Each of us engages in CEA/CBA-like thinking every day, frequently subconsciously. “And ultimately, something like (CEA/CBA) must necessarily be employed in any rational decision” (729).

In this report, the term CEA/CBA is used to refer to the class of techniques that includes both CEA and CBA. In practice, the comparison of costs and benefits is accomplished through a spectrum of approaches, ranging from sophisticated computer-based mathematical programming using large amounts of epidemiological and other data to partially intuitive, best-guess estimates of costs and benefits. Some analyses may take into account the results of clinical trials of a technology and model the technology’s effect on health outcomes. Others may assume that the alternative technologies under study have equal effectiveness and concentrate on the difference in costs involved.

Thus, there is a continuum of analyses that examine costs and benefits. One end of the continuum comprises what will be referred to as “net cost” studies. In net cost studies the emphasis is on costs, and such studies in the past have often assumed benefits or efficacy to be equal. At the other end of the continuum are
analyses that attempt to relate the use of technologies under study to specific health-related outcomes and to compare the costs of the technologies to the differential health benefits. Thus, CEA/CBA includes a set of analytical techniques, differentiated by the specific costs and benefits that are considered and the manner in which they are analyzed.

Both CEA and CBA require analysts to identify, measure, and compare all of the relevant costs and consequences of alternative means of addressing a given problem. The objective of CEA/CBA is to structure and analyze information in a manner that will inform and thereby assist policy makers. It is these individuals, and not analysts, who will decide which, if any, of the competing program or technological alternatives will be proposed or implemented.

The principal technical distinction between CEA and CBA lies in the valuation of the desirable consequences of programs. In CBA, all such consequences—benefits—are valued, like costs, in numerical terms, almost always dollars. Conceptually, therefore, CBA permits an assessment of the inherent worth of a program—Do the benefits exceed the costs?—as well as comparison of competing program alternatives—Which of several programs generates the largest excess of benefits over costs? With all costs and benefits measured in the same (monetary) unit, CBA is designed to allow comparisons of similar or of widely divergent types of programs. Thus, in theory at least, CBA might be used to decide whether certain public resources should be allocated to construction of a dam or to construction of a hospital.

In CEA, certain basic desirable consequences are not valued in monetary terms, but rather are measured in some other unit. In health care CEAs, common measures include years of life saved and days of morbidity or disability avoided. The reason for a nonmonetary measure of program effectiveness is either the impossibility or undesirability of valuing important outcomes in dollars and cents. Unlike the bottom line of a CBA, a CEA is not a net monetary value; rather, it is expressed in units such as “dollars per year of life saved.” CEA permits comparison of cost per unit of effectiveness among competing program alternatives designed to serve the same basic purpose. Unlike CBA, however, the technique does not allow comparison of programs having widely different objectives—because the effectiveness or outcome measures differ—nor does it permit assessment of the inherent worth of a program. Is a cost of $50,000 per year of life saved acceptable? Obviously, this last question requires a social and political judgment; it is not a technical matter.

Choice of CEA or CBA will depend on technical considerations, the predisposition of analysts and their clients, and on the type of question being addressed. Neither technique is necessarily superior to the other. CBA may be the theoretical ideal, since it permits direct comparison of the desirable and undesirable consequences of diverse programs, but problems of benefit valuation are myriad, particularly in social welfare areas such as health care. CEA avoids the methodologically difficult and morally ambiguous task of assigning monetary values to such nonmonetary measures as years of human life. By rejecting the monetary measure of CBA, however, the CEA analyst loses a unifying metric with which to weigh and compare different types of effectiveness. How are two programs to be compared when one program averts many deaths but has limited effect on disability, and the other prevents considerable disability but averts only a few deaths? Methodological advances (such as measures of quality-adjusted life years) may in time ameliorate this inadequacy, but considerable barriers to using CEA to evaluate programs with significantly different effects still remain.

Both CEA and CBA can be used for purposes of planning for the future or evaluating past program performance. As planning tools, the techniques involve prospective analysis, i.e., an attempt to predict the costs and benefits (or effectiveness) of alternative future programs. Analysis may draw on past or existing programs for data and ideas as to how to model the structure of the future programs, but the focus remains distinctly prospective. In addition, as evaluation tools, CEA and CBA involve retrospective assessment of the realized costs and
benefits (or effectiveness) of existing or past programs. Frequently, a retrospective evaluation will have a prospective or planning intent: The question is asked, should a program be continued into the future and, if so, how should it be modified?

Finally, in this brief introduction to CEA/CBA, it is useful to distinguish these techniques from others that are frequently confused with them. The two “sides” of a CEA or CBA—assessment of a program’s costs and desirable consequences—are important forms of analysis in their own right. The latter—assessment of effectiveness—is traditionally the focal point of evaluation in health care. A wide variety of evaluative approaches, including randomized clinical trials and epidemiological studies, form the basis of assessment of the efficacy or effectiveness of numerous medical and public health practices (405). Similarly, though less common-

HISTORY OF CEA/CBA

The common-sense principles of CEA/CBA have been promoted for centuries. Formal application of CEA/CBA, however, is a phenomenon of the present century. In 1902, the River and Harbor Act directed the Corps of Engineers to assess the costs and benefits of river and harbor projects. In 1936, the Federal Flood Control Act required that “the benefits (of projects) to whomsoever they may accrue must be in excess of the estimated costs,” though the Act provided no guidance as to how benefits and costs were to be defined and measured. In the same decade, both the Tennessee Valley Authority and the Department of Agriculture implemented program budgeting systems which included rudimentary attempts at formal CEA/CBA. Official Government criteria for appraisal of river development projects were first enunciated by the Bureau of the Budget in 1952 (753).

Early in the Kennedy administration, the Defense Department, under Secretary McNamara, adopted a program budgeting system which employed CEA/CBA to evaluate alternative defense projects. Success in these endeavors, combined with a burgeoning Federal budget, led President Johnson in 1965 to require the implementation of planning-programing-budgeting (PPB) systems throughout the Federal bureaucracy. CEA/CBA represented both the spirit and the letter of the new initiative to rationalize Government resource allocation decisionmaking (475).

PPB met with mixed and limited success, reflecting a lack of resources to implement it effectively, political and bureaucratic opposition to it, and unrealistic expectations of its role and potential (729). The formal system did not survive for long, though many Washington observers believe it left a legacy of continuing improvement in the use of rational analysis in Government decisionmaking (748). And recently, the philosophy and logic of CEA/CBA and PPB have been reincarnated in the form of zero based budgeting.

As formal evaluative techniques, CEA/CBA assess public sector resource allocation decisions where conventional private sector techniques,
such as capital budgeting and return-on-investment analysis, will not suffice. Commonly, the inadequacy of conventional private sector techniques reflects the absence of a smoothly functioning market to allocate resources as desired, resulting from either technical problems or distributional considerations. The former motivated the early applications of CEA/CBA. An example is the provision of national defense, which does not occur in the private sector because national defense is what is known as a pure public good, defined as a good which, when provided for one individual, benefits all individuals, since no one can be excluded from receiving the benefits and since one person’s consumption of benefits does not reduce their availability for other people. It is impossible to “sell” national defense in a private marketplace, because consumers are aware that they will receive it free if it is provided for anyone else, and if they were to buy it themselves, they would be providing it free to everyone else. Therefore, national defense will exist only if it is supplied by the public sector.

Other sources of technical market failure are closely related to the “pure public good” problem. These include significant economies of scale—in e.g., decreasing average costs as the size of a project increases (e.g., a dam)—and externalities—loosely, costs or benefits experienced by other than the immediate decisionmaker (e.g., pollution of a downstream community’s water supply by a firm dumping waste material upstream). This report will not elaborate on these sources of market failure, but merely emphasize that they require nonmarket decisionmaking and hence provide candidates for CEA/CBA (753).

HEALTH CARE AND CEA/CBA

In the period of a decade, society’s principal health system goal has shifted from increasing access to health care to controlling the rapidly inflating costs of care. The dilemma today is in containing costs without sacrificing desired benefits such as improved access to health care and quality. Thus, a logical approach would seem to be to identify and reduce the use of tests, procedures, and visits which are medically ineffective, unnecessary, or excessively expensive relative to their limited effectiveness. Increasing numbers of procedures and medical devices are being cited as candidates for attention as skepticism about the value of much medical technology replaces the enthusiasm of former years. Interest in and encouragement of the analysis of the safety and efficacy and, more recently, cost effectiveness of technology has accompanied the growing concern with health cost inflation (405).

Public efforts to improve financial access to care—primarily through medicare and medicaid—account for the rapid growth in Government’s share of the national health care bill. Combined with increasing depth and breadth of
private insurance coverage, social programs have reduced the linkage between receipt of health care services and financial liability for them. Third-party payment is particularly extensive in the highest cost component of the health care system—hospital care, where Government and private insurance pay over 90 percent of the total bill (720).

The consequence of the growth in third-party liability is that most medical resource consumption decisions are divorced from liability for their financial implications. Thus, a physician may order an additional lab test which has a very low probability of improving a diagnosis, but which will not impose any direct and immediate additional financial burden on the insured patient. Russell has observed that "... as third-party payment has increased over the years, the benefit required to justify a decision in the eyes of doctors and patients has declined. This has led to increased use of resources in many ways—including the introduction of technologies that otherwise might not have been adopted at all and, more often, the more rapid and extensive diffusion of technologies that had already been adopted to some extent." In some respects, the principal constraint on the provision of care is the state of the art and the availability of technology.

Thus, a number of factors have come together to create a perceived need for formal evaluation on the economic and medical implications of individual technologies: The market's ability to evaluate and ration has deteriorated to the point where it plays a minor role at best; as a Nation, we want to assure equitable access to needed medical care and to minimize care which is ineffective, unnecessary, harmful, or excessively costly; we want to contain health care costs which currently impose significant burdens on many citizens and whose continuing real growth threatens everyone; and we confront an array of new and future medical technologies that may be introduced into the practice of medicine with little regard for their cost implications (705). In this environment, attention has turned to non-market means of assessing and controlling medical resource allocation. CEA and CBA have been attracting interest as possible techniques for performing the assessment function.

GROWTH AND COMPOSITION OF THE HEALTH CARE CEA/CBA LITERATURE

Chapter 3 and, to a lesser extent, chapter 2 of this volume are based in large part on an extensive review of the health care CEA/CBA literature. A detailed descriptive analysis of the composition of that literature, including trends over time, is presented in appendix A. A portion of that analysis is presented here, however, as background for chapters 2 and 3.

A bibliography on CEA/CBA in health care is presented in appendix B. This bibliography contains approximately 600 references, Primarily from the years 1966 through 1978. OTA'S analysis of this literature shows a clear and dramatically growing interest in health care applications of CEA/CBA. As described in appendix A, each of the references was classified according to the following dimensions: 1) year of publication, 2) type of analysis (CEA or CBA), 3) publication audience, 4) medical function of the technology, 5) physical nature of the technology (drug, device, etc.), 6) decision orientation (e.g., societal perspective), and 7) subject matter. Only a minority of the bibliographic citations are actual

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CEA/CBAs. The majority address CEA/CBA or CEA/CBA-related issues. Several citations, however, are connected to CEA/CBA only through their titles; their actual content is either cost or effectiveness related alone.

Widespread interest in health care CEA/CBA seems to be a phenomenon of the 1970's. Figure 1 shows the growth in the numbers of published CEA/CBAs and the numbers of CEA/CBA-relevant articles. This growth has greatly surpassed the increase in the overall medical literature. Figure 2 shows that the growth has been especially rapid in medical journals as compared to nonmedical health care journals. This trend is suggestive of an increased economic consciousness on the part of physicians, but it does not allow any firm conclusion to that effect.

Prior to 1975, the annual number of CBAs generally exceeded the number of CEAS. The reverse has been true since then. The reasons for this shift are difficult to determine. Some discussion of possible explanations is included in appendix A, and an examination of the differences between CEA and CBA, and the implications of those differences, can be found in chapters 2 and 3.

Analysis of the literature by medical function of the technology under study shows that prevention and diagnosis each account for slightly over a quarter of the references, with treatment accounting for the remaining 44 percent. Recently, however, there has been a shift away from studies of prevention, which dominates the other two categories in the earlier years, and toward those of diagnostic technologies and treatment technologies. (See table 1.) In terms of decision orientation, health care CEA/CBAs retain as their principal orientation a societal perspective on problems, though studies with an individual practitioner orientation seem to be becoming increasingly common.

Given the strength of recent growth in the literature and social forces promoting future consciousness of cost effectiveness, OTA anticipates continued significant growth in the literature over the next several years, particularly in the medical literature. It is possible that the relative preference for CEA over CBA will increase.

<table>
<thead>
<tr>
<th>Year</th>
<th>Prevention</th>
<th>Diagnosis</th>
<th>Treatment</th>
<th>Other</th>
</tr>
</thead>
<tbody>
<tr>
<td>1966</td>
<td>0.0</td>
<td>0.0</td>
<td>0.0</td>
<td>5</td>
</tr>
<tr>
<td>1967</td>
<td>0.0</td>
<td>0.0</td>
<td>3.0</td>
<td>6</td>
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<td>1968</td>
<td>2.5</td>
<td>3.0</td>
<td>3.5</td>
<td>2</td>
</tr>
<tr>
<td>1969</td>
<td>1.5</td>
<td>0.5</td>
<td>2.0</td>
<td>2</td>
</tr>
<tr>
<td>1970</td>
<td>3.0</td>
<td>2.0</td>
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<td>1971</td>
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<td>4.0</td>
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<td>1973</td>
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<td>15</td>
</tr>
<tr>
<td>1974</td>
<td>2.5</td>
<td>5.0</td>
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<td>1977</td>
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</tr>
<tr>
<td>1978</td>
<td>18.0</td>
<td>25.5</td>
<td>18.5</td>
<td>31</td>
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<tr>
<td>Total</td>
<td>88.0</td>
<td>88.8</td>
<td>141.2</td>
<td>207</td>
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*Includes mixes of all three functions (prevention, diagnosis, and treatment). administration, general, and unknown

SOURCE: Office of Technology Assessment

Concern with the cost effectiveness of technology has motivated much of the recent CEA/CBA work in health care, and this motivating concern probably will persist for several years.

The single disease class that has captured the most attention in the literature is also the Nation's current leading cause of death: cardiovascular disease (CVD). More than two dozen papers in the bibliography concern CVD, and an additional 16 citations relate to hypertension screening and treatment. Other major disease problems have also received considerable attention. Cancer screening programs have been the subject of over 20 papers, including 9 on breast cancer screening (27,95,230,303,331,375,376), although cancer treatment per se has not received attention. Eighteen papers have addressed mental illness problems and programs (31,99,113,218,223,351,352,353,369,414,580), and 18 others pertain to dental care (49,59,75,126,127,187,229,245,283,331,390,518). Drug abuse (24,186,225,243,265,266,269,278,326,328,358,444,464,486) and alcoholism (259,268,281,474,532) combined account for a similar number of references. Renal disease, the subject of 18 papers, has received attention dispropor-

*Primary prevention of cancer also has received no attention in this literature, but many opportunities for primary prevention lie outside of the conventional personal health care system. Studies of the costs of air pollution, for example, and of the benefits of abatement do concern themselves with cancer prevention.
Figure 1.—Diffusion of Health Care CEA/CBAs by Year (1966-77)

Key
---
1. Number of CEAs + CBAs per year from column 3 of table A-2, app. A
2. CEAs + CBAs + related papers per year, from column 5 of table A-2 app. A

SOURCE Office of Technology Assessment
Figure 2.— Diffusion of Health Care CEA/CBAs in Medical and Nonmedical Health Care Journals by Year (1966-77)

Key: — CEA and CBAs in medical journals, per year (from column 1, table A-3, app. A)
- CEA and CBAs in nonmedical health care journals, per year (from column 3, table A-3, app. A)

SOURCE: Office of Technology Assessment
tionate to its prevalence, but reflective of the political and economic importance associated with public funding of dialysis (80,146,298,327,361,363,367,459,471,520). The Federal Government’s mid-1960’s interest in disease control programs, and in kidney disease in particular, made this the only disease problem to have more than one citation in the period prior to 1969.

Two general classes of health problems—communicable diseases and birth defects—have captured considerable attention. A variety of communicable diseases (including cholera, influenza, malaria, measles, polio, rubella, tuberculosis, and venereal disease) have been the subject of over two dozen papers (1,23,65,100,101,160,173,180,289,297,368,377,406,438,461,472,473,506). Since the detection and treatment of communicable disease have distinct “public goods” characteristics, they are logical subjects for CEA/CBA, and it is not to surprising to find that half of all the communicable disease papers date from before 1974. By contrast, another class of problems—the prevention of birth defects—has been studied much more in recent years, with only 2 of 15 papers predating 1974 (34,114,221,246,370). Several birth defect disease problems have received isolated attention (e.g., Down’s syndrome, spina bifida, Tay-Sachs disease), but at least one—phenylketonuria—has been the subject of three studies (78,517,553).

Several disease problems emerge in the guise of surgical procedures intended to treat them. Each of the following operations is the focal point of at least one reference in the bibliography: radical cystectomy (63), tonsillectomy (68), cholecystectomy (191), herniorrhaphy (222,394), appendectomy (398), synovectomy (416), joint replacement (534), and hysterectomy (103,275). In addition, there is a large number of papers relevant to surgery and CEA/CBA but not identifiable with a specific surgical procedure (3,39,40,74,214,231). Many of the surgery-related papers were contributions to a recent book on the subject (73).

Close to 30 papers were classified as nonspecific screening and prevention (43,87,105,106,107,109,133,157,158,227,239,309,320,362,428,455,458,478,484,489,497,535). Some of these related to particular activities (e.g., multiphasic screening (87,105,106,107)), while others discussed CEA/CBA issues more generally.

In recent years, a great deal of policy discussion and regulatory activity has concentrated on the adoption, diffusion, and use of expensive, sophisticated capital equipment. Thus, it was with considerable interest that OTA explored whether such equipment had been the focal point of numerous CEA/CBAs. With one exception—the computed tomography (CT) scanner—the answer is a striking no. The CT scanner was the most talked about medical technology of the 1970’s, and both the quantity and nature of the general interest are reflected in the CEA/CBA literature on CT. Some 18 citations are on this technology, all but 2 of them published in 1977 and 1978 (2,26,28,32,42,83,166,167,169,211,300,301,317,408,527,541,559,594).

Will other equipment-embodied technologies emerge as the subject of much attention in the literature? As the controversy on specific technologies grows, particularly related to their cost implications, additional CEA/CBA papers can be anticipated. Electronic fetal monitoring is an example of one such technology which has already been the subject of several papers (34,35,435,436). The work of the National Center for Health Care Technology, combined with general interest and concern, might increase the proportion of CEA/CBA literature focusing on equipment-embodied technologies.

A variety of services accounted for a significant proportion of the articles. Some of these services have relatively tangible outcomes and hence are good candidates for CEA/CBA. Six studies of pharmaceutical services basically involve issues of efficiency, with equity concerns of less importance (20,357,592,599). Aside from moral considerations, some studies related to reproductive health lend themselves to reason-
ably objective analysis. An obvious example, abortion, was the subject of only one paper during the period covered (338), though continued policy debate and development may lead to increased analytical interest (84). In addition, the literature review yielded several articles on family planning and on maternal and child health programs.

Other services address social needs that are extremely difficult to quantify in a meaningful fashion. In general, one would expect that such services would not receive a great deal of attention in literature which places a premium on quantification and measurement. Exceptions most likely would reflect a policy of unusual social importance. Above, it was noted that 18 CEA/CBA-relevant articles in the mental health area were found. Similarly, there were a dozen papers on geriatric services (148,149,286,319,568), and an additional four papers on institutional versus home care, with the patient type not indicated (121,321,348,486). Given current problems and anticipated growth in the elderly population, continued interest in this subject matter would not be surprising. Two other areas of considerable current interest are occupational health and rehabilitation. The literature search identified more than 10 articles on relevant topics (21,61,89,111,112,141,174,175,270,373,423,546).

Program services is not the only area in which social importance recommends analysis while quantification problems limit it. Manpower programs illustrate another area in which technical innovations—often, in this case, substitution of one type of personnel for another—produce outcomes which are difficult to quantify usefully. Nevertheless, analysts have made a dozen contributions on this subject (82,90,120,142,226,316,374,437).

Related to the dearth of equipment-specific studies, relatively few diagnostic procedures, apart from screening procedures, have been the subject of CEA/CBA attention. A few procedures have received isolated discussion—for example, fiberoptic laparoscopy and colonoscopy (224), sigmoidoscopy (581), and gastrointestinal exams (207)—but only radiology has received frequent attention (2,26,28,32,42,83,166,167,169,211,300,301,317,408,430,431,433,527,541,559,594). Weinstein (569) has identified the evaluation of diagnostic procedures as deserving of CEA/CBA efforts. His plea is supported by the growing body of literature which indict the increasing use of diagnostic tests as a major source of medical cost inflation (752). The evidence suggests that everyday, mundane tests are at least as significant contributors to that inflation as the more sophisticated and expensive technologies (745), yet the former have received very little CEA/CBA attention. Again, problems of measuring and valuing the outcomes of diagnostic procedures stand in the way of ready application of CEA/CBA (360,559).

In closing this section, two other areas which seem underrepresented in the literature should be mentioned. For the last several decades, drugs have epitomized the scientific growth of medicine and dramatically altered the practice and outcomes of health care. Drugs have been the subject of hundreds of biochemical and medical studies, and within the social sciences, of numerous analyses of medical technical change. Yet aside from implicit and tangential interest in them (e.g., as a component of hypertension management), drugs have not often been the subject of CEA/CBA analysis. (See, however, reference 190 for a case study on cimetidine.)

Finally, the literature reveals very little evidence of attempts to compare the costs and benefits (or effectiveness) of specific medical interventions with nonmedical interventions to deal with health problems. Although this background paper focuses on medical approaches, one might have anticipated identification in OTA’s literature search of a few studies which cross the medical-nonmedical border. With the exception of early Department of Health, Education, and Welfare efforts (240), however, studies of this type were not found. Conceivably, heightened awareness of prevention alternatives (743) will motivate formal efforts to grapple with medical-nonmedical comparisons in the future.
DECISION TECHNIQUES RELATED TO CEA/CBA

Other bodies of literature are related to the application of CEA/CBA in health care, but OTA, in the attempt to set reasonable bounds for this inquiry, did not systematically explore these. For example, more attention could have been given to the areas of decision analysis, multiple objective programing, and health status indexes (HSIs). In all three of these areas, there is a rich and growing health-related literature. In omitting them during our literature search, OTA did not identify some applications specifically related to CEA/CBA in health care. Each area will be considered briefly below.

Decision analysis is a collection of analytical methods used to assist in making decisions under uncertainty. This technique common ly uses the familiar decision tree diagram, depicting alternative decision pathways (or "branches") each of which is accompanied by a probability that a certain event will occur (335,558). Since CEA/CBA studies ordinarily include many uncertain variables, some element of decision analysis may often be desirable, as is discussed by Schweitzer (478). (For an excellent review of decision analytic application to health care, see reference 735.)

Multiple objective (or multiobjective) programing is another field of study whose application may be important to the use of CEA/CBA in the health field, but which has received relatively little attention partially because the general field itself is quite new. Multiobjective programing is a subfield of mathematical programing, which in turn lies within the discipline of operations research. For a comprehensive, but non-health-related, discussion of the subject, see Cohon, 1978 (719). Essentially, multiobjective programing consists of a set of analytical techniques, such as linear programing, which attempts to find solutions to a problem which has more than one objective. The rationale behind the use of these techniques is that many problems—especially public policy ones—which require decisions, have multiple objectives, many of which may be conflicting. That is, by achieving one objective, another objective must be sacrificed. The purpose of multiobjective programing in public policy decisionmaking is for the analyst to provide the decisionmaker with options and their probable consequences. As Cohon describes it (719), when a problem is solved for a single objective, and when there actually are multiple objectives, one of two undesirable events is apt to occur: Either some objectives are ignored, and therefore treated as if their value were zero; or the analyst, in an attempt to combine objectives, assigns relative weights to them. Either of these actions results in the analyst's actually making the key decisions which are supposed to be made by the decisionmaker. In multiobjective programing, by contrast, the analyst describes the degree to which each objective is or is not met as a result of each course of action. With the analytic process accomplished, decisionmakers are then faced with the political process of deciding which course of action to follow.

HSI research is the third field which is not fully assessed in this report. Nevertheless, a preliminary investigation suggests that the field is maturing in an orderly, scientific manner, that the body of literature is growing fairly rapidly, and that HSI research holds significant promise for future evaluation of all social and technological interventions which affect the public's health status.

The Federal agency that has been most interested in HSI research and application has been the National Center for Health Statistics (NCHS), whose job it is to monitor and report on the Nation's health and whose Health Interview Survey instrument has played a key role in much of the HSI work noted below. Located within NCHS is the Clearinghouse on Health Indexes, an office which maintains a current computerized and indexed bibliographic file on all health-index-related literature. Quarterly, the Clearinghouse publishes the "Bibliography on Health Indexes" which includes annotated citations of recent published and unpublished studies.

OTA's preliminary review of the HSI literature revealed several significant research
groups that are active in health status measurement. The San Diego group, working on the "Health Index Project," has done pioneering work in developing both survey instruments and relative weighting scales for physical health measures which include values for levels of well-being (functional status and symptom-problem indicators) and disease transitional probabilities (e.g., 726,731,732,733,741,742). Another group is working on the "Sickness Impact Profile (SIP)" study, which includes multiple weighting scales and one overall scale for physical and social health as well as general health perceptions (e.g., 707,708,728). A third group at RAND is developing multiple indexes, using an expanded definition of health, consisting of physical, mental, and social health and general health perceptions (79,711). This research effort is part of the RAND Health Insurance Study and will be used to help assess the impact that various insurance mechanisms have on health status.

OTA's discussion of the aforementioned research is not meant to indicate that other research in the area of HSIs is either not being done or not being done well.

ORGANIZATION OF THE REPORT

Chapter 2 examines issues relating to the methodology of CEA/CBA, essentially organized by the components of analysis. It is oriented less toward describing the methodology of CEA/CBA as conducted in practice than toward describing the components of an ideally conceived CEA/CBA process. Consequently, it should not be construed as a practical "cookbook" for use by someone wishing to learn how to "do" a CEA/CBA. Chapter 3 is a critical review of the published literature of CEA/CBAs. It describes how the components of CEA/CBA have been addressed in actual practice. Chapter 4 contains OTA's findings in regard to the methods of CEA/CBA. It also includes a set of 10 principles of analysis developed by OTA to guide an approach to formal analysis.

There are five appendixes. Appendix A is an analysis of the growth and composition of the health care CEA/CBA literature. Appendix B is a bibliography of CEA/CBA in health care. It includes a discussion of the criteria for inclusion of items in the bibliography. Appendix C is a collection of abstracts of items in the bibliography of appendix B, including the 19 case studies of medical technologies prepared as part of OTA's full assessment, *The Implications of Cost-Effectiveness Analysis of Medical Technology.* A brief description of the main report and other background papers of OTA's assessment is presented in appendix D. Appendix E lists the members of the OTA Health Program Advisory Committee and the authors of case studies conducted as part of this assessment.
2. Methodological Issues
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Applications of cost-effectiveness analysis/cost-benefit analysis (CEA/CBA) can be quite complex, especially in the field of health care. The effort to apply CEA/CBA, therefore, requires a systematic and often rigorous approach. The problem to be solved may not be obvious, nor may its objectives. Is the problem one of health? If so, what is its scope? Is the objective to reduce deaths? Or deaths due to cardiovascular disease? Or is the problem one of efficiency? Is it to determine the best way to find a cancerous lesion? Or to lose weight? The answers to questions such as these help to determine the scope and nature of the analysis.

The framework for the analysis is also partially determined by the perspective of the evaluator. In reviewing an insurance package to evaluate a preventive service for a client, for example, a private health insurer probably would limit his or her concern to a comparison of the costs of providing the preventive service with the projected decrease in costs due to a decrease in future medical care utilization. From the perspective of the private health insurer, therefore, the problem has to do with the efficiency of the preventive program. The perspective of society as a whole is a broader one. Society’s concerns would necessitate measuring not only the direct medical cost savings (if any), but also the indirect costs (such as lost (or saved) time associated with treatment, recovery, illness, or death) and the amount and value of life, limb, and misery involved. The benefits derived by the private sector, therefore, may be a subset of or different from the benefits derived by society as a whole.

In this chapter, the methodologies of both CEA and CBA are presented. First, the theoretically preferred orientation that a CEA/CBA should have is presented. Ordinarily, for example, a health problem rather than a given technology or procedure would be an “ideal” focus of an analysis, because this orientation will allow the analyst to study alternative means to achieve some specified health objective. Following a discussion of identifying, measuring, and valuing benefits/effectiveness and costs, the implications of—and approaches to—special problems that confront the analyst are discussed. These problems include valuing costs and benefits that occur over time, reducing uncertainty or making estimates in the face of uncertainty, and taking into account the concept of equity. Also discussed are alternative methods for presenting findings and interpreting the results of a CEA/CBA. Finally, the inherent limitations of the technique are identified.

Throughout this chapter, CEA and CBA are assumed to be fundamentally the same technique, in that both are structured methods that are designed to assist a decisionmaker in the allocation of resources. In actual practice, however, CBA attempts to measure all costs and benefits of a given process/technology and to value them in monetary terms, whereas CEA ordinarily attempts to measure and value the resources expended and to compare them to only health status changes. In CEA, therefore, the final product is usually presented in terms such as cost per life or cost per year of life saved, or cost per degree of blood pressure lowered, and so forth. Although this more limited approach to assessing the worth of a technology is often practical, especially when the decisionmaking setting is limited in scope, a broader approach to assessing the worth of a technology may be more applicable for general public policy. For instance, in the case of a county health department seeking to know the most cost-effective method of controlling alcoholism, measures such as the cost per cured/prevented alcoholic may be sufficient information on which to base a decision. However, at a higher public policy level, a local health systems agency, for example, may need to set priorities among such diverse projects as an alcoholism program, a
health education program, an immunization program, and a mobile coronary care program. Its decision process will require more as well as different types of information, such as the specific populations affected in each case, the relative changes in health status, future health care expenditures, reimbursement possibilities, and political acceptability. At the national level, even more diverse programs compete with one another, requiring more diverse information such as changes in productivity (e.g., for alcoholism programs), criminal activity (e.g., for drug programs), property loss, social security payments, health care expenditures as well as health status. Therefore, as the policy perspective broadens, the information requirements for resource allocation decisions also broaden.

This chapter describes the methods of CEA/CBA from a broad policy perspective, a perspective which Congress will ordinarily have. The principal departure from conventional wisdom is that OTA assumes that the use of CEA in public policy decisions—like that of CBA—ordinarily requires a comprehensive examination of all relevant costs and benefits. In addition, since CEA does not value all variables in a common (monetary) metric the way CBA does, it will be argued that the benefit/effectiveness part of a CEA can be analyzed in unlike terms (e.g., money saved, population groups treated, and disability days avoided). This approach has inherent limitations. First of all, CEA so broadly construed is contrary to what many analysts consider CEA to be. Second, with the results of a CEA presented as the cost per “array” of health and nonhealth benefits/effectiveness, such analysis will not permit easy comparisons between programs. As stated in chapter 1, however, the position taken by this report is that CEA and CBA are not decisionmaking techniques, but rather systematic methods to compare the costs and significant effects of a given course of action.

This expanded concept of CEA/CBA is not meant to imply that other more limited studies are not useful or valid, especially in resource allocation decisions of a more limited scope. As chapter 3 shows, many technically excellent analyses examine only the more important benefits/effectiveness. The purpose of the expanded definition of CEA is simply to place this analytical technique in a more general perspective, especially in light of its use in the public policy arena.

DEFINING THE PROBLEM, OBJECTIVES, AND ALTERNATIVES

Ideally, a health care CEA/CBA should start with a broadly defined health problem such as premature death, excessive disability, or unnecessary pain and suffering. A broad problem definition would lead to equally broad objectives—to reduce premature deaths, excessive disability, or unnecessary pain and suffering. Alternative means of achieving such broadly defined objectives are numerous, encompassing a wide scope of possible programs. In theory, the programs need not be limited to the field of health care per se; alternative means of enhancing health include airport safety procedures and environmental pollution control, as well as immunization programs and surgical interventions.

In fact, the ultimate health care CEA/CBA would provide guidance for society’s allocation of money in order to maximize the society’s health status. An approach this broad, however, would require not only technical sophistication beyond that of the current methods, but also the ability to shift funding among widely divergent programs, such as immunization activities, cancer research, emergency treatment systems, automobile safety, and fire and police protection.

Many public and private health-related programs in our pluralistic society are, in effect, fairly autonomous, however, and shifting funds from one program to another is difficult. In the real world, therefore, the identifiable health problem addressed through analysis may be more realistically limited to deaths or disability due to a given disease. Limiting the scope of the problem, in turn, limits the objectives and nar-
rows the alternatives. For instance, the problem that is defined may be confined to a given disease and the objective to that of reducing the resulting deaths and disability. Possible alternate actions include preventing the disease as well as curing or ameliorating it, but a still narrower definition of the problem—such as that of curing existing disease—would preclude prevention. However, as the scope narrows, the alternative possibilities can often be examined in greater depth, a counterbalancing advantage.

As a general rule, when an analyst examines a variety of alternatives, time and other limited resources preclude consideration of all or even many viable specific programmatic alternatives. In such cases, a possible strategy is to select representatives of diverse programs (e.g., treatment, screening, medical prevention, nonmedical prevention). Needless to say, the analyst should select programs not clearly dominated by others of the same type. Thus, a comparison of kidney disease treatment with prevention programs would not fairly represent the treatment end of the spectrum by selecting only inpatient dialysis, since both outpatient dialysis and home dialysis are viable options.

A CEA/CBA can also start from an entirely different focus. For instance, instead of examining alternative means of ameliorating a societal health problem, the analysis may consider the ability of a given technology to accomplish specific objectives. That is, rather than addressing the general problems of excess mortality or even mortality due to heart attacks, either of which could include solutions outside the scope of the health care system, the analysis may examine the cost of a mobile coronary care unit relative to its ability to reduce mortality. This reduction in scope may have the advantage of allowing more detail, and possibly more rigor in the analysis, but it sacrifices the ability to consider relevant alternatives in solving the overall health problem.

A particularly perplexing problem for CEA/CBAs concerns technologies in which a cause-and-effect relationship to health status is not established, although an association is widely believed. An obvious example is a diagnostic procedure (e.g., an X-ray). In the case of such technologies, objectives must often be defined in terms of intermediate outcomes (e.g., number of blood tests per minute or amount or quality of information produced). Analyses in which the objectives are so defined beg the question of the ultimate value of the diagnostic procedure, and may thereby call into question the value of the CEA/CBA itself.

For a CEA/CBA to be technically possible, the principal objectives should be quantifiable; when that is not possible, reasonable proxies should be available. This requirement places a severe constraint on the evaluation of health care procedures, the reason being that key objectives are often intangible. Unfortunately, such a constraint tends to place analysts and policymakers in a double bind, because health resource allocation decisions are often required irrespective of the ability to quantify objectives. When key objectives cannot be adequately measured, the temptation is to measure only the quantifiable objectives, relegating the intangibles to inconspicuous footnotes or ignoring them altogether. Thus, a hospice program which may be adopted in the absence of formal analysis, on the basis of the intangible benefits of dying with dignity and without pain, may be rejected under the scrutiny of a formal, rigorous CEA/CBA, based solely on economics—since the only quantifiable objective may be the reduction of health care cost.

The principal danger of performing a CEA/CBA when the important objectives are not quantifiable is that the results may be misrepresented or misinterpreted: That which is quantified may take on undue importance; that which is not, regardless of its importance, may be ignored. Thus, not only is it advantageous for an analysis to be premised originally on a health problem, but the important objectives or reasonable proxies should also be quantifiable. In structuring the analysis, therefore, it is important to array the objectives in priority at the outset, and then to analyze the quantifiability of each one.

There are at least two exceptions to the above statements. First, sometimes a minor objective by itself may indicate the desirability of a program even when the major objectives are not
quantifiable. In the case of the hospice program cited above, for example, although the principal purpose may be humanitarian in nature and thus impossible to quantify adequately, medical cost savings, although a much lesser consideration, may be sufficient to indicate adoption. Notice that this exception to the decision rule applies in only one direction: In the aforementioned case, the lack of medical cost savings would ordinarily not be sufficient in itself to indicate rejection. The second exception arises in the case of a CEA/CBA being performed from a nonsocial perspective. Often, this type of analysis has an orientation strictly toward minimizing health care costs; hence, the broader societal health objectives can be subordinated by a more narrow economic concern.

A final note concerns the overall responsibility of the analyst with respect to the scope of the study. On the one hand, it is helpful if the analysis provides information regarding the marshalling of resources in the most efficient manner conceivable, within the context of society’s overall principles, regardless of artificial constraints, whether they be legal, political, or customary in nature. Although some alternatives may not be feasible under the present legal structure, or may be politically or economically unacceptable, a thorough analysis might identify them (e.g., see reference 335). On the other hand, in the interest of realism, the analyst should (when feasible, and to the extent of the analyst’s knowledge) identify those alternatives that are politically, legally, and economically feasible at the present time. The broader scope is important for stressing what could be accomplished in the long term, but often only if society is willing to challenge some of its more established institutions. The narrower scope is important for stressing what can be accomplished in the short run, given the existing system.

**ASSESSING PRODUCTION RELATIONSHIPS**

Just as defining the problem, objectives, and alternatives is essential in establishing the overall conceptual framework for the analysis, defining the process of health care is essential in establishing the technical framework for the analysis. That is, in order to evaluate the worth of a health care technology, we must know the resources (people, money, equipment, supplies) which are used, the manner in which they are combined, and the outcome (the saving of life/limb, the increase in happiness, the decrease of pain or of number of hospital days) which is produced. Health outcome is ideally measured in terms of net changes in health status.

Often, there is more than one way to produce a given product. Take the relief of a headache as an example. The input might be a head and shoulder massage, an aspirin, or acupuncture. As evidenced by the last example (acupuncture), sometimes we know how to produce an output without actually understanding the precise manner in which it is produced. In most cases, different inputs and varying amounts and combinations of the inputs result in varying quantities and qualities of the output. Therefore, comparing one production process with another can be very complex.

In health care CEA/CBAs, production relationships are related to the problem which is defined. Narrowly defined problems with intermediate outcomes are the most easily characterized: The production of an X-ray or a blood test, for example, is reasonably well understood; the production of a change in health status is not. Efforts to assess the effectiveness of health care, therefore, have often avoided measuring changes in health status. Early efforts to evaluate health care measured the amount and quality of the inputs, implicitly assuming that more doctors and more nurses were better than fewer, that board-certified specialists were better than general practitioners, newer facilities were better than older, and that the latest technology was better than the existing technology. In 1969, Donabedian suggested that quality of care be measured in terms of structure, process, and outcome (723). Structure refers to the inputs, process refers to the manner in which health
care is practiced as defined by some norm (i.e., the manner in which inputs are combined), and outcome refers to the success of health care in terms of health status. Most of the subsequent success in evaluating health care has been in the area of “process” evaluation and has taken the form of peer review and medical audit, such as is employed by the Professional Standards Review Organizations. More recently, there has been encouraging work in measuring outcome in terms of health status changes (406, 78, 79, 516) which has been made possible by the pioneering work of several groups of researchers who are developing techniques that measure health status (708, 726, 741).

Part of the reason that the results of health care are so difficult to understand is that there are numerous intervening, or exogenous, variables which complicate the analysis—variables such as age of patient, degree of patient compliance, environmental changes, and genetics. As a result of these variables, analysts sometimes use elaborate mathematical models to try to simulate both the disease process and the production process (e.g., see reference 335). One of the more basic techniques currently in use is decision theory, a diagrammatical expression of probable outcomes. In some instances, when outputs of a process are known but the process by which they are produced is not well understood, analysts can make use of operations research techniques which mathematically manipulate the quantity of inputs and the manner in which they are combined to “simulate” the production and/or disease process. These methods are known generically as simulation techniques. Despite the technical aura of much of the terminology, simulation methods can be helpful in simplifying a complex process into what are believed to be the essential relationships. They can be used to examine the changing nature of the outputs as both inputs and the manner in which they are combined are varied.

To assist in public policy decisions, analysts may perform a CEA/CBA by studying a process retrospectively, and then extrapolating the results into the future. In such cases, certain potential complications should be noted. For example, many studies are done on a small scale, and the observed input-output relationships cannot necessarily be assumed to be the same on a large scale (as any baker knows, quadrupling the ingredients does not successfully produce a loaf of bread which is four times the normal size). An even more complex problem is that of the rate of technological change. To assume that a complex technological process will not change over time is obviously foolish, but to predict how it will change is fraught with uncertainty (e.g., see reference 559). Other problems include predicting the efficiency gained by learning better how to use a new technology, and predicting relative changes in future costs of inputs (e.g., labor v. capital). Finally, what works in one setting may not work in another. A technology applied in an urban setting may not work in a rural one; a carefully controlled study in a teaching hospital may demonstrate a technology’s efficacy—or potential effectiveness—but may not be useful in predicting its actual effectiveness outside that special setting (405).

Marginal Valuations

The worth of a technology should be assessed at what economists term “the margin.” That is, the analysis should seek to compare the added, or marginal, cost of producing the next unit of benefit (see reference 559). In an evaluation of computed tomography (CT) scanning, the issue is not any longer whether the technology itself is cost effective, but, rather, whether the various applications of the technology are cost effective. Should CT be used for confirming suspected brain disease/trauma, or for ruling out brain disease/trauma when persistent headaches are presented? In what instances are body scans indicated—or cost effective? In general, the relevant inputs or costs which must be considered in a CEA/CBA of a health care technology will be tied to whether the technology is already in place or whether it has yet to be adopted/purchased.

Joint Production Considerations

Finally, many technologies have multiple applications, and the technological process being studied is seldom applied in isolation. These two
considerations can have enormous effects on cost and benefit calculations. For instance, since a single blood test can be and is often used as a source of information for numerous diseases and bodily functions, analyzing the cost effectiveness of drawing blood for only one purpose is inadequate if the total cost is used; it either overstates the associated costs, understates the potential benefits, or both. Likewise, a CEA of a Pap smear program should be done in recognition of the fact that many other health evaluations are not only possible but are ordinarily performed during the examination, whether formally or informally. That is, the woman who is given a Pap test may be screened for other pelvic disorders, high blood pressure, fever, skin rashes, weight problems, wife battering, and many other conditions, all of which carry certain potential benefits and all of which should be assigned some of the cost (or, conversely, less cost should be assigned to the Pap test); or the CEA should be evaluating the complete ob-gyn examination rather than just a Pap test (335). Of course, including the effects of joint production adds greatly to the problems of measurement and valuation, but these difficulties in no way diminish the conceptual importance of their full consideration in a complete analysis. Often, for instance, a very small incremental, or marginal, increase in cost to an existing production process, can have large benefits—other times, the reverse is true.

Thus, in order for the health care production process to be adequately described, a causal relationship of inputs to outputs should be demonstrated, joint production effects should be considered, and the effects of exogenous variables should be examined. In addition, the analysis of the use of an existing technology should include marginal changes of costs and benefits. Unfortunately, many variables are much easier to describe conceptually than they are to measure empirically.

As mentioned earlier, however, since many diagnostic procedures have intermediate outcomes as their objectives, a direct association with health status change may not be known. In such cases, if inputs and outputs cannot be shown to be causally related, at least an associative relationship (however distant) to some health problem should be noted.

IDENTIFYING, MEASURING, AND VALUING BENEFITS/EFFECTIVENESS AND COSTS

At the heart of every CEA/CBA is the identification, measurement, and valuation of relevant costs and benefits/effectiveness associated with the production process. The identification and measurement procedures for both techniques are essentially the same; it is in the valuation process that the two techniques differ. The reader will recall that CBA ordinarily requires that all costs, effects, and benefits be valued in monetary terms, whereas CEA requires that only nonhealth status changes be so valued. But there are other differences between the two techniques.

One of the inherent difficulties in describing the elements of both CBA and CEA simultaneously—as is done in this report—is that, despite conceptual similarities of the two methodologies, details sometimes differ for technical reasons. The classification of costs and benefits/effectiveness is one example. It is convenient to look on “costs” as those resources which one must give up in order to gain some benefit or desired effect. Conversely, benefits are those resources which are gained from the expenditure of other resources used to produce them. These definitions hold for the “costs” of buying or implementing the technology being assessed and for the health “benefits” attributable to the technology. But what about the medical cost savings which may result? Are they benefits, or are they negative costs (i.e., to be subtracted from the technology’s cost)? The answer is either. In CBA, costs are generally considered to be only those costs directly associated with the technology being assessed (which includes the expenditure of “indirect” costs such as time and lost productivity). All changes in resources resulting
from those costs, including medical cost savings, are considered to be benefits, some of which are positive, some are negative. In CEA, on the other hand, generally all net medical/health resource changes are compared with all net health status changes (516), which requires that medical cost savings be treated as negative costs, rather than as benefits. In this report, for the convenience of exposition, medical cost reductions will be considered under the discussion of benefits.

In the following two sections, both benefits and costs will be discussed in terms of their identification, measurement, and valuation.

Benefits/Effectiveness
Identification

One primary advantage of a CEA/CBA is the requirement that all relevant aspects of the use of technology be considered explicitly. With respect to the identification of benefits, this implies that the analyst may look beyond the obvious, beyond that which is intended, sometimes so far beyond that the effects are several orders or generations away. The analyst should look for effects through not only his/her own eyes, but perhaps also from the perspectives of society, private individuals, and private institutions as well. That which one person perceives as a benefit or cost may be perceived by another in an entirely different light. Consider elective hysterectomy, for example. The patient weighs as costs the financial costs plus the psychological/physical trauma against the benefits of preventing pregnancy, uterine cancer, or both; whereas the health insurer may weigh only the cost of the operation with expected reduction in future maternity or gynecological care costs (see reference 304).

The effects of technology in the health field can be far reaching and varied; they can also be obvious as well as obscure. They often follow directly from the problem under consideration, the objectives specified, and the framework in which the problem is approached. Not all benefits or effects are positive—some may be negative (e.g., deaths due to surgery) and some may be indeterminant (e.g., incurable disease maybe discovered). Regardless, all effects should be identified and enumerated. To identify all benefits/effectiveness, each of the following categories should be considered: 1) personal benefits/effectiveness, 2) health resource benefits/effectiveness, 3) other economic benefits/effectiveness, and 4) social benefits/effectiveness.

Personal benefits/effectiveness.—The primary purpose of health care technology is to enhance the health and well being of individuals; consequently, the expected benefits/effectiveness should be examined in light of individuals’ personal health objectives such as lowered anxiety, alleviated pain, reduced risk of sickness or death, enhanced quality of life, and so forth. Seldom will the analyst come to the conclusion that an individual has seen the doctor for a checkup or given up smoking in order to save future medical bills, which is not to say that medical expenses are unimportant. However, CEA/CBAs frequently attach great significance to medical cost reduction, while often ignoring patients’ personal motives (see reference 304).

Health resource benefits/effectiveness.—A direct result of the use of health care technology is the change in use of other health care resources. For instance, preventive programs are often advocated because they are thought to enhance health and thus decrease future medical expenses. However, procedures such as screening may discover disease to such an extent that direct medical costs are, in the short run, actually increased. This phenomenon is likely to be observed when asymptomatic individuals are screened for socially latent problems such as venereal disease, mental illness, and drug abuse and for chronic conditions such as hypertension. Regardless of whether future medical costs are decreased or increased in the aggregate, shifts in medical resources will almost certainly occur and these shifts should be identified.

Other economic benefits/effectiveness.—Secondary effects resulting from changes in health status are often strictly economic. Healthy people are more productive than are sick people. These effects should also be identified. From certain points of view, such as the family’s, society’s, or the firms’, they may be very important, whereas from the health insurer’s point of view,
they may be totally irrelevant. Consequently, an efficiency study performed by a health care provider for an insurance firm may ignore such economic considerations; a socially oriented CBA/CEA should not.

Social benefits/effectiveness.—Finally, society has collective objectives which stem from its underlying values and traditions—objectives which are not strictly economic and not directly related to health status. These objectives may be concerned with the equitable distribution of medical care—ensuring that the poor have adequate access to health services—or with protecting the rights of the unborn, the mentally ill, or the comatose patient. Also, health and medical care resources may be employed to compensate certain of the Nation’s citizens for the lack of adequate housing, nutrition, employment, or parental care. All of these effects, intended or not, should be explicitly identified.

The special case of intermediate outcomes. —Notwithstanding the ultimate goal of improving health and welfare, many technologies, particularly diagnostic ones, can best be evaluated only in terms of intermediate outcomes such as blood counts per minute, clarity of X-ray film, or number of pounds lost per week. This methodological limitation is an especially disturbing one, since diagnostic information often leads to increased use of other diagnostic and therapeutic resources, resulting in higher expenditures (569). There are, however, certain benefits from diagnostic technologies which can easily be overlooked: Such technologies often provide for patient reassurance; they may avoid therapeutic interventions; and they may assist in furthering medical knowledge. Health promotion programs also are often difficult to assess in terms of improved health status, resulting in the necessity of measuring intermediate outcomes. Examples are weight control and antismoking programs.

Therefore, when final outcomes resulting from a health care process cannot be adequately identified, intermediate outcomes should be identified and the uncertainty of the link with final health status ought to be noted.

Measurement

Benefits/effectiveness initially should be counted in whatever units are most appropriate: Medical cost savings/expenditures are counted in dollars; reduced disability in days (or weeks, months, years); reduced mortality in years; changes in health status in well-years. Likewise, intermediate outcomes are counted as number of blood tests taken, number of persons examined, and so forth.

Some benefits/effectiveness are difficult to measure because they may be only partially, known, or not known at all. As was discussed in the section regarding the production of health, the efficacy and/or effectiveness of many interventions has not been demonstrated, and in those cases where it has, the technique seldom is efficacious and/or effective 100 percent of the time. In an earlier report (405), OTA found little evidence that health care technologies have been adequately and systematically evaluated. Without valid efficacy/effectiveness and safety information, the value of CBA/CEAs may be greatly diminished. Furthermore, even when there is good evaluative research on the technology in question, the information may not be directly applicable to the setting in which the technology will be used. Much of the good research is done under nearly ideal conditions such as in a controlled or partially controlled environment with the best data by the best researchers and clinicians; since applications of the technology will not normally have the benefit of such conditions, the projected “benefits” may be significantly overestimated. In any case, probability theory and sensitivity analysis can be used to embrace the concept of uncertainty, a subject which is explored more thoroughly in another section below.

Unfortunately, the intangible benefits/effectiveness are difficult to measure, although they are often the more important ones. The personal and societal benefits/effectiveness listed above, such as relief from anxiety and pain, for example, can often be estimated only by indirect methods such as patient satisfaction questionnaires, or by techniques which simulate an indi-
individual’s willingness to pay for the result. In the main, however, intangible benefits/effectiveness cannot be adequately measured—and consequently must remain only “identified.” This should not eliminate the desirability of the analyst’s including a statement of their probable importance.

Valuation

Valuing benefits/effectiveness is the next step of the process. Basically, the objective at this stage is to determine their worth. Sometimes the value is self-evident, such as when the benefit is money saved. Since the techniques of CBA and CEA are designed to compare cost with benefit or effectiveness, the analysis is much easier when both sides are measured in money—for instance, spending $100 in order to save $350 is easily understood. Many of the applications of health care CEA/CBAs concern benefits/effectiveness which are not easily translated into money, however, and there is disagreement concerning their worth. Some health care technologies save lives, limbs, days of disability, and discomfort; other technologies produce information (e.g., X-rays and laboratory procedures). What are these benefits/effectiveness worth? The answers seldom are obvious.

Valuation of benefits is further complicated by the problems of risk-averseness associated with individual preferences. For instance, McNeil, et al. (736) demonstrated that patients preferred radiation treatment over surgery for lung cancer even though surgery provided the better chances for survival. The explanation given was that the surgery itself carried with it a risk of immediate death, and, consequently, patients preferred the assurance of a certain, but perhaps shorter, life to facing the risk of immediate death. In addition, patients were willing to trade off a perceived increased quality of life with longevity. Valuing such individual preference is difficult and, even more important, recognizing that they even exist is easily overlooked. (For a more thorough discussion of risk preference and risk behavior, see reference 755.)

The attempt to value benefits/effectiveness often poses serious problems, perhaps even more extreme than the problems their measurement poses. In those cases where measurement is deemed impossible, impractical, or unreliable, attempts to valuate may not only provide no further useful information, but may actually mislead the reader by implying that the results are more valid than they actually are. An example of this may be the value of bereavement support in a hospice program. This benefit can certainly be identified, but it is not easily measured. Attempting to place a dollar value on it would probably be misleading.

Much of the controversy surrounding the valuation of health outcomes centers around the value of life, an issue which is directly pertinent only to CBA, since CBA alone expresses all costs and benefits in dollars. The oldest and most common method of valuing life is the so-called “human capital” approach, which values life in terms of earnings potential. The value is computed by summing the earnings lost due to premature death or to disability; conversely, it is done by summing the expected future earnings saved by postponing death or avoiding disability. All future moneys are discounted to a present value at some specified rate. (Discounting is discussed in another section below.)

One of the first problems encountered by human capital theorists was the problem of consumption: If, conceptually, life is valued in terms of its financial return to society, should not the individual’s own consumption be excluded from the benefit calculation? However, this solution would require valuing life at zero for those who consume all they earn, and valuing life negatively for those who deficit spend. Regardless of whether earnings are considered net of consumption, though, the human capital method is really valuing livelihood—i.e., one’s earning potential—not life.

The human capital approach also poses distasteful problems such as valuing men more highly than women, since males have traditionally earned more than females. Likewise, the working population is valued more than the very young or the very old, and whites more than nonwhites. In addition, this method fails to value other effects such as the psychic costs of death to friends and relatives.
Although the human capital approach has enjoyed wide application (owing principally to the absence of a better method), few people are satisfied that the value of life can truly be captured solely by estimating earnings potential. This has led to interest in other methodologies.

A conceptually more appealing approach is the "willingness-to-pay" technique (e.g., 470). The idea is to attempt to capture the value, to an individual, of reducing the risk of death or disability by small amounts, and using the information to imply the value of life itself. This method has its own conceptual problem in that the imputed values are still income related: A rich person will be willing and able to pay more to reduce the risk of death than will a poor person. Also, there is a question as to whether an individual can understand what a small reduction in statistical probability of death means. Nevertheless, the willingness-to-pay approach is the only technique that attempts to estimate individual perceptions as to one's own worth, which, presumably, includes such diverse notions as personal values, risk averseness, family obligations, age, income, personal desires, and even a philosophical outlook on life. Despite its conceptual appeal, however, there is no indication that such a method can arrive at a consistent value for all human life. It also ignores the value of one's life to others in society.

Other methods of valuing life have attempted to make use of imputed values from life insurance holdings and from jury verdicts. The former suffers from the same conceptual problem as the human capital method since "earnings" not "life" is insured; the latter suffers from gross inconsistencies (291). Neither method has found much acceptance.

Notwithstanding the unending criticism of the techniques to value life, the concept is an important one. Klarman writes (291):

As Mishan observes, a rough measure of a precise concept is superior to a precise measure of an erroneous concept. It is agreed that the notion of the value of human life, apart from livelihood, is sound. And a numerical estimate of this value would be useful in comparing how worthwhile alternative programs are. Comparisons of programs would gain in relevance and aptness if all benefits were counted, including saving of human life or gains in life expectancy. This potential gain is much more likely to be realized if all benefits are entered into the model, rather than having some appear only in footnotes.

CEA attempts to avoid this valuation controversy by simply counting the lives or years of life saved (or lost) and not transforming the numbers into money. Once money is allocated to save lives, however, the value of life is implied—an important point which is easily overlooked. Notice that in CBA, the analyst must choose a value to complete the analysis; in CEA, the policy maker chooses the value, albeit indirectly. For instance, when analysts assess competing life-saving programs using CBA, they must choose a specific value (or range of values) for life. The most attractive program, in terms of the analysis, is that which computes to have the highest net benefit; if benefits exceed costs, adoption of the program would ordinarily be recommended. Analysts using CEA, however, compute the cost per life or year of life saved. Although the most attractive alternative is that which provides the most effectiveness for a given cost, the decision to adopt the "best" program depends on the implied value which the policymaker places on life, or on health status change. For instance, of several life-saving programs, the most attractive may cost $100,000 per life saved. The decision to adopt that program depends on whether the policymaker thinks that $100,000 per life saved is reasonable.

A common misconception regarding the two techniques is that CEA avoids value judgments. In fact, many value judgments are made, albeit often implicitly. These include judgments such as the equating of different lives—is a young life worth the same as an old one? Judgments such as the equating of years of life—is 1 year of life for 15 people equal to 15 years for 1 person? Judgments such as equating all days of disability—is the day lost due to the common cold equal to that lost due to surgery? Clearly, CEA is not value neutral.

Time-related distortions.—Since many benefits/effectiveness of health care technologies oc-
cur during widely varying intervals of time, analysts must somehow place them in perspective in order to allow comparisons to be made. That is, they must be able to compare the value of reducing $1 of medical cost today with reducing it next year, with saving a life today with saving it next year, and so forth. The accepted practice is to transform each future effect to a present value by means of a discount rate, which is similar to an interest rate. Discounting has long been used for the valuation of financial resources, but in the health care field it has only recently been applied to the valuation of non-financial resources (78,79,406,572). (A more complete discussion on discounting is presented in a separate section below.)

Valuing multiattributed outcomes. —As noted previously, many individual processes in health and medical care produce widely divergent outcomes, ranging from diagnostic information to the relief of pain to the prolongation of life. Not only is each of these outcomes difficult to measure (if, indeed, the outcomes are ever identified) and to value in its own right, but the various outcomes are also difficult to compare with one another. For instance, how much pain is worth a life? That would be a difficult question even if pain could be accurately measured. Nevertheless, conceptually, the issue of comparing outcomes is important, and recently, considerable progress has been made in weighting outcomes so that health status changes can be combined. In a recent report, for instance, OTA used the concept of quality-adjusted life years to evaluate the cost effectiveness of pneumococcal vaccine (406). This technique attempts to weight differences in health status in relation to good health. Thus, a day of good health is assigned the value of “1,” a day of death the value of “0,” and days of sickness, depression, or disability values somewhere between these extremes. For instance, total disability may be assigned a value of 0.1, while a slight disruption of daily life due to the common cold may be assigned a value of 0.9.

Valuing intermediate outcomes.—Above it was suggested that in certain cases, often when evaluating preventive or diagnostic technologies, intermediate outcomes may be valid objectives, but their measurement begs their worth. What is the value of an X-ray? A blood test? Or a physical examination? Often, no value can be assigned to these outcomes. When this is the case, it is incumbent on the analyst to note that it is and to state the extent to which the technology is associated with final outcomes as well as their probable importance to the study’s result.

Costs

Identification

In this report, the term “costs” refers to the resources expended to produce an intended benefit or effectiveness. For instance, the costs of a screening program would include the amount of services provided (personnel time, supplies, capital expenditures), as well as the patients’ time which is forgone in the use of the service. Care should be taken to identify all resources which are expended or which must be expended. In general, the concept of “opportunity cost” is the true cost of a program. That is, the cost is equal to the value of the opportunities which are lost as a result of the investment in the program. Initially, costs should be identified in terms of the actual resources used in the production process—person hours, supplies, and so forth. In structuring the analysis, it is helpful to consider costs as a broader concept than simply financial resources.

Identifying the proper financial costs is always easier conceptually than it is in actuality. In the health care field, this task is even more difficult because charges often do not reflect true costs, a fact which is sometimes due to unsophisticated accounting procedures and other times due to the deliberate subsidization of one service by another. Hospitals, for instance, are known to operate some services, such as maternity wards, at a loss while operating certain ancillary departments, such as diagnostic laboratories, at a profit. The same was found true for neonatal intensive care (see reference 71).

Also, since many resources in the health field are often erroneously thought to be “free,” some costs may be understated. A good example is
the cost of volunteer time, where, under the opportunity cost concept, volunteer time is worth that which is forgone by its use. For example, rather than working in a hospital ward, the volunteer may have worked for the heart association. Or the service rendered by a volunteer may ordinarily be one which the organization must otherwise buy.

In other instances, owing to market imperfections, inappropriate use of resources may be used which can lead to the overstating of costs. Physicians giving immunizations, registered nurses making beds, and dentists cleaning teeth are examples.

Next, the identification of the technological resource costs will depend on the stage of the technology's development. For a new technology, costs may be difficult to identify, but should include the R&D costs as well as the capital costs associated with purchasing and operating the equipment. For an established technological process, where the analysis concerns the level of use, marginal operating costs can dominate. One potential problem of using past performance to project future costs is that costs may change as a result of increased efficiency, technological change, or changes in scale.

Other reasons why costs are difficult to identify are: 1) some costs, such as overhead, are common to many products; 2) some technologies produce multiple outcomes—if the CEA/CBA study concerns only one, the analyst must somehow determine which costs must be included, which are to be ignored, or how they are to be shared; and 3) often, during the production process other tasks are or can be performed at minimal incremental cost.

Measurement

Initially, measurement should consist of counting the minimum resources or units of service required to produce the intended benefit or effectiveness. Generally, this step in the evaluation will follow naturally from the identification process. In cases where substitution of resources is possible, however, care must be taken to count the generic service required. Thus, the number of hours of immunizations, of making beds, and of cleaning teeth should be counted, not the number of physician, nurse, or dentist hours required to accomplish the respective tasks. The same argument can be used for measuring volunteer time.

Measuring costs when joint production factors must be considered is extremely difficult, often not very reliable, but may be critical to the validity of the analysis. Ignoring joint production effectiveness simply because it is hard to measure can lead to a considerable overstatement of costs.

Valuation

Most analysts believe that valuation of costs poses substantially fewer problems than valuation of benefits, because many cost resources have a real or easily imputed market value. In cases where costs reflect opportunity costs and where they are measured in dollars, valuation is essentially complete, except when the costs occur during different periods of time, in which case discounting is required. When costs are measured in generic terms, such as type and amount of services provided, valuation can be relatively difficult and sometimes controversial. This is because the professions in the health field often successfully restrict others from performing tasks, which could otherwise be safely performed at a more economical level (749). Nevertheless, the analyst should critically evaluate the resources required to accomplish the job, taking note of political, legal, or technical constraints to providing the service at the most efficient level possible.
VALUING BENEFITS AND COSTS OVER TIME: DISCOUNTING

Costs and benefits seldom occur at the same point in time. But in order for valid comparisons to be made, they can be treated as if they all occurred in the present, through the application of a method termed discounting.

The rationale for discounting future costs and benefits stems from the fact that resources can be productively invested for future gains, as well as from the observation that people expect to be rewarded for postponing gratification. For instance, in order to induce individuals to save, interest must be paid, even in the absence of inflation. The rate of interest determines the future value of the amount invested. Thus, $100 invested at 5-percent interest will become $105 in 1 year. Discounting is the reverse process: $105 next year has a “present value” of $100 when the discount rate is 5 percent.

Thus, just as an amount, \( p \), invested at interest, \( i \), has a future value of:

\[
p (1 + i)^{\text{in year } 1}
\]

\[
p (1 + i)^{\text{in year } 2}
\]

and

\[
p (1 + i)^{\text{n in year } n}
\]

an amount of money, \( p \), \( n \) years in the future at discount rate, \( i \), has a present value of:

\[
\frac{p}{(1 + i)^n}
\]

Likewise, a stream of future benefits or costs is the sum of each amount, discounted at rate, \( i \), from whichever year the benefit/cost is incurred.

There is general agreement among economists and policy makers that discounting future nonmonetary benefits/effectiveness. Fortunately, when benefits/effectiveness occur over a long time, almost any discount rate used makes them less and less important to the outcome of the analysis in a fairly short period of time (480). This phenomenon results in making the rate used and the uncertainty of future events less important than they otherwise would be.

Setting the Discount Rate

The particular rate chosen can have a substantial impact on the outcome of the analysis, since investment in health programs often means spending present moneys, which are not discounted, for future benefits, which are. In such programs, the higher the discount rate, the less attractive the program appears. As an example, suppose we spend $1,000 today, expecting to save $2,000 in medical costs 10 years later. In order to compare the expected benefit ($2,000 savings) with the costs of the program ($1,000), we must discount the benefit to its estimated “present value.” Consider the varied results using different discount rates:

<table>
<thead>
<tr>
<th>Discount rate (%)</th>
<th>Present value of benefit</th>
<th>Present value of net benefit (B – C)</th>
</tr>
</thead>
<tbody>
<tr>
<td>0</td>
<td>$2,000</td>
<td>$1,000</td>
</tr>
<tr>
<td>5</td>
<td>1,228</td>
<td>228</td>
</tr>
<tr>
<td>7</td>
<td>1,017</td>
<td>17</td>
</tr>
<tr>
<td>10</td>
<td>771</td>
<td>– 228</td>
</tr>
</tbody>
</table>

And, if the benefit were not realized for 20 years, the results would be:

<table>
<thead>
<tr>
<th>Discount rate (%)</th>
<th>Present value of benefit</th>
<th>Present value of net benefit (B – C)</th>
</tr>
</thead>
<tbody>
<tr>
<td>0</td>
<td>$2,000</td>
<td>$1,000</td>
</tr>
<tr>
<td>5</td>
<td>754</td>
<td>– 246</td>
</tr>
<tr>
<td>7</td>
<td>517</td>
<td>– 483</td>
</tr>
<tr>
<td>10</td>
<td>297</td>
<td>– 709</td>
</tr>
</tbody>
</table>
Many programs in the health field have even longer benefit time horizons. Thirty years is not uncommon, especially for prevention or health promotion programs such as antismoking clinics and pap testing. Thus, we see the power of discounting and the resultant importance of the choice of rate.

Most economists believe that the correct rate is the opportunity cost of capital in the private sector, subject to certain adjustments (e.g., the adverse effect of pollution produced by private sector investments). That is, society could opt to invest the money in the private sector and earn “benefits” at a substantial rate (perhaps as high as 15 percent) which represents the opportunity that is lost by investing in the health program.

An alternative argument, also persuasive, is that the discount rate for social programs is considerably lower than the opportunity cost of capital since society’s objectives include the equitable distribution of benefits to future generations. Klarman (291) refers to this argument as reflecting society’s “social rate of time preference.” Referring to the numerical examples in the text above, the reader will note that low discount rates result in future benefits’ appearing more attractive. That is, society would more readily invest money in the present to reap future health benefits if a low rate were used (706). In any case, whether the correct rate is the private cost of capital or a lower social rate, agreement would still not be reached on the precise number because of the fact that interest rates vary not only across time but also across investment opportunities in any single point in time. In the absence of agreement, the accepted method to treat this uncertainty is to present the results testing several rates—a technique generally referred to as sensitivity analysis. (Sensitivity analysis is discussed in a separate section below.)

**Valuing Nonmonetary Benefits/Effectiveness Over Time**

How does one compare the value of a life or a day of disability which is saved in the future, with the same benefit which is saved in the present? In CBA, all such benefits are transformed into monetary terms, a controversial process discussed earlier, and then discounted the same as any other future financial asset. In CEA, however, benefits are expressed in nonmonetary terms such as lives or years of life saved. Should these be discounted as well? Weinstein and Stason (575) presented a persuasive argument in the affirmative. One way to explain the need for discounting future nonmonetary benefits is to assume no discounting, and for the sake of clarity, to assume no inflation. Consider a lifesaving program which costs $1,000 and saves, immediately, 10 years of life (i.e., $100 is spent per year of life saved). Assume also a linear relationship between costs and benefits, if that $1,000 were invested at 5-percent interest instead of being spent on the life-saving program, in 1 year we would have $1,050 which could be used to save approximately 10½ years of life; in 2 years there would be $1,102 which could be used to save over 11 years of life; and so forth. Therefore, unless a year of life is valued more highly in the present than in the future, the rational decision will always be to put off spending the money for an additional year. Discounting all benefits/effectiveness to present values avoids this irrational incentive.

In conclusion, then, although the discounting of future benefits/effectiveness and costs is conceptually correct, there is not, nor is there soon likely to be, consensus regarding the rate for two general reasons. The first is technical in nature: Interest rates vary across both time and investment opportunities. The second is conceptual: The discount rate can reflect the private opportunity cost of capital, or a lower social rate of time preference. The results of CEA/CBAs should be presented using several discount rate estimates in order to examine the influence of the rate on the results—again, a technique referred to as sensitivity analysis, discussed immediately below.
ADDRESSING PROBLEMS OF UNCERTAINTY: SENSITIVITY ANALYSIS

Discussions in the preceding sections have noted the uncertainty of knowledge regarding the etiology of disease and regarding diagnostic and curative techniques. Superimposed on these variables are changing personal habits, interactive environmental conditions and often unforeseeable future technological developments. In addition, there is lack of agreement as to the magnitude of health status changes and the value of the discount rate. How can we have confidence in predicting results in the face of all this uncertainty?

One possible answer is to place the results obtained from analysis in perspective, to examine closely the assumptions upon which the analysis rests, and to test the sensitivity of the results to reasonable changes in these assumptions.

Uncertainty can be classified into that which is due to random events and that which is due to ignorance. Unfortunately, many events in the health field suffer from both types. The first, random type, refers to events which occur according to a probability distribution. In general, these are events associated with large numbers. An example is the number of heart attacks occurring in a large population at any given time. This event is thought to be random and is statistically predictable. It is different from the chance of a heart attack occurring to an individual, which is dependent on nonrandom variables such as a person’s living habits and genetic heritage. In some instances, events in the health field are thought to be random, but their probability distribution is not known, which makes prediction more difficult.

The second, and more troublesome, type of uncertainty is due to ignorance. Sometimes the problem is simply lack of information—we do not know what causes cancer, or what triggers certain allergic reactions—in which case we have the option of buying more information, either through more research, more time, or both. In other instances, the uncertainty is due to future events over which we have no control—women may smoke more, or there may not be an influenza epidemic this year—in which case the best we may be able to do is to examine trends or use expert opinion.

When evaluating a health care technology or program in the face of the unknown, the analyst has a rather impressive sounding arsenal of techniques. For random events, probability theory can be used, often through the application of decision analysis, which is a diagraming of the possible courses of action, each branch accompanied by a known or imputed probability. When probabilities are not known, expert judgment can be substituted. Thus, without knowing the cause or even the dynamics of a given random process, the analyst can attempt to predict the likelihood of an outcome. But there are other techniques from the field of operations research such as Monte Carlo and Markov Chain methods which allow manipulation of a simulated process until the outcome mirrors empirical findings such as incidence rates of a disease (see reference 335). These analytical methods can provide valuable insight as to what process may be occurring. They may also provide a false sense of security to a policy maker, since the terminology and the technical sophistication which is required often mask the tenuous assumptions on which the methodologies rest.

Sensitivity analysis is the examination of an uncertain event under different assumptions. Earlier we discussed discounting, concluding that the precise discount rate was unknown, and that a consensus may never be reached. Under this uncertainty, one logical course for the analyst is to test the sensitivity of the results to several discount rates. For instance, one can test a low, high, and middle value—an approach which is most helpful when there is a wide range of reasonable estimates. Or one can incrementally change the rate about the suspected mean—an approach that is feasible when the range of
possibilities is relatively narrow. In either case, if the results of the study vary widely when the different values are used, one can have less confidence in any single set of results. Conversely, if the results change little, then the precise rate may be unimportant. In some CEA/CBAs, the decision criteria rest on the rank order of alternatives, not the absolute values involved, and the analyst need only determine whether the ranking itself is disturbed.

There are other approaches that increase the confidence one can place in analysis in the face of uncertainty (744). For instance, a technique known as “worst case analysis” can be done by assigning to the uncertain variables values that least help the program (i.e., which the analyst believes to be the preferred one). If the program still is preferred, one can have more confidence in recommending it. Another method is termed “break-even analysis,” in which assumptions are varied until some minimally acceptable result is obtained; one can then ask whether the assumptions are realistic. For example, a CBA requires a value to be placed on life, yet there is no generally accepted value. The value can be varied upward, starting from zero, however, until the analysis indicates that the program is acceptable; then the value for life can be examined. Perhaps it is so low (say $1,000) that all would agree that life was worth at least that amount. In such a case, analysis can proceed more confidently in the face of the extreme uncertainty of this critical variable. In other instances, the analysis will not indicate adoption of the program unless a very high value is placed on life. Here, the preferred course of action may not be so apparent. Techniques such as worst case and break-even analyses are often more helpful in identifying exceptionally good programs than in ruling out bad ones. Nevertheless, these and other similar techniques can be helpful in reducing uncertainty.

To summarize, sensitivity analysis can produce three important results:

1. It can demonstrate the substantial dependence of a conclusion on a particular assumption.
2. It can demonstrate that an assumption does not significantly affect a study’s conclusion, and hence that the tenuousness of the assumption is not a source of concern.
3. It can establish a minimum or maximum value which a variable must have for a program to appear economically worthwhile.

Finally, uncertainty can often be reduced; it should never be ignored. Results of a CEA/CBA should be accompanied by statements regarding the confidence which the reader can place in them. A sensitivity analysis is most helpful in this regard.

**EXAMINING ETHICAL ISSUES**

Ethical issues permeate both the process and the use of CEA/CBAs in health care. Some of these issues have already been touched on (e.g., valuing life), others have only been hinted at (e.g., using uncertain information). In general, there are powerful ethical arguments both for and against using CEA/CBA-type studies to help make decisions. The arguments for using CEA/CBA center around the concept that “some information is preferable to no information,” whereas the arguments against tend to be based on the actual or potential misuse of the technique. Here, we will present a brief discussion of the ethical issues involved. Readers who are interested in a more detailed discussion of this topic are referred to appendix D of the main report of this OTA assessment. That appendix—“Values, Ethics, and CBA in Health Care”—was prepared for OTA by the Hastings Center.

Some of the ethical arguments against using CEA/CBA stem from the fact that the delivery of health care itself has strong ethical overtones. For instance, many public policies are directed at eliminating or reducing financial and social barriers to health care. Because CEA/CBA is looked on by some as a rationing mechanism
based on costs, there is bound to be resistance to its use.

From a methodological standpoint, the ethical arguments against using CEA/CBA concern the difficulty in valuing that which is often most important: life, pain, happiness. They also concern the misuse of information. There is the fear that quantified variables will take on undue significance, and that assumptions will be treated as if they were fact. These arguments are particularly compelling since both the analyst and the decisionmaker may be responsible for such problems. For instance, this assessment finds that the process of CEA/CBA is subject to systematic methodological bias, whereby a given analysis can be “legitimately” performed in a variety of ways, each of which may affect the interpretation of the results. In addition, the policy maker may ignore the traditional caveats that are often, but not always, provided by the analyst.

There are also many other inherent problems that have ethical overtones, such as: 1) the value of a benefit may vary across individuals, or may be perceived to be different between the individual, society, or the relevant program (e.g., third-party payer), 2) the value of the benefit may differ between generations, 3) the value of quality of life is difficult to assess in comparison with other effects such as increased longevity, thus making tradeoffs difficult to analyze.

The counterarguments—by those in favor of using CEA/CBA—acknowledge the ethical problems, but say that if used in the proper perspective such analyses can help by making explicit the assumptions on which decisions are based. The Hastings paper (app. D of the main report) concludes: “We are persuaded that, in an important sense, the defenders of cost-benefit analysis are correct when they argue that policy decisions in the health field are being made daily on shaky grounds anyway, and that cost-benefit analysis is at least an attempt—however imperfect—to ground those decisions in real needs and real possibilities. The problem is not that cost-benefit analysis is not objective and not value-free, but rather that objectivity and value-freedom are unjustifiably attributed to it.”

PRESENTING AND INTERPRETING FINDINGS

We have mentioned the limitations to which most CEA/CBA studies are subject: the uncertainty of many key variables; the difficulty of identification and measurement of benefits and costs; the inability to value and incorporate many effects, such as ethical ones.

Implied throughout this chapter is the technical complexity of many studies. This type of study can lead to misinterpretation of results since: 1) the intended audience is often public officials or health care professionals, who may not be technically oriented, and 2) study findings are often reported in capsule form such as a news brief and are often introduced in the professional literature in abstract form. Consequently, the writers and analysts must be particularly careful in the way they present the results and interpret them for the reader. The presentation of the findings should identify the important variables and should discuss the confidence that the reader can place in the values that were used. A review of the findings and the significance of the sensitivity analysis, if used, is ordinarily necessary to place the results of the study in proper perspective.

There are also certain technical considerations that can significantly alter the way in which a study is interpreted. The first is the use of net benefit (that is, benefit minus cost), rather than the cost-benefit ratio as a criterion to compare programs. The former (net benefit) approach is usually preferred, especially when the alternative programs are widely variant in scope. As an illustration, consider two programs:

Program A costs $2,000 and reaps gross benefits of $4,000; program B costs $2 million and
reaps gross benefits of $3 million. A net benefit approach yields the following results:

<table>
<thead>
<tr>
<th>Program A</th>
<th>Program B</th>
</tr>
</thead>
<tbody>
<tr>
<td>$4,000 – $2,000 = $2,000</td>
<td>$3 million – $2 million = $1 million</td>
</tr>
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</table>

Clearly, program B is preferred, given the ability to finance the project and setting aside for the example all considerations of equity and distributional effects.

However, a benefit-cost ratio (B/C) would yield the following results:

<table>
<thead>
<tr>
<th>Program A</th>
<th>Program B</th>
</tr>
</thead>
<tbody>
<tr>
<td>$4,000</td>
<td>$3 million</td>
</tr>
<tr>
<td>$2,000</td>
<td>$2 million</td>
</tr>
<tr>
<td>2</td>
<td>1.5</td>
</tr>
</tbody>
</table>

Now, program A is clearly preferred. Notice that the ratio gives the reader no indication of the size of the expected benefits, nor the size of the program. Also, although program A gives a better rate of return for the money invested, there is no reason to believe that it can be increased in scale and still maintain the high rate of return.

The B/C ratio is also sensitive to whether an effect of a health program is considered as a benefit or as a negative cost. In the discussion of costs and benefits, it was pointed out that medical cost savings, resulting from an investment in disease prevention/health promotion, are treated as negative costs in a CEA (i.e., the “savings” are subtracted from the costs) and as benefits in a CBA. This distinction is technically important only when a cost-benefit ratio is employed; when costs and benefits are netted, it makes no difference whether a particular item is considered a benefit or a negative cost.

The interpretation of an analysis can also be distorted as a result of problems of scale. For example, if it is impossible to compare equal cost or equal effectiveness alternatives in a CEA, cost-effectiveness ratios can be misleading. Consider the following hypothetical case presented in tabular form:

<table>
<thead>
<tr>
<th>Lives saved</th>
<th>Program A</th>
<th>Program B</th>
</tr>
</thead>
<tbody>
<tr>
<td>$100,000</td>
<td>10</td>
<td>–</td>
</tr>
<tr>
<td>$200,000</td>
<td>–</td>
<td>15</td>
</tr>
<tr>
<td>C/E $10,000 per life saved = $13,333.33 per life saved</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

According to the strict ratio rule, program A is preferred; it costs less per life saved. But if there is no possibility of replicating program A (i.e., saving another 10 lives for an additional expenditure of $100,000), might we not prefer program B? For $100,000 more than the cost of program A, it saves an additional five lives. Is that not a worthwhile expenditure? It is at this point that the question becomes largely a social or political one. CEA has contributed information which may inform and assist social decision-makers, but it has not produced an economically preferable conclusion. However, the CEA technique is usually used to compare alternative means to achieve some objective. In this case, the decision to invest has already been made, and the analysis is used to choose the most efficient method. Thus, if the decision has been made to reduce deaths due to heart disease, the cost per life saved may be compared between blood pressure screening programs, inpatient coronary care units, mobile coronary care units, and cardiopulmonary resuscitation classes for the general public. Alternatively, when operating under a budget constraint, the number of lives saved per dollar amount available is compared between programs.

A related point is that an analysis for an existing technology should be performed at the margin. That is, the additional cost of using one more unit should be compared to the additional benefit derived. In some instances, the additional cost is so small that one additional unit will be extremely cost effective even if the expected benefits are small. In other instances, the additional cost may not be large but the added benefit is infinitesimal. Neither of these subtle, but valuable, insights will necessarily be gained if the analysis uses only average (as opposed to marginal) costs and benefits. A marginal analysis will help to determine the optimal size of a program and the point at which a given technology is no longer cost effective (468,559).
LINKING ANALYSIS TO POLICY IMPLEMENTATION

As noted at the outset, a CBA/CEA should consider all the relevant costs and benefits/effectiveness, regardless of to whom they may accrue or when they may occur. Also, although an analysis can take on a program or organizational perspective as well, a CEA/CBA is ordinarily performed from a societal point of view. In identifying the appropriate societal costs and benefits/effectiveness, these variables need to be viewed from perspectives other than that of society, in order to make the analysis more relevant to public policy decisions.

It is frequently noted that ours is a pluralistic society—one with many individuals and institutions making decisions that ultimately affect the allocation of society’s resources. The field of health care is no different. “Society” does not make decisions; private consumers, physicians, Congress, administrators of hospitals, managers of philanthropic organizations, and officials of medicare or medicaid and of local government agencies, and other people within society do. In addition to considerations of societal efficiency, their decisions depend on such diverse notions as reimbursement guidelines, community interests, the attracting of professional staff, intangible humanitarian objectives, pride, financial solvency, and sometimes institutional survival (335,336).

Also, because of the manner in which health care in the United States is organized and financed, there is ample reason to believe that the objectives of key private individuals and institutions have an entirely different focus than the objectives of society. For instance, in health promotion/disease prevention programs, costs are often incurred by a private party in the present, whereas benefits usually accrue in the distant future—and they accrue to others as well as to the party who funded the program.

On the other end of the spectrum are diagnostic and therapeutic procedures. For these, private incentives tend toward overutilization. The procedures are often paid for by insurance on a fee-for-service basis; hence, increased utilization tends to be financially rewarding to the provider without being costly to the patient. This situation has given added emphasis to nonmarket controls such as the certificate-of-need process that health systems agencies require of an institution for major capital investments. Likewise, the current interest in a hospital, or systemwide, revenue cap perhaps stems in part from the lack of financial incentives toward cost-decreasing technologies (758).

All these reasons, then, lead to questioning of the applicability of traditional, societally oriented CEA/CBAs. The problem is similar to that of a mass screening program when there has been made no provision for treating discovered disease. In both cases, the information produced is very important, but is useless unless a system is in place to use it. Just as the answer in the case of the screening program is not to discard the screening, the answer in the case of CEA/CBA may not be to discard the technique. The answer in part is to make the analysis more relevant—by attempting to identify the private objectives, and by noting when they conflict with and when they support society’s objectives. If this is done well, decisions may better reflect reality.
3. Literature Review
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INTRODUCTION

Application of cost-effectiveness analysis/cost-benefit analysis (CEA/CBA) to health care represents a fairly new endeavor. The novelty of CEA/CBA within medicine has both positive and negative reflections. On the one hand, the growth in the literature demonstrates considerable enthusiasm for the technique; perhaps more importantly, it may indicate increasing cost consciousness within the medical community. Further, it might signal an increased awareness of the idea that a comparison of costs and benefits has always been an implicit value of health care decisionmaking—and that to allow resource allocation decisions to continue to be made, perhaps unwittingly, without more explicit consideration of the costs in relation to benefits is not desirable. On the other hand, the enthusiasm for CEA/CBA is often undisciplined, perhaps reflecting the inevitable growing pains of any field of inquiry. The vast majority of literature contributions whose titles identify them as related to CEA/CBA have serious technical flaws or conceptual weaknesses in structure or interpretation.

Also included in the literature are many important exceptions to this general assessment. Several studies exhibit both the desired technical features and the potential to lend insight into important issues of health resource allocation. Through such examples, as well as direct “instruction” (e.g., articles that review methods, cited below), a small cadre of skilled practitioners of CEA/CBA seems to be providing the intellectual leadership to improve the general quality of the literature and advance the state of the art. This group includes both physicians and economists, and several of the recent exemplary studies have resulted from multidisciplinary collaboration (e.g., 472,575).

Two contextual aspects of the evaluation presented in this chapter warrant emphasis at the outset. First, many of the limitations of health care CEA/CBAs are endemic to—and, more importantly, inherent in—almost all CEA/CBAs. For example, the inability of most health care CEA/CBAs to incorporate distributional considerations (177,179) is shared by CEA/CBAs on education, defense, energy, transportation, and so on. This chapter attempts to identify generic CEA/CBA problems and to distinguish them from problems that are specific to the health care literature. The chapter also distinguishes problems that are resolvable from those that are inherent in the process of analysis.

Second, literature reviews often restrict their attention to the most prominent articles and books in the literature, as is the case in the earlier reviews of health care CEA/CBA identified in appendix A. There is a logic to this approach: These publications reflect and indeed create the state of the art; because they are widely read, they have an influence on professional thinking and on future contributions to the literature disproportionate to their numbers. Nevertheless, such publications constitute only the most visible portion of the literature. The 10, 20, or 30 articles repeatedly cited in health care CEA/CBA reviews represent considerably less than a tenth of the publications that can be readily identified as part of this literature, often by explicit inclusion of the words “cost-benefit” or “cost-effectiveness” in their titles. A few such publications are clearly mislabeled; many others are on the right track but are so “poorly” handled that a CEA/CBA purist might exclude them from a CEA/CBA bibliography. Even though the less well-known publications have a readership and general influence far smaller than that of the more prominent contributions, however, they may constitute the principal exposure of many practicing health professionals to the language, concepts, and applications of CEA/CBA.

In order to capture the essence of what CEA CBA means to health professionals, it is impor-
tant to critique the entirety of the literature. Thus, this review represents an attempt to integrate typical-practice and state of the art features of the literature. The basis of the review is an assessment of general tendencies in the literature as a whole, including the 90 percent that to a large extent has escaped attention in previous reviews. Common problems and deficiencies are frequently counterbalanced by reference to successful attempts to address the deficiencies. Thus, while this review adopts a generally critical stance toward the literature, it acknowledges the many examples of technical proficiency in the practice of health care CEA/CBA. For a review that concentrates on the high-quality end of the spectrum, the reader is referred to the recent paper by Weinstein (569).

METHODOLOGY AND REVIEW ARTICLES: CONVEYING PRINCIPLES AND PRACTICE TO THE HEALTH CARE COMMUNITY

All good studies inform readers about CEA/CBA methods, either implicitly through its application (e.g., 122) or explicitly through discussion of methodological issues (e.g., 148). Recent books have served several functions: attempting to convey basic principles, break new methodological ground, and demonstrate the utility of CEA/CBA through specific applications (73, 516). Over the years, however, there has been only a handful of articles written solely to present or to evaluate the state of the art in health care CEA/CBA.

During the period studied (1966-78), the first two such articles were published in the first 2 years of the period. In 1966, Crystal and Brewster (722) wrote an introduction to CEA/CBA in the health field. In 1967, Klarman published the first of two prominent reviews he has written, this one appearing in the American Journal of Public Health (295). From then until 1972, no significant health care review or methodology contributions appeared in print, with the exception of a chapter by Grosse (241) in a book oriented toward students of economics and policy analysis. That chapter is particularly noteworthy for its review of CEA/CBA applications in the Department of Health, Education, and Welfare (HEW) during the author’s tenure as an HEW official, Grosse conveyed much of the same material 2 years later in an article published in 1972 (240), though again the audience was not specifically health care professionals.

That same year, however, witnessed publication of a book that has become one of the health care community’s most widely read and frequently cited contributions, Cochrane’s Effectiveness and Efficiency: Random Reflections on Health Services (97). This short book appears to have had a profound and sustained impact in turning the thoughts of health care professionals toward issues of resource scarcity and the link between efficiency and equity. It is at least possible that Cochrane’s book played a significant role in the rapid growth in health care CEA/CBA that began in 1973.

The most often-cited review and discussion of health care CEA/CBA is Klarman’s second article (291), which appeared in 1974. The following year, Dunlop (153) published a review that characterized the early literature, thus providing some interesting contrasts with current CEA/CBA practice. In 1975, the major portion of an issue of the New England Journal of Medicine (NEJM) was devoted to a discussion of CEA methodology and to several illustrations of its application. To many observers, this NEJM issue stands as a landmark in the evaluation of medical practice. It should be noted that none of the previous reviews had been published in a medical journal.

Two years later, another issue of NEJM offered readers a package of two articles and an editorial, including a discussion of CEA methodology (574), a sophisticated application of it (516), and an important, thoughtful treatment of the limitations of formal analysis, including the potential for a “tyranny of numbers” and associated disregard of equity considerations.
Many health services researchers consider this package, combined with Weinstein and Sta-son’s book (575), to be a milestone in health care CEA/CBA.

A recent review was presented at an Urban Institute Conference on Medical Technology in December 1978 (569). This paper offered a state-of-the-art assessment of the literature and a review of “a nonrandom sample” of health care CEA/CBAs. The most noteworthy feature of the paper is the author’s discussion of remaining methodological issues. Although several of the issues have been of concern since the inception of formal CEA/CBA, others represent subtle, sophisticated problems, the existence of which is testimony to progress on more basic issues. Indeed, the paper serves as a vivid reminder simultaneously of the frustrating, seemingly intractable problems of CEA/CBA, and of the gradual yielding of some of them to sustained conceptual and empirical struggle.

DEFINING THE PROBLEM, OBJECTIVES, AND ALTERNATIVES

In a review of the early health care CBA literature, Dunlop (153) concluded that the most common use of CBA had been to analyze disease-specific programs of intervention. By beginning with disease problems, several early analyses had the opportunity to explore a wide range of alternative interventions. For example, the interest of LeSourd, et al. (327) in identifying efficient means of grappling with kidney disease led these investigators to compare the costs and benefits of a variety of programs ranging from prevention of disease to treatment of renal failure. Similarly, Acton (4) employed both CBA and CEA to examine several alternative prehospital programs for reducing deaths due to myocardial infarction. Even in studies of narrower problems—for example, the treatment of existing disease—comparison of alternatives characterized much of the early analyses. Thus, the focus of Klarman, et al. (298) on kidney disease treatment precluded consideration of prevention alternatives, but the authors examined all of the major therapeutic alternatives.

Most of the contributions to the early literature shared a focus on a problem and specific objectives that had a distinct health (or disease) starting point. At the extreme, HEW analysts used CEA and CBA to examine resource allocation across a wide variety of disease and accident control programs (240,241). More narrow problem definitions implied fewer and less disparate alternatives, but the health relevance of the objective was generally clear. Thus Weisbred’s (577) examination of the costs and benefits of medical research was restricted to the case of polio, but the analysis centered on the health consequences of polio research and consequent prevention of the disease.

In recent years, there have been numerous attempts to use CEA/CBA to analyze programs having clear health relevance (e.g., 122,472, 473,573), but two factors seem to be increasing the proportion of studies whose health relevance is implicit, tangential, or simply unclear. The first is a tendency to assume that certain programmatic outcomes are desirable, without questioning their ultimate health implications. Common examples are studies that conclude that certain screening efforts are “cost effective” because they are inexpensive, but that lack any exploration of the costs and health effectiveness of followup of the cases found (133). The second is a technical factor and reflects the current importance of cost containment as a health policy issue. Much analysis has moved from a focus on promoting health toward concern with achieving efficiency in the provision of existing health services, including particularly a group of intermediate medical services whose ultimate health impact cannot be discerned. In this case, the question becomes how to provide a medically accepted service most efficiently, without being able to evaluate definitively (if at all) its health implications.

Illustrative of this phenomenon is the CEA/CBA literature on computed tomography (CT) scanning, the single most studied technology of
the last 2 years examined in OTA’s bibliographic search (1977-78). As observed in chapter 1, the CT scanner is the only expensive, equipment-embodied technology to have been the subject of considerable CEA/CBA attention, but it may be in the vanguard in this respect, rather than being an exception. Furthermore, the CT scanner exemplifies the difficulties involved in evaluating diagnostic procedures (360, 558, 559), an area that has been identified as deserving of much greater CEA/CBA effort (569). Despite sharing the same technology—the scanner—as a starting point, the authors of the numerous CEA/CBAs diverge significantly in their perceptions of the objectives of scanning and hence in their evaluations of its cost effectiveness. At one extreme, the diagnostic effectiveness of scanning is assumed, with no attempt to link diagnosis to either patient management or outcome; cost effectiveness is measured as the cost savings from using the CT scanner, as opposed to alternative techniques, to perform a given volume of diagnoses (211). At the other extreme, effectiveness is defined in terms of effects in disease management and patient outcome (28). The latter seems the socially most desirable concept of effectiveness, but the problems in its determination are substantial, and it misses additional benefits such as those associated with decreasing patients’ uncertainty, directing short-term patient management, and contributing to greater medical understanding (2,32).

Needless to say, the differing objectives result in widely varying assessments of the desirability of scanning.

Determining objectives for purposes of analysis is frequently regarded as a trivial exercise, but examples from the literature illustrate that it may require considerable thought and that the absence of such effort can damage the quality of analysis. A failure to appreciate the limits of a selected objective can mislead both analyst and decisionmaker. For example, when HEW analysts decided to compare the cost effectiveness of alternative disease control programs, they selected lives saved as the measure of effectiveness. This variable (and variants on it, such as life-years or quality-adjusted life years (QALYs) saved) is a common measure of health impact in CEAs, but it is not a comprehensive one. HEW analysts recognized this by observing that an arthritis control program could never be justified on the basis of lives saved, but that the program ranked as one of the better investments when the benefits associated with disability avoided were taken into account in a CBA framework (240). Fairly few health care CEAs make explicit reference to the nature of the biases their effectiveness measures introduce. It may be that many such biases have not even occurred to the analysts.

Fein (177) has noted the tendency of health care CEA/CBAs to “relegate to a footnote” a variety of nonquantifiable benefits (e. g., caring, or reduction of pain). With a reference to both intangible benefits and distributional effects that cannot be valued, he noted that as “the numbers gain currency . . . a ‘climate of opinion’ is created: that which is measured is important and vice versa.” This problem is common to analysis in virtually all areas, though its importance is probably greatest in the social welfare fields such as health, education, and justice. In some cases, though, benefits that are difficult to quantify or value have escaped even footnoting. For example, in an otherwise sophisticated CEA comparing hysterectomy and tubal ligation as sterilization alternatives, Deane and Ulene (134) ignored the preferences of the women involved. The authors carefully analyzed the direct costs of the procedures and indirect costs of complications and later disease, but the emotional reactions and feelings that might be expected to dominate many women’s decisions received no consideration in their analysis. Problems of measuring and valuing intangible benefits pervade the health care CEA/CBA literature. They are exacerbated by the failure of many analysts to identify such benefits in specifying objectives.

In recent years, there appears to have been a narrowing of problem definition in health care CEA/CBA. Accompanying this has been a reduction in the number and scope of alternatives examined through CEA/CBAs. The extreme—an analysis of a single program or procedure, with the only “alternative” being its absence—has become reasonably common in the literature. Another development, exemplified by the analyses of Eddy (157,158) and Schoenbaum, et
[472,473], represents an intermediate position between a single-program analysis and a comparison of numerous qualitatively diverse alternatives: Several analysts are using mathematical techniques to design or determine the optimal (i.e., most cost-effective) structure of a program by analyzing the effects of changes in several parameters and assumptions (e.g., compliance rates, diagnostic accuracy, therapeutic effectiveness). In essence, such analysts are examining a large number of “programs” of a single type. Even though confining analysis to a single program type implies limitations, this approach holds the promise of making significant contributions to policy understanding and program development.

ASSESSING PRODUCTION RELATIONSHIPS

Technical aspects of analysis clearly differentiate high-quality analyses from the more typical contributions. Nowhere is this more evident than in the modeling of production relationships. A summary characterization of the difference is this: The better studies carefully consider and address production issues, whereas the typical contributions adopt a “black box” approach to production (that is, they observe existing programs’ inputs and outputs and ignore current inefficiencies and predictable future changes).

In part, this difference is legitimate, reflecting the diverse purposes of analyses. Many of the better studies have a prospective, or planning, intent. The studies are oriented toward predicting the costs and benefits or effectiveness of alternative future programs, so the analysts model idealized versions of these future programs, recognizing significant variations from current similar programs when such exist (e.g., scale, efficiency, relative costs, technological change). By contrast, many of the more typical analyses have a retrospective or evaluative purpose. In these the analysts wish to assess the performance of a program in terms of its realized costs and outcomes. Even for this type of assessment, however, it is usually important to examine the black box of production so as not to attribute to inputs outcomes that occur by chance.

When the purpose is retrospective evaluation, the identification and measurement of experienced inputs and outcomes are appropriate. Often, however, it appears that authors who have a prospective planning objective in mind have not thought through the limitations of ex post evaluation. Implicit in their analyses is the assumption that existing programs are accurate models of the alternative futures. Occasionally this may be reasonable, but the assumption is fraught with hazards. Common errors in the literature include:

- failure to account for scale effects, i.e., taking an existing program (e.g., a worksite hypertension screening and treatment program) and assuming that a national program intended to serve (say) 1,000 times as many people will require 1,000 times as many inputs (i.e., costs) and produce 1,000 times the output;
- failure to consider how environmental factors might alter program inputs and outcomes (e.g., assuming that the production function for an urban worksite hypertension screening and treatment program would serve as a valid model for planning a nationwide screening and treatment effort both inside and outside of work settings);
- ignoring predictable technical changes over time (e.g., assessing the “future” of CT scanning, assuming that the technology — and hence inputs and outcomes — will not change from what exists at present);
- ignoring predictable increases over time in the efficiency of operation of a technology or program, i.e., the “learning curve” phenomenon (e.g., assuming that the performance of program personnel will not improve over time as they gain familiarity and experience);
- ignoring likely shifts in the relative costs of inputs (e.g., the price-wage rate — of labor rising more rapidly than the price of equipment) and consequent changes in the mix of inputs used;
failing to identify avoidable inefficiencies in the existing "model" program and thus inputting them to the structure and operation of future programs; such inefficiencies could reflect an inefficient input mix (e.g., having a high-priced technician perform a function which could be automated inexpensively) or an inappropriate use of technology (e.g., condemning CT scanning as exorbitantly expensive because it is used indiscriminately, rather than limiting uses to those which are medically and economically justifiable); and conversely, failing to anticipate that both the inputs and outcomes of a carefully monitored program in a major medical center may not be replicated as the program diffuses into general practice.

Most of these deficiencies of ex post evaluation for prospective planning become more severe the more novel the technology or program in question. A familiar, established, and successful program is more likely to represent a good model for planning purposes than is a new, or, especially, an experimental program. Yet a major role of a forward-looking CEA/CBA ought to be to assess the potential costs and benefits (effectiveness) of a program before it has diffused throughout the medical system.

CT serves as an excellent example of the great difficulties of undertaking useful analysis early to influence planning and decisionmaking. These difficulties span the spectrum of applications of CEA/CBA, but they are particularly severe in an area such as medicine in which technological change occurs rapidly and frequently. It was exceedingly difficult to perform an adequate analysis of CT scanning prior to its diffusion. Yet all of the studies in the literature relied on that early experience for data, and most of the early studies failed to anticipate changes that have already occurred, only a few years following publication of the studies. Furthermore, anticipated changes in radiological technology may make CT scanning technically obsolete within a few years, yet the nature and amount of relevant information are not adequate to incorporate this factor into an analysis intended to assist planning. The CEA/CBA literature on the CT scanner does not address this issue.

Formal modeling is difficult, a simple fact that may account for the lack in much of the literature of imaginative, useful characterization of production relationships. At a minimum, modeling requires talent in disciplined conceptualization; frequently, it also necessitates application of specific mathematical or formal modeling skills. The latter, in particular, are not available in abundant supply. Medical education generally includes no consideration of such skills, and few analysts with appropriate training from other disciplinary backgrounds have devoted their attention to health care CEA/CBA issues. There are, of course, notable exceptions. By example, through methodological contributions, and by direct discussion of issues, numerous authors improve both the current and future state of the art of assessing production relationships. Review articles have communicated basic principles, improving the critical abilities of readers and, one would hope authors of future studies. Specific CEA/CBAs in the literature have illustrated skillful conceptualization, use of mathematics, and formal modeling techniques (e.g., 22,122,134,157,159,479,480,516).

Mathematics and formal modeling can intimidate, impress, and confuse the uninitiated. In order to put the formalism into proper perspective, it is imperative that authors clarify the implications of both explicit and implicit assumptions in the modeling and emphasize the limitations of their studies. There is a significant risk that the uninitiated will be overly impressed with formalism, so the caveats should be more than simple disclaimers. Yet only a minority of health care CEA/CBAs have taken this requirement seriously (e.g., 122,516).

High-quality analysis of production relationships does not require sophisticated modeling efforts. A few studies have exhibited both elegant conceptualization and structural simplicity. For example, in their analysis of the national swine flu immunization program, Schoenbaum, et al. (473) considered the effects of varying acceptance rates, probability of an epidem-
ic, and other factors in a manner that was technically sound and readily understandable. Particularly in the medical literature, which is read by an audience that generally is mathematically unsophisticated, the clarity of studies such as this one probably serves to educate and to build interest in well thought out CEA/CBAS.

IDENTIFYING, MEASURING, AND VALUING BENEFITS/EFFECTIVENESS AND COSTS

Benefits/Effectiveness

A central concern of many health care CEA/CBAs, both conceptually and empirically, is adequately capturing the health consequences of programs. Only one such consequence lends itself to unassailable, objective measurement: reductions in mortality. Another common, if not universally accepted, measure of health improvement, however, is reduced days of morbidity or disability. Neither of these measures accounts for variations in the quality of the resulting days of less impaired health. Analysts have adopted a few means of adjusting for this quality factor, but to date there has been nothing approaching consensus on specific methods of adjustment.

Analysts’ inability to quantify satisfactorily certain health benefits appears to be the primary reason for their exclusion from formal calculations. A second reason is the difficulty of identifying what it is that patients seek and receive from health care. As noted above, comprehensive analyses of the tangible costs and benefits of treatment alternatives have sometimes ignored the emotional or psychological motivations that may lead patients to prefer one treatment over another, as if the patients were merely inputs into a physical production function (134). In other words, patients’ objectives and values are not limited to measurable physical health improvement, and if patients’ objectives do not represent social concerns, the very reason for considering a health program is challenged.

Obviously, the significance for an individual CEA/CBA of the inability to quantify certain benefits depends on the relative importance of those benefits in the program under consideration. Certain health problems present seemingly insurmountable barriers to the objective measurement of their benefits, yet their importance has prompted analysts to grapple with them in a CEA/CBA framework. Examples include mental retardation (99), mental illness (463), and care of the terminally ill. Without succeeding in quantifying the intangibles, the efforts of analysts to deal with problems such as these have contributed to an increased understanding of the nature of the problems and the associated programs.

For many health programs, the principal health benefits are the more tangible, or quantifiable, reductions in mortality, morbidity, and disability. Nevertheless, CEA/CBA assessment of benefits (effectiveness) is far from problem free. How does one measure and value benefits (effectiveness) in units that are commensurable with each other or with costs? Days of morbidity avoided, for example, are not directly comparable to days of mortality avoided.

In the health care CEA/CBA literature, there are three principal approaches to this problem: 1) accepting it as an unresolvable problem, and selecting a single (presumably dominant) outcome as the index of benefit or effectiveness; implicit in this approach is the assumption, or hope, that nonmeasured benefits vary proportionately and positively with the single outcome measure; 2) employing an index of health effects or of health status; and 3) adopting one of two methods of valuing major outcomes in monetary terms. The first two of these provide effectiveness measures for CEAs, while the third
yields the monetary benefit measurement needed for CBAs. A fourth approach, rarely found in the literature, would be to not force an aggregation of effects or benefits. A possible “array” method is discussed in chapter 4.

The first approach—the most common one in the literature—is the easiest to accomplish and perhaps to understand. It is also, however, the least conceptually appealing, because of its unsatisfactory (often implicit) assumption that decreases in mortality, for example, correlate highly with decreases in such factors as morbidity, pain, and suffering. A prominent example, noted earlier, comes from the mid-1960’s HEW disease control program analysis in which “lives saved” served as the proxy for all health benefits in the CEA comparison of programs. As the analysts observed, “lives saved” as the effectiveness measure relegated arthritis to the bottom of the list of cost-effective programs. When the programs were compared by means of cost-benefit calculations, however, the ability to reduce arthritis-related morbidity and disability made the arthritis control program appear quite competitive with the programs that saved the most lives (240).

The single-measure index of effectiveness continues to dominate health care CEAs, but modifications point the way toward more refined measures of health benefits. “Lives saved” is a gross but important index of effectiveness for many health programs. “Life-years saved” adds an element of quality to the nature of deaths averted. This measure has been employed in several CEAs. A further refinement involves adjusting the life-years to reflect the quality of those years. Klarman, et al. (298) provided an early example of quality adjustment in their CEA study of alternative renal disease treatments. They argued that a year of life with a well-functioning transplanted kidney was superior to a year of life on dialysis—given the time, inconvenience, and discomfort associated with the latter. Consequently, they arbitrarily valued a year of life on dialysis as equal to 0.75 year with a transplanted kidney.

The idea of quality-adjusting provided the basis for Weinstein and Stason’s (575) use of an index of health effects in their study of hypertension screening and treatment programs. Their QALY involves adding changes in life expectancy to changes in quality-adjusted life expectancy resulting from reduction in morbidity, and subtracting changes in quality-adjusted life expectancy due to iatrogenic illness and treatment-induced side effects. Selection of appropriate weights remains arbitrary and hence a problem. An earlier attempt to develop a health status index produced weights (ranging from 0 to 1) that corresponded to a spectrum from death to complete health (726). Despite the conceptual appeal of such an index, the inherent weighting problems plus the so far inadequate empirical data base have led to very limited CEA/CBA application of this type of index (79).

The third approach to valuing benefits in commensurable units is to translate all quantifiable outcomes into monetary terms—benefit measurement for CBA. A common approach, monetary assessment of benefits is also the most controversial approach in the evaluation of health care programs. The principal issue, as discussed in chapter 2, is the valuation of human life. As indicated in chapter 2, the human capital approach employs a market measure of the value of life, whereas willingness-to-pay asks how people value their own lives, subject to their ability to back up their valuations with economic resources. Willingness-to-pay has considerable conceptual appeal, but to date no one has succeeded in developing techniques to produce consistent and meaningful estimates of willingness-to-pay (4). The human capital approach has its own conceptual attributes, but with its imputation that the worth of a life is determined solely by productivity, it has fallen into disfavor among many practitioners of health care CEA/CBA. Since the human capital approach is empirically more manageable and consistent, however, the vast majority of CBAs have employed this form of valuation. This is not to suggest, however, that the approach invariably has been applied correctly.
should be measured as the costs of illness avoided. Some analysts have used existing cost-of-illness estimates as direct measures of benefit, without recognizing that many of the illnesses avoided would have occurred years into the future and hence that benefits should have been discounted. This has had the effect of inflating benefit estimates, in some cases considerably (309).

In any given study, beyond the choice of a basic approach to measuring benefits lies determination of the specific measure(s). In CEAs, the effectiveness measure is often reasonably obvious, with different analysts selecting similar measures, thereby facilitating cross-study comparisons. Treatment of kidney failure provides an example in which different analysts have selected the same measure of effectiveness—life-years saved—and despite a difference of 10 years in publication dates, their analyses have produced consistent results (298,513). For some topics, however, effectiveness measures are less obvious, with the result that different investigators have selected qualitatively distinctive measures and undertaken analyses that produced quite different and not directly comparable results. The problem seems especially relevant to the area of diagnosis (360). Its presence in the literature can be anticipated to grow if analysis of diagnostic procedures increases, as some observers believe it should (569). Resolution of the problem, if possible, may lie in imaginative efforts to translate diagnostic accuracy into effects on patient management and health outcome. Among the CT papers, only Baker and Way’s (28) attempted to do this. Their scaling of effects involved arbitrary and subjective judgments, but Baker and Way’s effort stands out as one of the few published attempts to bridge the diagnosis-health outcome gap.

The literature includes few examples of such efforts to grapple with challenging assessment problems. Nevertheless, other approaches have been adopted. A few studies identify and array noncomparable measures of effects, including rank-ordered ones (148). The argument underlying this approach is that if effects are important but cannot be measured in a common metric, decisionmakers will find it more useful and less misleading to see them arrayed in an “unfinished” CEA (i.e., one lacking a “bottom line” cost-effectiveness ratio) than to have one or more of them dropped for the sake of calculating a “final” cost-effectiveness ratio. Despite its “incompleteness,” the CEA by Doherty, et al. (148), for example, contributed information and structure which can facilitate understanding of a policy issue.

Consistent definition of effectiveness can vary across health care functions. For example, mental retardation illustrates a substantive health problem for which assessments of prevention v. treatment necessarily involve quite different, noncomparable measures of effectiveness. Prevention of retardation (e.g., through phenylketonuria (PKU) screening), is commonly valued in a cost-benefit framework for its ability to avoid expenses of institutionalization and other care by preventing the birth of retarded children; that is, the benefits of the program are future costs avoided (e.g., 78,79,482). By contrast, many of the desired effects of programs providing care for an existing group of the retarded are less tangible and less economically oriented; the “costs avoided” metric is clearly inadequate (99). Obviously, the prevention-treatment effectiveness distinction is by no means universally applicable. Analysts have successfully relied on a consistent outcome measure in comparisons of prevention and treatment alternatives for kidney disease (327), myocardial infarction (4,122), and others.

In addition to addressing all of the problems noted above, analysts must identify and acquire data needed to measure benefits. The quality of data is rarely examined carefully in health care CEA/CBAs, yet it is a common constraining factor across most studies. Obviously, assessment of the health outcomes of a variety of procedures depends on the availability of valid, reliable experimental or epidemiological data; such data do not always exist, and even when they do, they are not always accessible. Benefit valuation for CBA requires in addition that such data be translated into their economic implications. The methods for doing this are conceptually clear, and solid empirical assessment of the costs of illness has been performed (385,721,
Nevertheless, there are significant variations from one study to the next, implying that use of differing estimation procedures and data could compound spurious variations in benefit estimates introduced by the use of different health-outcome data sources. The issue of the reliability and validity of cost-of-illness estimates is currently under study in a contract funded by the National Center for Health Services Research. The National Institutes of Health is in the process of publishing a bibliography of some 2,000 references relating to cost-of-illness estimation.

Data for several health care CEA/CBAs have relied on subjective rankings, surveys, and the like (4,28,78,79). The issue of how valid and reliable such data can be has yet to be resolved, though several studies find considerable variation depending on how survey questions are phrased (4,550). Clearly, conceptual and empirical work on benefit assessment measures is a pressing need in health care CEA/CBA (569).

Analysts in the United States have access to more numerous and varied data sources than do investigators in many other countries. Compared to the ideal, however, even U.S. data sources exhibit serious deficiencies. Many surveys are plagued by poor methods, producing unreliable data that contribute to misleading analyses (565,740). The national data collection effort is hindered by considerable duplication of effort and inconsistency across data sets, both resulting in part from a lack of interagency coordination. The development of better organized and planned basic data collection is essential to improving the quality of health care CEA/CBA.

Two remaining benefit assessment issues are noted here, with discussion deferred to later sections. One is the practice of discounting benefits, occasionally handled well in the literature, frequently ignored. The other is analysts’ handling of distributional or equity concerns.

Costs

Apart from the problems of measuring and valuating benefits/effectiveness, the literature reveals numerous examples of poor or inaccurate measurement and valuation of costs. Deficiencies associated with cost assessment are frequently more insidious than those associated with benefit assessment, because authors commonly devote less attraction to them. Since authors often do not discuss cost analysis problems, they fail to alert readers to them; furthermore, the analysts themselves in many cases seem unaware of the deficiencies of their approach, data sources, etc.

Costs are a reflection of resources consumed. Thus, many of the difficulties that have plagued cost assessment are perfectly analogous to those discussed above in the examination of analysts’ handling of production relationships. Rather than repeat that discussion, this section simply notes several common problems: 1) often analysts have measured realized (ex post) costs in an analysis intended for prospective planning without allowing for learning, technical, and economic changes which seem likely to occur; 2) they have failed to distinguish the cost implications of running programs under optimal v. average conditions; 3) they have not always accounted for the differential valuation of costs occurring at different points of time (the discounting problem, discussed in the next section).

Just as there are problems unique to benefit assessment (e.g., valuation of lives saved), certain problems hinder cost analysis in particular. Chief among these in the health care literature is the use of inaccurate or inadequate proxies for true costs—a significant problem because of its pervasiveness and, evidently, the failure of many investigators even to be aware of it. A major source of inaccuracy is the use of market prices as measures of costs. The assumption that prices closely mirror true costs seems reasonable in some smoothly functioning markets, but market imperfections can distort the relationship between input prices and their true opportunity costs. This common problem reaches its extreme—and hence introduces the most significant distortions—in cost assessment in health care.
care programs, particularly those associated with hospitals. In health care CEA/CBAs, the use of prices in lieu of true opportunity costs generally means adoption of published charges (e.g., from hospital billings or insurance charges) as the index of cost. Although occasionally analysts recognize that charges may not accurately mirror costs, very often the problem is not even acknowledged (92). The vast majority of health care CEA/CBAs employ charges uncritically, frequently introducing potentially large errors in the estimation of the true costs of the programs in question. At a minimum, analysts ought to explore the relationships between charges and actual market costs.

Inadequate cost assessment often results from failure to take into account costs which are real but hidden. For example, very few health care CEA/CBAs account explicitly for the costs of patients’ time traveling to medical facilities and waiting for and receiving services. CBAs occasionally capture some of this by valuing lost productivity, but, most commonly, lost productivity measurement relates only to days of morbidity, disability, or mortality avoided, and not to hours involved in seeking and receiving care; and lost productivity is not the only time cost associated with health care services. This problem is exacerbated, however, by the fact that many employees are covered (e.g., through sick leave) for time off from work for medical visits. Thus, neither the physician nor the patient perceives the time as “lost,” and analysts sensitive to the time-cost issues might overlook the fact that the time imposes real costs on society (e.g., physical productivity lost).

A second example of real costs that have escaped attention in health care CEA/CBAs is the value of volunteers’ time mentioned in chapter 2.

An unresolved cost assessment issue is whether analysts ought to assume efficiency in program operation or build in “slack” for likely inefficiencies. The former is appropriate for evaluating the ideal, but the latter seems more likely to reflect what will come to pass should the program be implemented. This issue has received virtually no attention in the empirical literature. Common practice has been to measure resources used in programs, rather than to identify efficient resource use, but only a few studies suggest that the investigators have even contemplated the difference.

A technical cost issue of considerable importance derives directly from the discussion in an earlier section of the relative lack of attempts by analysts to distinguish marginal from average resource consumption. Most commonly, authors have used average total costs of existing programs to predict the costs of program expansion, modification, etc. When capital costs are substantial or marginal costs vary significantly, failure to distinguish marginal from average costs can produce, and often has produced, misleading cost estimates. Although some analysts have demonstrated sensitivity to the distinction, direct extrapolation from average costs dominates the health care CEA/CBA literature.

As in the case of benefit measurement, data availability and quality problems hinder effective cost analysis. For example, data on charges are relatively accessible, but many cost data (in particular, data needed to reflect opportunity costs) are not. The current interest in cost containment has promoted governmental efforts to acquire more and better cost information, but the acquisition and appropriate use of cost data will linger as a major problem in CEA/CBA for years to come.

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'That people value such lost time is demonstrated by the willingness of many individuals to accept significant charges from private physicians in lieu of waiting a long time in lower cost medical clinics. The waiting-time mechanism of rationing medical services is highly inefficient socially, producing a “deadweight loss,” that is, patients lose their free time, and no one gains directly from that loss.
VALUING BENEFITS AND COSTS OVER TIME: DISCOUNTING

The discounting of benefits and costs realized over time is one of the most technical features of CEA/CBA. It is also one of the principal sources of analytical weakness in the health care literature. Owing to the potentially profound influence of discounting on valuation of costs and benefits (effectiveness), the absence of discounting in numerous health care analyses severely discredits those analyses. In addition, the failure of some investigators to test the sensitivity of their findings to the value of the discount rate raises questions about the robustness of those findings. (Sensitivity analysis is discussed in the next section of this chapter.)

The literature is replete with examples of the total absence of discounting. In the studies that have employed discounting, the basic method is generally sound; that is, discounting has been done either technically well or not at all. However, only a minority of the studies that have used discounting have included examination of the effects of the value of the discount rate on the bottom line regarding net benefits or cost effectiveness. Yet, as several of these studies demonstrate, when significant realization of costs or benefits occurs well into the future, the discount rate selected and the method of discounting can play pivotal roles in determination of a program’s value (513,572). For example, in her study comparing programs to treat or prevent myocardial infarction, Cretin (122) tested the sensitivity of her cost-effectiveness estimates to variations in the discount rate. The prevention program—screening of school-age children for high cholesterol—necessarily involved benefits deferred well into the future. With costs and benefits undiscounted, the net cost per year of life saved ranged from $2,441 to $2,855, depending on assumptions. Discounting at 5 percent produced a cost per year of life saved of $9,353 to $12,640. At 10 percent, discounting caused the figures to leap to $66,660 to $94,460. These estimates compared with a range of $1,782 to $6,100 per year of life saved by treatment alternatives, depending on the program and the discount rate. Cretin’s article not only demonstrated the proper application of discounting, but it emphasized the dramatic effect that varying the discount rate can have on net cost estimation and hence on comparison of program alternatives.

A general CEA/CBA discounting question has received attention in the recent health care literature: Should effectiveness measures be discounted? Empirically, the question has been answered in the affirmative by Cretin (122), Stange and Sumner (513), and Weinstein and Stason (573), each of whom discounted effectiveness measures of mortality avoided in the future. The logic of discounting effectiveness is quite appealing, but the practice is fairly novel.

ADDRESSING PROBLEMS OF UNCERTAINTY: SENSITIVITY ANALYSIS

The discount rate is only one of numerous potentially significant influences on the magnitude of cost and benefit (effectiveness) estimates. As noted in chapter 2, it is a rare study that can be carried from conception to empirical conclusion without the necessity of the analyst’s making...
assumptions to substitute for uncertainties, data unavailability, conceptual problems, and so on. Despite this, it is not common practice in health care CEA/CBAs to test the significance of assumptions. Frequently, analysts do not carefully distinguish between assumptions and sound empirical observations.

Of the possible uses of sensitivity analysis to address uncertainties, only one has been applied with any degree of frequency in the literature: the direct testing of findings to determine if they are sensitive to important assumptions. Even this most common application of sensitivity analysis has been used rather infrequently, and with a few notable exceptions, it has been used primarily for testing sensitivity to discount rates. The ability of sensitivity analysis to determine whether a major uncertainty precludes a definitive analysis does not appear to have encouraged analysts to tackle health care evaluation problems in which such uncertainties were obvious at the outset. Nor have analysts used measurable costs and benefits to establish minimum or maximum values for quantified variables in order for a program to appear worthwhile. However, a few studies have approximated such uses of analysis. Centerwall and Criqui's (86) assessment of thiamine fortification of alcoholic beverages allowed them to avoid valuing health benefits, since net cost savings were positive.

Cretin’s (122) testing of the sensitivity of findings to variations in the discount rate illustrates art appropriate use of sensitivity analysis in its most common application. As Cretin’s analysis demonstrated, program evaluation is highly sensitive to discounting when significant benefits (or costs) are deferred well into the future, a characteristic of many prevention programs. Discounting the costs of the cholesterol screening program by 10 percent instead of 5 percent increased costs per year of life by over 600 percent; for the treatment alternatives, the benefits of which are more immediate, however, the corresponding increase in costs was on the order of 50 percent. The potential for such dramatic differences explains why “responsible analysts usually offer the user of analysis a sensitivity analysis with respect to the discount rate used” (569).

The authors of several of the more highly regarded studies have also tested the sensitivity of findings to other uncertainties. For many health care programs, patient acceptance or compliance is both a crucial variable and uncertainty, and hence a worthy candidate for sensitivity testing. The literature provides several examples. Schoenbaum, et al. (473) examined the effect of acceptance on the optimal structuring of the national swine flu immunization program. Eddy’s (158) analysis of breast cancer screening also related such factors to program design. Weinstein and Stason’s (573) study of hypertension control demonstrated how patient compliance can influence the outcomes of a CEA of disease management.

The literature offers only a few examples of sensitivity analysis applied to other cost and benefit (effectiveness) estimations, but those few are instructive. For example, LeSourd, et al. (327) found that the absolute magnitudes of individual benefit-cost ratios of kidney disease control alternatives were quite sensitive to variations in program size, target screening group, etc., but the relative rankings of the major programmatic alternatives (e.g., screening v. treatment, and within the latter, transplantation v. center dialysis v. home dialysis) were unaffected by the tested variations. In addition to testing sensitivity to the discount rate, Cretin (122) included high and low direct cost estimates for the screening program. The analysis demonstrated less sensitivity to the direct cost estimation than to discounting.

The use of sensitivity analysis reflects a more sophisticated appreciation of CEA/CBA than that which characterizes most of the existing health care literature. At one level, inclusion of thoughtful sensitivity analysis multiplies the number of figures in an analysis and can add considerable complexity to the presentation and interpretation of findings. However, both logic and empirical evidence indicate that the assumptions of an analysis can affect results significantly. Thus, both the credibility and useful-
Examinng Ethical Issues

As noted earlier, one of the limitations of CEA/CBA is its inability to handle distributional issues. Health care CEA/CBAs rarely have grappled seriously with distributional issues. Obviously, selection of a topic to study may be an implicit statement of concern with distributional issues, though it is expressed in terms of the objective of the analysis rather than as an analytical variable in the CEA/CBA (e.g., the relatively large CEA/CBA literature on mental illness or geriatric services). Health care CEA/CBA should not be singled out for its failure to incorporate distributional considerations successfully. This is the general state-of-the-art and perhaps reflects one of the inherent limitations of this form of analysis. But emphasis on this limitation is particularly important in the health care literature where a readership relatively unfamiliar with the techniques of this form of analysis, though this situation is changing.

The literature offers few examples of attempts to address the problem of differentially valuing the costs and benefits accruing to different groups of people directly. Nevertheless, the equity concern most often debated in the literature—the valuation of life—clearly relates to this fundamental problem, for in CBA benefits are estimated according to one’s productivity (the human capital approach) or affluence (the willingness-to-pay approach). Less often recognized is that CEA effectiveness measures presumed to be “value-free” generally imply values. The trend of the literature away from CBA and toward CEA may reflect growing distaste for explicit valuation of life or the belief that both conceptual and empirical limitations make the effort a “quixotic quest for a value of life” (569). Of course, alternatively, or in addition, the growing preference for CEA may simply reflect the fact that CEA is easier to understand and perform.

The appropriate handling of distributional issues remains one of the least developed features of CEA/CBA in the health care literature and elsewhere. Even though both theoretical and empirical progress can be anticipated (569), the major problems of dealing with equity concerns seem unlikely to be resolved in the foreseeable future.

Presenting and Interpreting Findings

As emphasized throughout this report, two factors place a major responsibility on analysts to present and interpret their findings carefully and clearly: 1) technical limitations (inherent in analysis or in the abilities of particular analysts) often seriously restrict the possibility of arriving at unequivocal, definitive conclusions; 2) the readership of health care CEA/CBA is generally unsophisticated about the techniques of this form of analysis, though this situation is chang-
In addition, numerous readers will focus on, if not limit their attention to, the abstracts and conclusions of articles.

An overall assessment of the health care CEA/CBA literature suggests that relatively few analysts have addressed this responsibility successfully. Those few are generally the authors of the studies identified as technically high quality. Of course, the handling of the presentation and interpretation of findings is a characteristic against which the quality of analyses is judged, but it appears that a thoughtful, useful conclusion to an analysis tends to follow a technically and conceptually well-conceived study. Examples abound. The analysis by Schoenbaum, et al. (473) clearly identified factors that could influence the success and optimal structure of the national swine flu immunization program. Cretin’s (122) concluding analysis and remarks clarified the crucial role of discounting and demonstrated the need to interpret the sensitivity analysis. Cretin purposefully and constructively made it impossible for the reader to conclude that there was an obvious “best” approach to reducing the toll of myocardial infarctions. Stason and Weinstein (516) discussed how compliance and a variety of other factors could affect their conclusions, though Fein (177) still found it necessary to emphasize limitations. Doherty, et al. (148) emphasized information organization and presentation in their assessment of health programs for the elderly; they refused to “reduce” their analysis to a “bottom line.” The authors of all such studies seem to be motivated by “the philosophy that it is not so much the results of a (CBA/CEA) that are likely to have an impact on policy as the process of structuring information in a systematic framework that highlights the key uncertainties and the most important value tradeoffs” (569). This is inevitably reflected in these analysts’ presentation and interpretation of their findings.

By contrast, most health care CEA/CBAs seem oriented toward a “bottom line”—generally the estimation of a benefit-cost or cost-effectiveness ratio. Aside from questions of measurement underlying the cost and benefit (effectiveness) components of these ratios, even this basic “bottom line” has been technically misinterpreted in numerous studies. At the extreme, at least one article with a title beginning “Cost-Benefit Ratio . . .” does not contain a single cost-benefit ratio. Few analysts exhibit awareness of the deficiencies of a benefit-cost ratio as compared with a measure of net benefits. The benefit-cost ratio clearly dominates in empirical health care CBAs.

Cost-effectiveness ratios, and the words “cost effective,” are employed even more uncritically than are benefit-cost ratios in CBAs. In many articles, “cost effective” refers to one of the two words but not both: That is, some authors have employed “cost effective” when they mean that a program or technology is effective, irrespective of cost; and other authors have used “cost effective” to connote “cheap,” irrespective of effectiveness. There are several instances of purported CEAs in which only a single program or technology is examined and is then adduced as being cost effective, despite the absence of an alternative against which to compare it (133).

Subtleties of technical interpretation of CEA/CBA “bottom lines” largely have escaped attention in the health care literature. Only a few analyses demonstrated awareness that “cost effectiveness” of a use of a technology need not imply overall cost effectiveness. For example, in certain delivery settings, an automated electrocardiograph (EKG) may be more cost effective than a manually read EKG, but if the ease and availability of the former lead to excessive use, the national EKG bill might actually rise without necessarily contributing to improved health (18). Perhaps the most dramatic demonstration of the difference between average and marginal cost effectiveness was Neuhauser and Lewicki’s (397) estimation that the cost per additional case of colon cancer found by repeated stool guaiac rose from under $1,200 for the first stool guaiac to $47 million for the sixth!

Even when the use of a ratio or net benefit measure is technically correct, lost in such a number are the assumptions that underlie it and the intangible unmeasured costs and effectiveness that are excluded from it. A few studies have presented results in a manner that makes these factors clearer. The most common strategy has been careful discussion of how the “bot-
bottom line" could be affected by such factors. Alternatively, some authors have presented ranges of results reflecting sensitivity to assumptions (122,327). A third approach, less commonly adopted, has been to step back from the bottom line and provide a tabular display of programs and their (noncommensurable) effects. This approach does not yield a conclusion as to which of several competing programs is the “best,” but it does array alternative sets of consequences effectively and thereby might aid decisionmakers by clarifying tradeoffs (148).

**LINKING ANALYSIS TO POLICY IMPLEMENTATION**

In health care studies as elsewhere, the gap between CEA/CBA studies and policy formulation almost invariably has been bridged by a leap of faith that assumes a theoretically desirable program can be translated readily and directly into an operational one. Health care CEA/CBAs always have had a policy orientation, but the literature is nearly devoid of empirical attempts to make the adjustments (needed to reflect political and cost realities) proposed in the new literature linking analysis to policy implementation (336). Health care CEA/CBA perhaps should not be faulted for this lack; the implementation literature is simply too new.

Luft (336) in a contribution to the implementation literature, used two health care examples, development of freestanding surgicenters and use of work evaluation units “for objective testing of functional work capacity to supplement the usual information concerning the health status of patients who have recently had a myocardial infarction.” Through these examples, he demonstrated how role players’ differing interests can block implementation of socially desirable programs, and how analysts can use recognition of differing interests and influences in developing predictive CBAs. Empirical application of this important conceptual contribution might increase the realism and usefulness of CEA/CBAs.

**CONCLUSION**

The assessment in this chapter of the quality of the health care CEA/CBA literature has relied primarily on judgments of how the practice of analysis compares with a set of theoretical standards. Two caveats related to this approach must be recognized. One is that words like “cost effective” have been used in the literature much more freely than they would have been had all
authors meant to adhere to strict CEA/CBA definitions. Nevertheless, since articles employing these words freely contribute to the health care community’s perception of the meaning of terms and uses of analysis, it is appropriate to include them in a review of CEA/CBA literature.

The second caveat is that several of the standards of ideal (or idealized) analysis may be unattainable. If so, a review of the literature will necessarily have a critical tone. Many of the flaws of the health care CEA/CBA literature reflect inherent, or at least very common, analytical problems. Examples include difficulties incorporating distributional concerns into formal analysis and deficiencies of data accessibility, quality, and consistency. Some common CEA/CBA problems impose unusually severe burdens on health care studies. The difficult and often controversial valuation of less tangible costs and benefits, such as the saving of life and reduction of physical suffering and emotional distress, is often central to the health care analyst’s chore. Even more basically, the estimation of production relationships seems particularly challenging in health care, where the difficulty of attributing health outcomes to health care inputs has led many scholars to rely for evaluation on intermediate (nonoutput) measures such as structure and process. Technical change occurs with such extraordinary rapidity that forward-looking health care CEA/CBA are particularly handicapped. Furthermore, even some commonly accepted “second-best” CEA/CBA practices are hard to justify in health care CEA/CBA, one example being the use of market prices as measures of true opportunity costs.

Not all of the flaws in the health care literature are attributable to inherent difficulties. The relative novelty of CEA/CBA in health care seems to account for the exaggerated importance of several errors. Representative are the absence or mishandling of discounting and the presentation of purported CEAs that examine only one program (i.e., no alternatives) and conclude that it is cost effective. More significant is the tendency of investigators to use purely retrospective evaluation of existing programs to develop policy proposals for the future, with little or no regard for the changes that will transform the structure and functioning of such programs. Many studies are plagued further by the “black box” approach to ascertaining production relationships: The identification of inputs and outputs without devoting sufficient attention to the efficiency of production, or even to basic questions of causation versus correlation.

By contrast, the best of health care CEA/CBA makes the novelty of the literature a source of encouragement. A handful of skilled analysts are breaking methodological and substantive ground, working on evaluative techniques, and producing informative, thought-provoking analyses. In recent years, investigators have demonstrated how analysis can yield insight into the nature of timely policy issues (473), contribute to efficient program planning (158), grapple with technical evaluation problems (573), and address inadequately studied technical aspects of medicine, such as diagnosis (360, 559). Such works may presage a variety of interesting, useful developments in a field whose novelty provides a set of wide-open methodological and substantive opportunities.

Illustrative of recent methodological developments of considerable promise is the growing analytical comprehensiveness of CEAs and the trend away from comparing direct program costs with single-outcome measures of effectiveness (e.g., “lives saved”). Recent efforts to incorporate indirect costs and develop more inclusive indexes of effectiveness (e.g., QALYs) have begun to transfer a major virtue of CBA—its comprehensiveness—to CEA, while greatly reducing the accompanying problem of explicitly valuing noneconomic health benefits. Several studies demonstrate comprehensive cost accounting, with both positive costs and “negative costs”—indirect economic benefits—aggregated

\(^{1}\)The analysis of the national swine flu immunization program (473) was conceived, in part, as an experiment to see whether a formal analysis, relying heavily on (and perhaps in part opinion through use of a Delphi) could be accomplished quickly—prior to a policy decision—and still produce useful information. Despite limitations—failure to anticipate social, legal, and medical problems and their economic sequelae—the analysis served to inform and put issues into perspective for much of the health care community.
on the cost side of the CEA equation. The remaining noneconomic values constitute the programs’ effectiveness. In some instances, the remaining effectiveness measure is a simple single outcome —sterilization, for example (134) — while in others it is a more complex index, such as QALYs in hypertension control (573). In still others, effectiveness measurement or valuation is made irrelevant by the fact that complete cost accounting indicates a positive net benefit before “remaining effectiveness” is taken into account (86). The narrowing of the gap between CBA and CEA is made vividly clear by this last case. It is also interesting to note that Cretin (122) called her study a CBA, yet she did not place a dollar value on years of life saved and she presented results in terms of costs per added years of life—a typical CEA “bottom line.” One might be tempted to dismiss this as a case of mislabeling, but in fact the growing economic sophistication and comprehensiveness of CEAs have introduced a healthy terminological ambiguity.

OTA’s assessment of the quality of the literature has relied on a comparison of practice to a set of theoretical standards. Nevertheless, there are other bases for assessment of quality. For example, if one believes that quality is best reflected in the validity and reliability of results, one might seek internal or external measures of validity and reliability. An example of an internal measure is comparison of findings across studies of the same topic. To be sure, one must be wary of one study’s replicating the method of earlier studies, or of use of the same data sources leading to a shared bias (i.e., consistent but not valid results). In the absence of a shared bias, however, consistency of results is suggestive of meaningful findings.

The literature does provide a few cases of multiple analyses of a single subject. Studies of renal disease treatment offer an excellent example. Two contemporary analyses ranked treatment alternatives in the same order —transplantation being most cost effective in one study (298) and cost beneficial in the other (327), followed in both studies by home dialysis, and last, center dialysis. These results were confirmed in a study published 10 years later using more recent data (513). Similarly, three separate studies of PKU screening concluded that this is a socially desirable medical practice (78,517,553). By contrast, analyses of CT scanning have produced widely discrepant findings, reflecting differences between head and body scanning, technical changes (realized and anticipated) over the time period covered by the studies, and differences in investigators’ perspectives as to what constitutes effectiveness in scanning or, more generally, in diagnosis (2,28,211). Although a systematic comparison of analyses on single subjects was not attempted in this review, that might prove to be an enlightening approach to evaluating the literature.

Assessment of the quality of individual contributions to the literature has received primary attention in this chapter. Chapter 1 and appendix A examined the overall composition of the literature, but “quality” judgments were limited to observation of the conspicuous absence of certain substantive concerns, such as important disease problems (e.g., diabetes) and medical techniques (e.g., a large number of diagnostic techniques other than screening). Here it should be noted that an interesting indication of the overall composition of the literature is the mix of CEA/CBAs with positive and negative findings. If some medical practices are socially and economically desirable and others undesirable (or of questionable desirability), one might expect a “balanced” literature to include a good mix of positive and negative findings. A lack of balance certainly need not reflect poorly done individual studies. Rather, it might result from analysts’ having a systematic bias in favor of studying desirable or undesirable programs. For example, if CEA/CBA were applied primarily to analyzing programs whose worth has been challenged, one might anticipate a preponderance of negative findings in the health care CEA/CBA literature. A preponderance of positive findings could follow from medical professionals’ analyzing (or commissioning analyses of) projects whose diffusion into practice they favor. Dominance of either positive or negative findings might reflect systematic underestimation or overestimation of either benefits or costs. For example, as discussed above, few
analyses include a realistic assessment of the costs of implementing a policy and of the possible dilution of benefits that may follow. These factors should produce overly optimistic results, i.e., they introduce a distinct bias toward positive findings. On the other hand, many health care programs are characterized by important intangible benefits, the value of which frequently is not incorporated into analysis. This factor introduces a bias toward unduly negative findings.

The reading of the literature suggests a dominance of studies having positive findings. To be sure, there are notable exceptions, with some analyses producing distinctly negative findings (28,365,397,570) and surprisingly few deriving equivocal results (11). Also, there may be a shift taking place, with movement from the positive toward the negative. This could reflect the general questioning of medical technology and growth of cost consciousness, both of which emerged strongly in the 1970’s.

Chapter 1 noted that this report was restricted to considering personal health care services. In concluding this review of the literature, it seems appropriate to observe that the community of health care CEA/CBA analysts seems to have established a similar boundary. Unless policymakers and analysts remain cognizant of the existence of that border and its implications, this limitation can mislead technical aspects of analysis and, more importantly, reinforce narrower views of health resource allocation. A prominent example of a technical problem is the recent emphasis on measuring “net health-care cost” in CEAs (574). The socially relevant concept should perhaps be net social cost, in which net health-care cost is but one important component.

One of the drawbacks that can arise from limiting policy analysis to medical care parochialism is a failure to explore the possibility of cost-effective alternatives to personal health services. In the effort to reduce mortality and disability due to motor vehicle accidents, how might high-way safety efforts—technical (e.g., safer road surfaces and shoulder barriers), legal (e.g., increased law enforcement), etc.—compare with improved emergency medical services? To reduce hypertension-related mortality and morbidity, what is the appropriate mix of medical interventions and community health education on risk avoidance? There is a paucity of comparative analyses across the medical-nonmedical, or personal health-public health, border. A noteworthy exception is comparison of community water fluoridation with a variety of individual treatment approaches to preventing dental caries. Noting this paucity is not meant to reflect adversely on either existing or future individual contributions to the health care literature—the quantity and importance of analyses of specific medical problems and technologies is sure to grow, a development to be desired. Rather, it is to suggest that policymakers, health planners, and individual health practitioners would benefit from the widening of perspective that “border-crossing” analyses could offer.

Although OTA’s literature search discovered a preponderance of studies with positive findings, there appeared to be many more studies with negative findings than with ambiguous findings. Those with ambiguous findings tend to be competence analyses, their ambiguity often reflecting allowance for variation in uncertain parameters (e.g., 122).
4

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INTRODUCTION

OTA’s assessment discovered no consensus among analysts and practitioners as to a standard method of cost-effectiveness analysis/cost-benefit analysis (CEA/CBA), although it did find agreement that no one method is appropriate for any two classes of technologies or for any two situations under which a technology is being assessed. In general, the disagreement on precise methodology is due more to the inherent nature of the analysis, the type and stage of development of the technology being analyzed, and the general social and political environment of decisionmaking than it is to the maturity of the methodology of CEA/CBA.

OTA believes that the fundamental approach to CEA/CBA should be based on clear, logical thinking, using explicit criteria within the framework of generally accepted methodological principles. OTA also considers the distinction between CEA and CBA to be largely academic and believes that valuation of intangibles, such as life and quality of life, should be governed more by factors external to the analysis than by the methodological approach itself.

This latter point requires further comment. During the Case Study Authors’ Workshop, conducted as part of this assessment, it was agreed that certain aspects of the method which is chosen for a given analysis will be governed by the intended audience. This finding, in effect, implies that CEA/CBA is subject to systematic methodological bias. One example of such bias would be limiting the scope of a study to compare certain alternatives but not others, e.g., comparing respiratory therapy treatments with each other but not including the option of no treatment (468). Another example would be considering some effects or benefits but not others, e.g., examining direct economic costs and benefits of alternative therapies but not examining convenience or anxiety factors (304).

Such systematic bias is not wrong methodologically; rather, it is a reflection of the fact that CEA/CBA is often part of a political process. For instance, if a health systems agency wishes to assess the value of an alcoholism program, or if alcohol/drug abuse proponents wish to argue for increasing funding for their programs, it is legitimate—methodologically and politically—to estimate net societal economic gain, including increased productivity (i.e., lost wages averted). On the other hand, if the Health Care Financing Administration is trying to determine whether, and for whom, artificial heart surgery or bone marrow transplant should be reimbursed, the use of increased productivity as a criterion may be less acceptable politically.

A related consideration is whether an analysis is being used to propose increased funding for a new or an existing program, or whether it is being used to recommend curtailing of an existing program. In the former case, almost any factor which helps to make the case for increased funding is politically acceptable—including increased wages of a more productive population. In the latter case—curtailing a health program—it is often unacceptable to use net changes in wages as a criterion, since many feel that programs should not be denied on the basis, even in part, of a person’s potential earning ability. OTA’s finding, therefore, is that since there are a variety of acceptable ways to perform a CEA/CBA, and since the results of an analysis often are affected by the methods chosen, it is very important that the process of the analysis be explicit in order to allow for public scrutiny. In a sense, the process of a CEA/CBA may be more important than the results.

In addition, OTA finds a paucity of—and consequently a need for—improved data, without which good analyses are impossible. For example, efficacy and effectiveness information for many technologies is generally not avail-
able; health care utilization data are often either not available or not available in a standard or accessible format; and cost data are often inaccurate and also nonstandardized. Members of the advisory panel for the assessment and case study authors expressed the conviction that since each specific analysis often requires a unique data set that will not be available in even the best of routine data collection systems, better routine data collection—although desirable and possibly necessary for better analyses—is ordinarily not sufficient. Therefore, an optimum mix of routine data collection and study-specific data collection needs to be defined, and when studies are funded, attention should be given to include funds for data collection.

The National Center for Health Statistics (NCHS) is an agency within the Office of the Assistant Secretary for Health of the Department of Health and Human Services and is one of the principal health services research agencies of the Federal Government. NCHS has played a major role in the development of national health statistics policy and programs. Under its current mandate—the Health Services Research, Health Statistics, and Medical Libraries Act of 1974 (Public Law 93-353)—NCHS is responsible for collecting and disseminating health data including information on the costs of illness, health care, and health financing.

The importance of data collected by NCHS cannot be overemphasized. Such information as incidence and prevalence rates of diseases, natural history of disease, medical care utilization, work loss, surgical rates, and premature mortality is crucial to CEA/CBA. NCHS has conducted cost-of-illness studies, and these have been used in other agencies’ CBAs and CEs.

Notwithstanding the considerable progress in solving these methodological issues (e.g., 708, 711,732,733), OTA finds, with notable exceptions (406,516), considerable reluctance within the general health care research community to accept the validity of HSIs. Part of this reluctance seems to be related to the immaturity of the research effort. For instance, there are several concurrent research efforts underway to develop an HSI, each method being related but still quite different from the others. Also, as noted above, reliability and validity studies are still underway. The other major reason for the reluctance of the research community to accept and use HSIs seems to be a lack of understanding of the techniques. For instance, although most serious CEA/CBA analysts are aware of the HSI literature and of its potential in their own work, evidently, very few of them have assessed for themselves its validity. Consequently, in their own writing most researchers are content to acknowledge the HSI research underway, but few feel confident in actually using it. In summary, OTA finds that research efforts to develop indexes of health are producing im-
portant results, but that these efforts have neither been fully evaluated nor widely accepted by the applied research community. The lack of acceptance is probably more related to the immaturity of the field and to the neglect of evaluation than to a rejection of the methodology. Further study of the validity and usefulness of HSIs appears to be warranted.

**METHODOLOGICAL LIMITATIONS**

The methodological weaknesses or shortcomings of CEA/CBA are of two general types: 1) those that are inherent in this form of analysis, and 2) those that are due to the lack of maturity in the state of the art of CEA/CBA and to the lack of analyst expertise and experience with CEA/CBA in health care. The latter can be expected to diminish as more experience accumulates. The 10 principles for analysis presented later in this chapter are directly relevant to lessening what will be referred to below as “weaknesses due to immaturity.” The “weaknesses inherent in CEA/CBA,” however, are likely to remain significant barriers to the usefulness of CEA/CBA in health care decisionmaking.

**Weaknesses Inherent in CEA/CBA**

Examples of weaknesses which are considered as inherent are: 1) the difficulty of predicting with precision the costs and benefits of new or not yet existing programs or technologies, 2) fundamental problems in quantifying or valuing certain important but less tangible health benefits, 3) controversy over the appropriate discount rate, 4) the inability of analyses to adequately incorporate equity considerations, and
5) the inevitability of significant uncertainty of important variables even in a perfectly managed study. In addition, the rapidity and profundity of change in technological medicine exacerbate the analytical process, a problem felt particularly acutely because the point at which an analysis might have the most significant impact on health resource allocation—before a technology has diffused into widespread medical practice—is also the point at which uncertainties are most often encountered. Although sensitivity analysis sometimes can demonstrate that inherent technical analytical problems do not affect qualitative conclusions, nevertheless such difficulties frequently preclude a definitive assessment of a program. In any case, the uncertainties which pervade analyses severely restrict the potential of studies, however high quality, to resolve definitively the “close calls” in which alternative programs are similar in both cost and effectiveness.

Another inherent weakness, discussed earlier, concerns the systematic methodological bias which results when CEA/CBA studies are tailored to consider certain costs and benefits/effectiveness and not others. Such bias, due either to political considerations or to the type and stage of the technology being evaluated, is inevitable.

Weaknesses Due to Immaturity

Many of the problems associated with the application of CEA/CBA in the health field are due to the relative newness of the technique. In some cases, the problems stem from a lack of agreement among the research community (e.g., concerning the precise specification of costs, the inclusion of future medical costs saved). In other cases, sufficient information is unavailable (e.g., population-based utilization data are not known, or efficacy and safety are unknown). Also related to the relative newness of CEA/CBA is the finding that the number of studies demanded is greater than the number analysts can perform. Consequently, insufficiently trained program staff, health care practitioners, and public policy analysts are doing analyses—often failing to follow generally accepted, but until now not widely disseminated, principles of analysis (e.g., discounting costs and benefits, performing sensitivity analysis, identifying alternative programs, and measuring opportunity costs).

Although there are fairly few examples of technically high-quality CEA/CBA studies in the health literature today, this situation may change as the state of the art of CEA/CBA matures and as analysts and decisionmakers gain more experience with CEA/CBA in health care. There should be a reduction in the number of problems due to immaturity such as: inappropriate or inaccurate specification of production relationships; inadequate identification of alternatives, measurement or valuation of costs or benefits; lack of discounting of future costs and benefits; and failure to examine sensitivities. Although one should not underestimate the difficulty of producing a technically high-quality study, in principle such problems can be resolved; clearly the practice of analysis can and should improve over time. Also, in time, both analysts and policymakers may better understand the inherent limitations of CEA/CBA so as to make use of such analyses in a more realistic perspective. Thus, the usefulness of CEA/CBA seems likely to increase in the future. The 10 principles of analysis presented below are suggested as one method of minimizing not only weaknesses of immaturity, but also weaknesses that are inherent to the technique.

TEN PRINCIPLES OF CEA/CBA METHODOLOGY

There is widespread agreement that 10 basic principles of CEA/CBA methodology apply regardless of the technology being assessed or the circumstances under which a societally oriented analysis takes place. These 10 principles are discussed below. (See table 2.)
Table 2.—Ten General Principles of Analysis
(for CEA/CBA methodology)

1. Define problem.
2. State objectives.
3. Identify alternatives.
5. Analyze costs.
6. Differentiate perspective of analysis.
7. Perform discounting.
8. Analyze uncertainties.
9. Address ethical issues.
10. Interpret results.

SOURCE Office of Technology Assessment

1. Define Problem

The problem should be clearly and explicitly defined and the relationship to health outcome or health status should be stated. The problem, for example, could be expressed in terms such as “excess infection rate” or “excess deaths.” The broader the definition of the problem, the more relevant alternatives there are to examine: “Excess deaths,” for example, could lead to comparing any preventive or therapeutic program which decreases mortality; excess deaths due to cancer, however, would limit the scope of study considerably; and excess deaths due to cervical cancer would limit it further. Nevertheless, whatever the scope, as long as the focus is on a health problem, the study can focus on alternative means to solve the problem or, conversely, to increase health status. Some studies, however, must necessarily focus on the efficient use of a technology. This is particularly true of diagnostic technologies, where the ultimate health problem may be far removed from the use of the technology.

2. State Objectives

The objectives of the technology being assessed should be explicitly stated, and the analysis should address the degree to which the objectives are (expected to be) met. In general, the objectives will be governed by the way in which the problem is defined: The broader the problem definition, the broader the objectives. Ordinarily, it is most relevant for the objectives to be in terms of lowering morbidity, disability, or mortality or, alternatively, increasing well-being. When the objectives are stated in terms of decreasing costs, the relationship between costs and health benefits is often lost, sometimes resulting in untenable assumptions of equal efficacy across treatment modalities. Often, objectives are stated in terms of achieving a certain level of benefit for the least cost, or, conversely, achieving the most benefit per dollar cost.

3. Identify Alternatives

Alternative means (technologies) to accomplish the objectives should be identified and subjected to analysis. The number of alternatives and the relevancy of the analysis will increase as the scope of the identified problem is increased. Whereas there are numerous means to lower death rates, for example, there are relatively fewer ways to lower deaths due to a specific disease, and even fewer ways to do this by employing a particular technology. One of the most difficult questions to answer in analyzing the cost effectiveness of a given intervention (such as Pap screening) is “cost effective compared to what?”

4. Analyze Benefits/Effectiveness

All foreseeable benefits/effectiveness should be identified, and when possible should be measured. The relevant benefits/effectiveness of health care technology in the health field often follow directly from the problem under consideration, the objectives specified, and the framework in which the problem is approached. Not all benefits/effectiveness are positive—some may be negative (e.g., deaths due to surgery) and some may be indeterminate (e.g., incurable disease may be discovered). Each of the following categories should be considered: 1) personal benefits/effectiveness, such as alleviated pain, reduced risk of sickness or death, enhanced quality of life, lowered anxiety, 2) health resource benefits/effectiveness such as increases and decreases in health care expenditures, 3)
other economic benefits/effectiveness such as increased productivity, and 4) social benefits/effectiveness such as the equitable distribution of medical care. When possible, and if agreement can be reached, it is helpful to value benefits in common terms in order to make comparisons across alternative programs easier.

5. Analyze Costs

All expected costs should be identified, and when possible should be measured in dollars. In general, the concept of “opportunity cost” is the most correct way to consider the costs of a program. That is, the costs are equal to the value of the opportunities which are forgone because of the investment in the program.

6. Differentiate Perspective of Analysis

When private benefits and costs differ substantially from social benefits and costs, and if a private perspective is appropriate for the analysis, the differences should be identified, Although CEA/CBA is generally considered a tool of social policy, it is helpful and important to recognize that private incentives differ from public incentives and since health care delivery is often funded, always demanded, and usually delivered by the private sector, its (the private sector’s) perspective may be very important to the relevancy of the analysis. For instance, the social benefits of elective procedures such as elective hysterectomy, cancer screening, and many psychotherapy programs are apt to differ markedly from the private benefits. Typically, a CEA will identify the “social” benefits in terms of cost reduction, whereas the primary private objective (i.e., expected benefits) of the patient may be decreased anxiety.

7. Perform Discounting

All future costs and benefits should be discounted to their present value in order for them to be compared with one another. Discounting can be thought of as a reverse interest rate. It is used to take into account phenomena such as the observation that, all things being equal, people prefer benefits (including health benefits) today rather than at a future time. Although there is no firm agreement as to the precise discount rate to use, if future benefits of alternative programs are roughly proportionate to one another, the rate which is chosen makes little difference to the outcome of the analysis.

8. Analyze Uncertainties

Key variables should be analyzed as to the importance of their uncertainty to the results of the analysis. That is, a “sensitivity analysis” should be performed. In its simplest form sensitivity analysis is nothing more nor less than the application of common sense when one is not sure of a fact: It is the examination of the uncertain event under different assumptions. Sensitivity analysis can indicate both when more information is needed and when insufficient information is irrelevant.

9. Address Ethical Issues

Ethical issues should be identified, discussed, and placed in appropriate perspective relative to the rest of the analysis and the objectives of the technology. Many health care programs have as their primary objective the equitable distribution of services; other programs include it as one of many objectives; still other programs affect the distribution of society’s goods and services without an explicit intention to do so. A CEA/CBA should identify all these effects. When possible, it should also measure them. Although such effects cannot ordinarily be valued, however, they are often germane, and sometimes essential, to the measure of worth of a health program.

10. Interpret Results

The results of the analysis should be discussed in terms of validity, sensitivity to changes in assumptions, and implications for policymaking or decisionmaking. This is important both because the intended audience is often a public official or a health care professional, neither of whom may be technically oriented, and because study findings are often reported in capsule form such as a news brief, and are often intro-
OTHER FINDINGS

In addition to conforming to the aforementioned 10 principles, all quantitative analyses should specify data sources, be written as clearly and as nontechnically as possible, and be subjected to peer and other types of review, including public scrutiny when appropriate, especially regarding assumptions upon which the outcome of the analyses may rest. In general, the more technical the analysis, the more important that the review be formalized and conducted by individuals who can challenge the methodology that is employed. Reviews of those CEA/CBAs that are not too technical, however, may facilitate public scrutiny regarding the validity and, especially, the appropriateness of key assumptions. Such scrutiny may be useful because the application of CEA/CBA in the field of health policy is only part of a larger political process.

Since this report is primarily designed to examine the policy implications of using CEA/CBA for health care resource allocation decisions, the methodological process which is envisioned is substantially different from what would be discussed if this report were being written for the academic research community.

It is necessary to make this distinction because CEA/CBA can be a very complex undertaking analytically and often requires a massive data gathering effort. For instance, disease progression transition rates must often be assigned and mathematical models must capture the dynamics of the process; the effects of medical intervention may need to be estimated by professional opinion or empirically evaluated through epidemiological observation or by formal clinical trials; joint production costs may need to be estimated using sophisticated dynamic programming techniques; and so forth. All this is expensive, time consuming, and apt to require very specialized computer support, analytical skills, and clinical judgment. On the other hand, the real world dictates that health resource allocation decisions must often be made without the benefit of such resources—that is, with little time, money, and technical expertise. These suboptimal conditions, however, do not relieve decisionmakers from the responsibility of weighing the consequences of decisions.

Since CEA/CBA is being spoken of or advocated as a mechanism to assist policy makers in making rational choices between competing objectives, OTA was asked to assess the technique for that purpose. The findings are that, as formally applied, the methodology could often be too complex, expensive, and time consuming if used as a routine method for decisions by public policy makers. In fact, the cost-effectiveness case studies conducted as part of this assessment serve to highlight the immaturity of the technique itself. Initial drafts of more than half of the studies, all of which were performed by respected health care researchers, were considered by reviewers to be inadequate with respect to the relevancy/usefulness of the results, the validity of the methodology, the tenuousness (or error) in the key assumptions, and/or the validity of the data used. Clearly, the field is not yet fully defined.

Nevertheless, the logic behind using CEA/CBA, even at an operational or policymaking level, appears sufficient to suggest that the 10 principles previously enumerated can and should be followed under most circumstances.

In no way, however, does this finding suggest that a complete analysis is either easy or unnecessary. There is clearly a need for ongoing and sophisticated studies of the cost effectiveness of specific technologies as well as a need for advancing the state of the art itself. For instance, much good research has been done in developing and testing a composite index which de-
scribes the health status of a population at any
given point in time (e.g., 707,711,731,732,733). That type of work should continue and perhaps
should receive more emphasis. Nevertheless for-
mal CEA/CBAs, however valid and effective
potentially, can be inappropriately used by de-
cisionmakers who lack the necessary resources,
skills, or understanding of the inherent limita-
tions. Defining a more practical, limited ap-
proach to the methodology seems clearly approp-
riate and does not diminish the worth of, or
need for, more sophisticated approaches under
different circumstances.

**NONAGGREGATED ANALYSIS–AN ARRAYING TECHNIQUE**

Since many of the methodological weaknesses
of CEA/CBA may be hidden, aggravated, or in
fact caused by the practice of deriving a cost-
benefit or cost-effectiveness ratio—that is, a
numerical bottom line—the *possibility of not
aggregating the often complex sets of calcula-
tions should be investigated and considered.*
Rather than aggregating, analysts might ex-
licitly list or **ARRAY** all the elements which are
included in, or would be affected by, decisions.
When costs and effectiveness could be quanti-
fied, that would be done; when they could be
combined, that would also be done. **Whenever
one or more important nonquantifiable varia-
bles would otherwise either be left out or be rele-
gated to a footnote, however, no effort to arrive
at a single combined benefit value would be
made.** A nonaggregated or array method of
analysis would give decisionmakers a greater
number of elements to consider, but it would
also make intangible or nonquantifiable factors
more explicit, and thus might also help force
consideration of those factors by decisionmak-
ers commensurate with the factors’ significance.
The arraying method can either be highly quan-
titative and analytical, using multiobjective pro-
graming techniques, or when that is not desir-
able or possible, it can be presented more quali-
tatively.
Appendixes

Appendix A: Analysis of the Growth and Composition of the Health Care CEA/CBA Literature
Appendix B: Bibliography on CEA/CBA
Appendix C: Abstracts of Selected Entries From the Bibliography on CEA/CBA
Appendix D: Description of Other Volumes of the Assessment
Appendix E: Health Program Advisory Committee; Authors of Case Studies

NOTE: The original versions of the analysis of the growth and composition of the CEA/CBA literature, the bibliography, and many of the abstracts were prepared for OTA by Kenneth Warner and Rebecca Hutton.
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Appendix A.—Analysis of the Growth and Composition of the Health Care CEA/CBA Literature

As measured by contributions to the professional literature, interest in health care applications of CEA and CBA has grown dramatically over the past decade. This appendix analyzes the extent and nature of the growth in this literature and examines its substantive content. An assessment of the quality of contributions to the literature was presented in chapter 3.

The method of the analysis of the growth and composition of the health care CEA/CBA literature that appears in this appendix is described in the first section below. The second section offers an empirical characterization of the magnitude and nature of the literature, examining the diffusion over time of health care CEA/CBA interest in several dimensions: numbers of publications; the mix of medical and nonmedical publication vehicles; relative preferences for CEA and CBA; medical functions emphasized; physical nature of subjects of study; and the decision orientation of analyses. Specific substantive topics and areas of interest which have dominated the attention of authors were analyzed in chapter 1. The material covered by that review accounts for roughly half of all the entries in the bibliography in appendix B. That section of chapter 1, therefore, should be regarded as part of this analysis.

Method

The empirical analysis in this appendix derives from counts and classifications of over 500 of the references in the bibliography of CEA and CBA in health care (app. B). With a few exceptions, the bibliography consists of references from the years 1966 through 1978, including CBAs and CEAs concerning personal health services, reviews and comments on such literature, and discussions of CEA/CBA methodology directed specifically to health care professionals. Appendix B includes a description of the bibliography’s contents, rules for inclusion or exclusion of references, and the literature search process.

Each reference from the years 1966 through 1978 was classified according to the following dimensions:

1. year (1966 -78);
2. type of analysis (CEA, CBA, general or unknown);
3. publication vehicle (medical journal; journal intended primarily for nonphysician health professionals, administrators, or health services researchers; nonhealth);
4. medical function of the program or technology (prevention; diagnosis; treatment, divided into cure, rehabilitation, maintenance, or palliation; administration; some or all of the above or unknown);
5. physical nature of the program or technology (technique, drug, procedure, equipment, personnel, system, some or all of the above or unknown);
6. decision orientation (i.e., whose decision? individual, organization, society, unknown ); and
7. subject matter (a specific program or technology, review article, methodology, combinations of these).

Classification involved numerous arbitrary judgments. Many of the assignments depended on the content of abstracts or even the wording of titles. Where available information suggested that each of two (and very occasionally three) categories was appropriate, half (or a third) credit was assigned to each. For example, in the “medical function” dimension, certain screening programs were recorded as half prevention and half diagnosis. (A comprehensive blood pressure control program was counted as one-third for each of prevention, diagnosis, and treatment.) For “type of analysis,” a few studies presented both cost-benefit and cost-effectiveness estimates. Accordingly, these were scored as one-half CBA and one-half CEA. The “unknown” or “other” categories were used liberally when it was difficult or impossible to categorize references accurately.

Although the possibility remains that many of the assigning were not optimal, OTA is unaware of any significant sources of bias. Thus, at a minimum the quantitative analysis should provide an accurate qualitative characterization of the size, nature, and contents of the literature.

Growth and Character of the Literature

Diffusion

The magnitude and rate of growth of the health care CEA/CBA literature are indicated in table A-1 and figure A-1. Table A-1 records the annual numbers of CEAs, CBAs, and related publications for the years 1966 through 1978. The annual sum of identifiable CEAs and CBAs (column 3) is plotted in figure A-1, as is the total of all CEA/CBA-relevant references (column 5).

As the data vividly demonstrate, widespread interest in health care CEA/CBA is a phenomenon of the 1970’s. Prior to 1970, the annual number of health care CEAs/CBAs and related publications never exceeded 16; after 1970, the number was never less than
rate of growth of the health care CEA/CBA literature vastly exceeds that of the medical literature in general.

The usual “mechanics” of a diffusion process suggest continued growth in the number of publications, but this general tendency should be reinforced in the early 1980’s by several influences in the health care environment: Establishment of the National Center for Health Care Technology, with its authority to assess the safety, efficacy, and cost implications of medical technologies should foster analytical activity; publicity associated with other governmental efforts should increase awareness and interest; a similar effect can be anticipated to follow activities within the medical profession, such as the AMA’s Resident Physicians Section’s recent publication of its report on cost-effective care; growth in attention to health economics issues in medical school curricula should promote interest and understanding among young physicians; and most generally, but probably most importantly, continued concern about the high and growing costs of care should itself generate numerous attempts to assess the cost effectiveness of medical technologies (703).

Publication Vehicles

Table A-2 shows the distribution by year of the health care CEA/CBA literature by type of publication. The purpose is to examine what proportion of the literature has been intended primarily for a physician audience, as reflected in publication in medical journals, and how this proportion has changed over time.
Figure A-1.— Diffusion of Health Care CEA/CBAs by Year (1966-77)
Table A-2.—Numbers of Health Care CEA/CBAs by Type of Journal and Year (1966-78)

<table>
<thead>
<tr>
<th>Year</th>
<th>Medical journals*</th>
<th>NEJM*</th>
<th>Nonmedical journals</th>
<th>Other*</th>
<th>Total</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>(1)</td>
<td>(2)</td>
<td>(3)</td>
<td>(4)</td>
<td>(5)</td>
</tr>
<tr>
<td>1966</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>1967</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>1968</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>1969</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>1970</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>1971</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>1972</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>1973</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>1974</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>1975</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>1976</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>1977</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>1978</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Total</td>
<td>211.0</td>
<td>3.1</td>
<td>162.0</td>
<td>153</td>
<td>525.0</td>
</tr>
</tbody>
</table>

*Journals read primarily by Physicians Excludes nursing, dental, public health, hospital Journals, etc. Includes psychiatric journals
**New England Journal of Medicine
**All nonjournal includes = physician oncology health journals, economics journals, policy analysis journals, etc
Books, chapters in books, unpublished papers, etc
Fractional entries for 1974 and 1975 reflect the inclusion of one article that appeared in a journal with publication date December 1974/January 1975

Figure A-2 plots columns 1 and 3 of table A-2 to illustrate the diffusion paths of medical and nonmedical journal articles. Although the paths follow each other closely, the graph shows a shift from a rough parity prior to 1973 to a clear majority of medical journal articles after 1973. In other words, the rate of growth of the medical literature has exceeded that of the nonmedical journal literature, particularly in recent years. This shift is clearly suggestive of a growing economic consciousness in the medical profession.

Column 2 in table A-2 records the annual number of CEA/CBA articles in the New England Journal of Medicine (NEJM). Several of the best, most influential health care CEA/CBAs have been published in NEJM (see ch. 3), hence its isolation here. It is interesting to observe that prior to 1975, the number of CEA/CBA-relevant contributions in NEJM exceeded one only once (in 1968), NEJM has published several relevant articles each year since 1975. Some observers believe that medical interest in CEA/CBA received its biggest boost from the publication of the controversial July 31, 1975, issue of NEJM which was devoted to CEA/CBA studies and discussions of their methodology and usefulness. (See the methodology review article section of ch. 3.)

Mix of CEAs and CBAs

Columns 1 and 2 of table A-1 distinguish analyses identified as CBAs from those identified as CEAs. Prior to the most recent years, the annual number of CBAs generally exceeded the number of CEAs. Since 1975, the reverse has been true, supporting the statement in a recent review by Weinstein that CEA "has been gaining in acceptance relative to benefit-cost" (569). The reason is not obvious. Weinstein attributes the shift to "the conceptual limitations of the (human capital) approach and the empirical barriers to the willingness-to-pay approach. " Complementary or alternative explanations relate to the apparent relative conceptual simplicity of CEA: Analysts use CEA because it is easier for the economic layperson—e.g., the physician—to understand; also, the recent relative growth in the literature in medical journals appears to include relatively more contributions by physicians, who, as economic laypersons, may find CEA easier to perform than CBA. Economists' traditional preference (at least in nonhealth care areas) for CBA may reflect a general conceptual bent toward valuing and directly comparing the positive and negative consequences of activities. It also probably reflects the successful use of CBA in early applications in which benefits were reasonably amenable to monetary valuation (e.g., water resource management).
Figure A-2.–Diffusion of CEA/CBAs in Medical and Nonmedical Health Care Journals by Year (1966-77)

Key:
- CEAs and CBAs in medical journals per year (from Table 3, Appendix A)
- CEAs and CBAs in nonmedical health care journals per year (from Table 3, Appendix A)

Source: [Technology Assessment]
Medical Functions

Tables A-3 and A-4 present categorizations of literature contributions by the general medical function which is the substantive focus of each paper. Table A-3 includes three broad categories (prevention, diagnosis, and treatment, plus a fourth miscellaneous category). Table A-4 breaks down treatment functions by their purpose: cure, rehabilitation, maintenance, or palliation.

Among the three broad categories, prevention and diagnosis each account for more than a quarter of the studies over the entire period, while the various types of treatment total just under half. If one divides the years covered into the period preceding 1974 and the period from 1974 through 1978, however, there is a significant shift in the relative mix, away from prevention and toward diagnosis and treatment. During the most recent 5 years, the numbers of both diagnosis- and treatment-oriented papers have exceeded the pre-1974 totals by a factor of four or five. By con-

In his recent review, Weinstein [569] observed: ‘Diagnostic procedures, apart from screening tests, have received little attention. This OTA analysis attributes nearly a quarter of the codeable literature to diagnoses not necessarily at variance with this observation, since it includes many screening programs in the diagnosis category.'

Table A-3.—Numbers of Health Care CEA/CBAs by Medical Function and Year (1966-78)

<table>
<thead>
<tr>
<th>Year</th>
<th>Prevent (1)</th>
<th>Diagnosis (2)</th>
<th>Treatment (3)</th>
<th>Other (4)</th>
</tr>
</thead>
<tbody>
<tr>
<td>1966</td>
<td>0.0</td>
<td>0.0</td>
<td>0.0</td>
<td>5</td>
</tr>
<tr>
<td>1967</td>
<td>0.0</td>
<td>0.3</td>
<td>1.7</td>
<td>3</td>
</tr>
<tr>
<td>1968</td>
<td>2.5</td>
<td>3.0</td>
<td>3.5</td>
<td>6</td>
</tr>
<tr>
<td>1969</td>
<td>1.5</td>
<td>0.5</td>
<td>2.0</td>
<td>2</td>
</tr>
<tr>
<td>1970</td>
<td>3.0</td>
<td>2.0</td>
<td>3.0</td>
<td>8</td>
</tr>
<tr>
<td>1971</td>
<td>6.5</td>
<td>3.5</td>
<td>4.0</td>
<td>11</td>
</tr>
<tr>
<td>1972</td>
<td>7.0</td>
<td>2.0</td>
<td>4.0</td>
<td>14</td>
</tr>
<tr>
<td>1973</td>
<td>14.5</td>
<td>4.0</td>
<td>10.5</td>
<td>15</td>
</tr>
<tr>
<td>1974</td>
<td>2.5</td>
<td>5.0</td>
<td>14.0</td>
<td>22</td>
</tr>
<tr>
<td>1975</td>
<td>5.0</td>
<td>100</td>
<td>14.5</td>
<td>22</td>
</tr>
<tr>
<td>1976</td>
<td>15.0</td>
<td>160</td>
<td>28.0</td>
<td>33</td>
</tr>
<tr>
<td>1977</td>
<td>12.5</td>
<td>17.0</td>
<td>37.5</td>
<td>35</td>
</tr>
<tr>
<td>1978</td>
<td>18.0</td>
<td>255</td>
<td>18.5</td>
<td>31</td>
</tr>
<tr>
<td>Total</td>
<td>88.0</td>
<td>88.8</td>
<td>141.2</td>
<td>207</td>
</tr>
</tbody>
</table>

Includes mixes of all three functions (prevent, diagnosis, and treatment), administration, general, and unknown.
SOURCE: Office of Technology Assessment

Table A-4.—Numbers of Health Care CEA/CBAs by Treatment Function and Year (1966-78)

<table>
<thead>
<tr>
<th>Year</th>
<th>Cure (1)</th>
<th>Rehabilitation (2)</th>
<th>Maintenance (3)</th>
<th>Palliation (4)</th>
<th>Total (5)</th>
</tr>
</thead>
<tbody>
<tr>
<td>1966</td>
<td>0.0</td>
<td>0.0</td>
<td>0.0</td>
<td>0</td>
<td>0.0</td>
</tr>
<tr>
<td>1967</td>
<td>1.3</td>
<td>0.3</td>
<td>0.0</td>
<td>0</td>
<td>1.7</td>
</tr>
<tr>
<td>1968</td>
<td>1.0</td>
<td>1.0</td>
<td>1.5</td>
<td>0</td>
<td>3.5</td>
</tr>
<tr>
<td>1969</td>
<td>1.5</td>
<td>0.0</td>
<td>0.5</td>
<td>0</td>
<td>2.0</td>
</tr>
<tr>
<td>1970</td>
<td>0.5</td>
<td>1.0</td>
<td>1.5</td>
<td>0</td>
<td>3.0</td>
</tr>
<tr>
<td>1971</td>
<td>2.0</td>
<td>1.0</td>
<td>1.6</td>
<td>0</td>
<td>4.0</td>
</tr>
<tr>
<td>1972</td>
<td>2.5</td>
<td>0.0</td>
<td>0.0</td>
<td>0</td>
<td>4.0</td>
</tr>
<tr>
<td>1973</td>
<td>4.5</td>
<td>3.5</td>
<td>2.5</td>
<td>0</td>
<td>10.5</td>
</tr>
<tr>
<td>1974</td>
<td>5.0</td>
<td>7.5</td>
<td>1.5</td>
<td>0</td>
<td>14.0</td>
</tr>
<tr>
<td>1975</td>
<td>6.5</td>
<td>3.0</td>
<td>5.0</td>
<td>0</td>
<td>14.5</td>
</tr>
<tr>
<td>1976</td>
<td>10.5</td>
<td>6.5</td>
<td>11.0</td>
<td>0</td>
<td>28.0</td>
</tr>
<tr>
<td>1977</td>
<td>25.0</td>
<td>4.5</td>
<td>8.0</td>
<td>0</td>
<td>37.5</td>
</tr>
<tr>
<td>1978</td>
<td>8.5</td>
<td>4.0</td>
<td>6.0</td>
<td>0</td>
<td>18.5</td>
</tr>
<tr>
<td>Total</td>
<td>68.8</td>
<td>33.8</td>
<td>38.5</td>
<td>0</td>
<td>141.2</td>
</tr>
</tbody>
</table>

SOURCE: Office of Technology Assessment
trast, the number of prevention-oriented contributions is only 50 percent greater than that of the earlier period (see table A-3).

This shift seems consistent with the relative growth in the medical journal share of the literature, assuming that physicians are relatively more interested in diagnosis and treatment, as opposed to prevention, than are nonphysician health professionals (including both providers and health services researchers). Also, consistent with the principal early nonhealth care applications of CEA/CBA, early health care CEA/CBAs concentrated relatively more on health care “public goods,” including especially communicable disease control, than on individual patient care, a growing concern today. Several excellent communicable disease prevention studies are found in the recent medical literature, but this is one of the few substantive areas in which the number of pre-1974 papers actually exceeded the number of 1974 through 1978. (See ch. 1.)

The shift away from prevention may not be permanent. The widespread perception that “technology” is a major villain in medical cost inflation, combined with the general medical orientation toward diagnosis and treatment, has contributed to growing interest in diagnostic and treatment technology, both in the CEA/CBA literature and in individual physician decisionmaking concerning the use of such technology. These interests will likely be sustained in the near future. However, the Federal Government’s recent emphasis on prevention (743), increasing public acceptance of the ideas of disease prevention and health promotion, and the conscious linking of prevention to cost containment (e.g., 564) may promote renewed interest in prevention-oriented CEA/CBA.

Table A-4 shows that half of all treatment-oriented papers are concerned with curative treatments, and the remaining half are divided roughly equally between medical rehabilitation and maintenance. Reflecting the inherent subjectivity and difficulty of quantifying “pain relief,” “comfort,” etc., the literature included not a single contribution that could be identified as dealing with palliation. The relative mix of treatment functions has not changed significantly in recent years. Of note is the unusually large number of cure-oriented papers in 1977.

Physical Nature of Subjects of Study

Is there a growing emphasis in health care CEA/CBA on individual technologies? OTA’s examination of the literature permits only an impressionistic answer. In attempting to categorize subjects by their physical nature, OTA was incapable of definitively assessing the vast majority as either technique, drug, procedure, equipment, personnel, or system. Most seemed to represent a mix of two or more categories; consequently, they were included in the “miscellaneous” category. Even some which could be categorized were categorized with a feeling of discomfort. A study of the cost effectiveness of CT scanning appears on the surface to belong under “equipment” (where it was categorized), yet that same study emphasizes the important role of the new technicians needed to operate the scanner.

A principal impression is that the literature covers a broad spectrum of types of programs and technologies, with procedures being the best represented category. In recent years, there appears to have been distinct growth in the attention devoted to equipment-embodied technologies, with CT scanning leading the way with some 18 references since 1975. (See ch. 1.)

Decision Orientation

The original intent of CEA/CBA was to assist in social decisionmaking, i.e., to identify and value program costs and benefits from a societal perspective. Businesses and individuals have long employed the ideas behind CEA/CBA to grapple with decision problems, but the CEA/CBA label seems to be applied with increasing frequency to analyses whose decision-assisting perspective is narrower than that of “society.”

Table A-5 permits an exploration of the distribution of “decision orientation” in the health care CEA/CBA literature and of changes in the distribution over time. The table suggests that the social perspective has dominated the literature over the entire period studied, accounting for roughly 70 percent of all publications in both the early and most recent years; if anything, its dominance has grown slightly over time. Nevertheless, it is also true that articles oriented toward individual (e.g., practitioner) decisionmaking have increased most rapidly in recent years. Comparing the pre-1974 period with the years 1974 through 1978, one observes a near doubling of the share of papers oriented toward the individual perspective. This growth has come at the expense of papers with an organizational orientation. While the latter two categories together account for fewer than 30 percent of the literature contributions, the shift may be significant.
Summary

Table A-6 summarizes highlights of this empirical description of the literature, breaking the period into the “early” years (those prior to 1974) and “recent” years (1974-78). As the table indicates, recent years have witnessed dramatic growth in the number of contributions to the health care CEA/CBA literature (item 1). More of this growth has occurred in medical than in nonmedical journals (item 2), and CEA is gaining favor relative to CBA (item 3). The early prominence of studies with a substantive prevention theme has diminished, while studies related to diagnosis and treatment have become more popular (item 4). Health care CEA/CBAs retain as their principal orientation a societal perspective on problems, though studies with an individual practitioner orientation are becoming increasingly common (item 5).

Table A-5.—Numbers of Health Care CEA/CBAs by Decision Orientation and Year (1966-78)

<table>
<thead>
<tr>
<th>Year</th>
<th>Individual (1)</th>
<th>Organization (2)</th>
<th>Society (3)</th>
<th>Unknown (4)</th>
</tr>
</thead>
<tbody>
<tr>
<td>1966</td>
<td>0</td>
<td>0</td>
<td>3</td>
<td>2</td>
</tr>
<tr>
<td>1967</td>
<td>0</td>
<td>0</td>
<td>5</td>
<td>0</td>
</tr>
<tr>
<td>1968</td>
<td>0</td>
<td>4</td>
<td>9</td>
<td>2</td>
</tr>
<tr>
<td>1969</td>
<td>0</td>
<td>2</td>
<td>2</td>
<td>2</td>
</tr>
<tr>
<td>1970</td>
<td>1</td>
<td>3</td>
<td>7</td>
<td>5</td>
</tr>
<tr>
<td>1971</td>
<td>4</td>
<td>2</td>
<td>14</td>
<td>5</td>
</tr>
<tr>
<td>1972</td>
<td>2</td>
<td>5</td>
<td>12</td>
<td>8</td>
</tr>
<tr>
<td>1973</td>
<td>2</td>
<td>7</td>
<td>27</td>
<td>8</td>
</tr>
<tr>
<td>1974a</td>
<td>5.5</td>
<td>2</td>
<td>21</td>
<td>15</td>
</tr>
<tr>
<td>1975a</td>
<td>2.5</td>
<td>1</td>
<td>24</td>
<td>14</td>
</tr>
<tr>
<td>1976</td>
<td>12</td>
<td>4</td>
<td>49</td>
<td>27</td>
</tr>
<tr>
<td>1977</td>
<td>13</td>
<td>4</td>
<td>50</td>
<td>35</td>
</tr>
<tr>
<td>1978</td>
<td>8</td>
<td>7</td>
<td>50</td>
<td>28</td>
</tr>
<tr>
<td>Total</td>
<td>50</td>
<td>51</td>
<td>273</td>
<td>151</td>
</tr>
</tbody>
</table>

\*Fractions entries for 1974 and 1975 reflect the inclusion of one article that appeared in a journal with publication date December 1974/January 1975.

SOURCE Office of Technology Assessment

Table A-6.—Overview of Trends in Health Care CEA/CBA Literature (1966-73 and 1974-78)\*a

<table>
<thead>
<tr>
<th></th>
<th>1966-73</th>
<th>1974-78</th>
</tr>
</thead>
<tbody>
<tr>
<td>Average annual number of publications.</td>
<td>18</td>
<td>76</td>
</tr>
<tr>
<td>Publications in medical journals as percent of total journal publications.</td>
<td>39%</td>
<td>62%</td>
</tr>
<tr>
<td>CEAs as percent of CEAs + CBAs</td>
<td>41</td>
<td>53</td>
</tr>
<tr>
<td>Percent of articles on:</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Prevention</td>
<td>44</td>
<td>22</td>
</tr>
<tr>
<td>Diagnosis</td>
<td>19</td>
<td>31</td>
</tr>
<tr>
<td>Treatment</td>
<td>36</td>
<td>47</td>
</tr>
<tr>
<td>Percent of articles with orientation:</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Individual</td>
<td>8</td>
<td>16</td>
</tr>
<tr>
<td>Organization</td>
<td>21</td>
<td>11</td>
</tr>
<tr>
<td>Society</td>
<td>71</td>
<td>74</td>
</tr>
</tbody>
</table>

\*All differences significant at p < 0.05

SOURCE Office of Technology Assessment

December 1974/January 1975
Appendix B.— Bibliography on CEA/CBA

Contents and Development of the Bibliography

The bibliography includes approximately 600 references for CEAs and CBAs on personal health services topics, reviews and comments on such literature, and discussions of CEA/CBA methodology directed specifically to health care professionals. It is intended to be as comprehensive as possible for the years 1966 through 1978. Given the lag between publication and indexing and citation, several articles and books published in 1978 may have been missed. Included in the bibliography are a few references from before 1966, several from 1979, and a few from 1980, though no systematic attempt was made to identify references outside of the 1966-78 period. Excluded from the bibliography are scores of CEAs/CBAs on health-relevant nonmedical subjects (e.g., traffic safety and control of environmental pollution), as well as dozens of books and articles on CEA/CBA methodology that are addressed to a general nonhealth technical audience (e.g., 737).

The line determining inclusion in or exclusion from the bibliography was not invariably a fine one. In general for methodology articles, borderline references were excluded. Thus, certain prominent articles which have had substantial impact on health care CEA/CBA are not found in the bibliography (though some are listed in “Other References”) because they were not necessarily directed at a health care audience. The seminal work of Rice on measuring the cost of illness is a good case in point (721,746), as is the related work of Acton and others on measuring the value of life (4,470, 730,761). These studies reside at the heart of a long-lived CEA/CBA intellectual debate, and each has formed the basis of numerous attempts to value the health benefits of programs; but the issues and techniques transcend categorization as health care methodologies. They are equally relevant to numerous human welfare programs outside of the personal health services arena. Similarly, several excellent studies of the social costs of specific illnesses are excluded because they are not CEA/CBAs of efforts to reduce these costs.

Whereas the rule for tangentially related methodology references was exclusion, OTA adopted a policy of inclusiveness when classifying references as CEA or CBA. As described immediately below, the literature search relied heavily on published and computer-based indexes. Many of the studies which purport to be CEAs or CBAs are not CEAs/CBAs under the definitions of chapter 1. At the extreme, for example, at least one article whose title advertises it as presenting a cost-benefit ratio does not include any such comparison of costs and benefits (47). Rather than weed out such references, OTA included all studies which are known to be CEA/CBAs or which are presented as such in their titles. Chapter 3 identifies most of the studies commonly held in high regard.

References included in the bibliography were obtained from four sources: 1) computer-assisted literature searches, 2) published professional literature indexes, 3) reference lists of individual articles, papers, and books, and 4) communication with leading health services researchers, including the members of the Advisory Panel to this OTA assessment.

Two computer-assisted literature searches provided numerous references. MEDLARS covered relevant citations from Index Medicus. For the years 1966 through 1975, this search covered the subject heading “Cost and Cost Analysis” (which, until 1976 included CEA and CBA). From 1976 to the present, the search was limited to the Index Medicus subject heading “Cost Benefit Analysis”, a heading introduced in 1976 which includes both CEA and CBA. The second computer-assisted search was conducted by the National Health Planning Information Center, using the key words “cost-benefit analysis” and “cost-effectiveness analysis.”

For the months postdating the MEDLARS search, Index Medicus was consulted directly. Published indexes also supplied many relevant economic studies not included in Index Medicus. Beginning with the 1966 editions, two indexes of economic literature were selected: the Index of Economic Articles and the Journal of Economic Literature (prior to 1969 entitled the Journal of Economic Abstracts).

NOTE: Articles and other materials that did not fit the criteria for inclusion in this bibliography but that were used in the writing of this background paper have been collected as “Other References” at the end of this report. References in the text to this bibliography or to “Other References” may be distinguished by the number of the reference. Those in the bibliography are numbered from 1 through 601; those in “Other References” are numbered from 701 upward.


204. Galliher, H., “Cost-Effective Planned Lifetime Schedule of Pap Smears: Estimated Maximal Potentials” (Ann Arbor, Mich.: University of
Appendix B—Bibliography on CEA/CBA


248. Hamilton, J., "A Method for Estimating the Cost, Growth Rate, and Efficiency of Radioiso-


310. ______ “The Economics of Screening for Colorectal Cancer” (Stony Brook, N. Y., American Health Foundation (unpublished)).


368. Merck, Sharp, and Dohme, Vaccine Cost-Benefit Analysis Program (West Point, Pa.: MSD, n.d.).


479. ______ and Luce, B., A Cost-Effective Approach to Cervical Cancer Detection, DHEW publication No. 79-32371 (Hyattsville, Md.: National Center for Health Services Research, January 1979).


492. Showstack, J., and Schroeder, S., “The Costs and Effectiveness of Upper Gastrointestinal Endoscopy,” in The Implications of Cost-Effec-


Appendix C.—Abstracts of Selected Entries
From the Bibliography on CEA/CBA


This CEA of computed axial tomograph (CAT) body scans employs an efficacy scale which ranges from 1 point (given when the scan is deemed to have saved a patient’s life) to 18 points (given when the scan is held to have led to a patient’s death). In the course of the analysis, the sensitivity, specificity, and accuracy of CAT body scans are evaluated. The authors note that less expensive tests, most notably ultrasound, are bypassed or performed simultaneously with CAT scans. Analysis indicated that ultrasound and CAT scans are of about equal clinical value in any given situation, but ultrasound costs one-fourth as much as CAT scanning. The authors observe that clinicians, when employing CAT scanning, often seem to have no clear expectations that it can affect patient management. They also note that for most conditions about which CAT body scans are informative, insufficient information is not the major factor limiting the success of therapy. Though this study, limited to hospitalized patients, would have missed any decreased admissions for diagnostic tests which may have resulted from the use of CAT body scanning, its authors believe that few savings can be expected from replacing other diagnostic procedures with CAT scans. They recommend that CAT body scans be ordered only if 1) more information would truly affect patient management, 2) more cost-effective diagnostic tests have failed, and 3) the likelihood of disease is high.

The authors caution that their study was done as CAT technology was rapidly evolving. This evolution has obvious implications, including the likelihood that current use patterns (frequency and motivation) differ from what they will become if and when body scanning becomes standard practice. As such, the study fails to distinguish between cost effectiveness today and in a steady-state situation in the future. In addition, the study does not identify potential cost efficacy (i.e., cost effectiveness under optimal conditions). Despite these drawbacks, this study stands out as an example of how titles can be misleading. The general principles of CBA are presented, with a “good” program described as one in which the net discounted benefits exceed zero. The author says that CBA was first applied to health care in response to rapidly rising medical care expenditures. When conflict between individual and societal interests is discerned, the techniques of CBA must be applied with sensitivity to the individual and public interests involved. Limitations of CBA in health care include difficulties in accurately accounting for the numerous complex costs and benefits encountered, in identifying and valuing long-range effects, and in determining a discount rate when costs and benefits are deferred many years.

CBA is described as applicable only where effects are nearly equivalent, so that the analysis becomes, in effect, a cost comparison. (However, one can also look at different effects resulting from equivalent costs. ) Three examples of CBA as applied in health care are presented: 1) consideration of cholecystectomy for silent gallstones, 2) renal transplantation or chronic hemodialysis for end-stage renal dialysis, and 3) intensive care unit support for different illnesses.

The author states that the accomplishments of CBA and related techniques in health are largely those of more comprehensive understanding of the advantages or disadvantages of a particular therapy or policy. In itself, CBA is seldom definitive, but in conjunction with political and professional judgments, it can improve decision making.


The bulk of this article is devoted to a comparison of the costs involved in five different options for implementing CAT scanning in a region of England. The net costs of CAT scanning are calculated as gross costs (e.g., purchasing, installation, staffing, etc.) minus savings from the decreased use of conventional neuroradiology and reduced bed days, presumed to result from the introduction of CAT. The article also includes a discussion of possible treatment improvements, unquantified and not included in the cost of calculations, that may result from the use of CAT. There is little discussion of the cost effectiveness of CAT scanning v. conventional neuroradiology, though the analysis of the five CAT implementation options seems based on the premise that CAT is more cost effective in certain circumstances. The authors acknowledge the lack of precision and certainty involved in the savings calculations, but contend that some savings do result from the introduction of CAT and must be assessed in any analysis.


This paper is an example of how titles can be misleading. Despite the title, there is not a single cost-benefit ratio in the entire article. The author compares the posttransplant course of patients who previously had had their kidneys removed to that of patients who had had no pretransplant surgery. The latter group experienced fewer rejections and better survival.

NOTE: Three types of entries are abstracted: 1) many of the better known studies or methodology articles, 2) several examples of articles of varying technical sophistication (that is, a sample of the general literature) and 3) most of the case studies prepared or supported by OTA as part of the overall assessment (see app. D) Inclusion here does not imply that any particular study is one of the "best."

This study examines the feasibility and potential usefulness of undertaking CEA/CBA of orthopedic joint prostheses. Two specific issues are addressed: 1) whether it is feasible to evaluate carefully and completely the orthopedic joint implant technology within a CEA/CBA framework; and 2) how could such an evaluation be useful in formulating public policy.

The authors present a state-of-the-art study of CEA/CBA as it pertains to this technology. They do not try to assess the technology. The study includes a description of the technology (joint implants) based on a review of the literature, communications with selected medical specialists, and conversations with representatives of the orthopedic prostheses industry. The authors briefly discuss alternative forms of treatment for arthritis and point out an important difference between the alternatives (e.g., drugs) and joint implants: Most alternatives are only short-run measures, whereas joint implantation is a long-term measure.

Few data are available regarding the efficacy of joint implants. Data regarding the efficacy of hip replacements are better than the data for other joint implants or alternative measures. They may even be acceptable. Efficacy studies are in progress for some implants. The authors did not explore the possibility of producing the result (successful joint implantation) in the most efficient manner possible.

Potential benefits were put into two categories: direct and indirect. Potential direct benefits discussed include relief of pain, improved functional status of joint, measures included in the “Sickness Impact Profile” (e.g., social interactions, ambulation, sleep, leisure, and emotions), quality-adjusted life years (QALYs), and earnings. Potential indirect benefits include averted expenditures for the caring for, and treatment of, individuals handicapped with debilitated joints, e.g., those with severe arthritis. These potential benefits were only mentioned; none were quantified or measured.

Most costs mentioned were not distinguished from charges, and “avoidable” costs are not specifically identified. Some indirect costs, e.g., loss of productivity when patient is hospitalized, were identified. The author points out that both indirect and direct costs of complications associated with joint implants must be included as well as the costs of followup care and rehabilitation therapy.

The authors note that all projected benefits and costs of neonatal intensive care are and use data from small, restricted population samples drawn from NICUS that vary in size, shape, and capabilities. Potential problems involved in analyzing neonatal intensive care services designed to provide advanced care to severely ill newborns. Costs are distinguished from charges. The study addresses the average cost per day of caring for the critically ill newborn and reimbursement policies and procedures. Numerous problems involved in analyzing neonatal intensive care services are identified. First, the definitions are very tenuous. Neonatal services in many hospitals do not fit into the classifications used. Technological or personnel capabilities vary considerably in different hospitals, and regulatory and reimbursement policies create incentives for hospitals to classify neonatal units inappropriately. Providers, paying units, and regulators disagree on uniform definitions that should be applied to different levels of care.

The major focus of the study is on efficacy, effectiveness, and costs of neonatal intensive care. Outcomes are defined in terms of improved mortality and morbidity rates and mental and physical development of critically ill newborns. Costs are distinguished from charges. The study addresses the average cost per day of caring for the critically ill newborn and reimbursement policies and procedures. No discount rate is used. Except in a very rough estimate of high and low figures for use and the cost effectiveness of caring for different birthweight infants, sensitivity analysis is not applied. Equity issues are not addressed.

The authors examine the incidence and severity of prematurity in the United States. They evaluate the social and biological aspects of prematurity, trends in infant mortality, and the incidence of underweight infants in the last two decades, and the effect of neonatal intensive care units (NICUS) on mortality and morbidity of premature infants at various birthweights. They also examine the use of NICUS via admission rates, estimated average length of stay, estimated total patient days, the number of hospitals with NICUS, and the number of intensive care beds.

Next, the authors examine the costs of neonatal intensive care, providing a caveat that the data on use and cost are very rough approximations. The authors derive the cost and use data from small, restricted population samples drawn from NICUS that vary in size, shape, and capability. Data were examined from three geographic regions and five individual centers, along with numerous studies on neonatal care, Costs associated with varying degrees of prematurity and severity of illness were examined, as well. In general, costs are directly related to birthweight and prematurity—the lower the weight and/or the earlier the
birth, the higher the cost. The average cost per day in the hospital for critically ill newborns is $267 for an average stay of 13 days. The average charge per day was about $394. The study looks at the existing system of reimbursement for the cost of neonatal intensive care in five States and via five payers: commercial insurance, Blue Cross, medicaid, self-pay, and private insurance.

NICUS have been shown to reduce mortality rates, and all indications are that NICUS are cost effective. Nevertheless, more data is needed to determine their full impact. The authors review studies of the cost effectiveness or benefit of neonatal intensive care. They use a hybrid cost-effectiveness and cost-benefit analysis developed by Marcia Kramer to measure marginal costs of providing neonatal intensive care. They also compare methods of care in Great Britain and France with those in the United States.

The authors suggest that Federal policies need to be changed to reflect changes that have occurred in neonatal care. In particular, they suggest, guidelines that establish maximum numbers of beds per live births and minimum sizes of neonatal care units need to be revised. Also, medicaid and Social Security provisions for reimbursement of neonatal care costs need to be reexamined. The potential ethical implications of neonatal intensive care need more discussion.


This article, technically a CEA, compares the effects of three alternative methods for the treatment or prevention of myocardial infarction: 1) a coronary care unit, 2) a mobile coronary care unit, and 3) an intervention/prevention program aimed at reducing serum cholesterol levels. Effects are measured in terms of the total years of life added as a result of each alternative program. Costs are classified as direct and indirect. Costs and effects of each strategy are modeled on the basis of a cohort of 10-year-olds followed throughout their lifetimes. In addition, the manner of implementation is varied. Costs and effects are calculated for each alternative method assuming 1) the method is newly introduced alone, and 2) it is newly introduced with the other alternatives ongoing. "Cost-benefit" ratios are calculated as the dollar cost per added year of life for each alternative, introduced alone. The author illustrates changes in the ratios that result from varying the discount rate (i.e., performs sensitivity analysis), using rates of 0, 5, and 10 percent. She also discusses problems of selecting a discount rate for comparing alternative programs that incur costs and accrue benefits at widely separated times. The author finds the results of her analysis inconclusive. She notes that this and other modeling processes involve many simplifying assumptions and require that parameter values be estimated even when supporting data is scant.


In this article, the authors discuss the method of CEA in general, contrast it with CBA, and illustrate it with an example involving alternative programs of health care for the elderly (e.g., home care, day care, etc.). They also discuss the problems of measuring costs by market prices which may "obscure the real opportunity costs of resource consumption." With regard to effectiveness criteria, the authors note that many can be specified only in terms of ordinal numbers denoting rank, and they warn against the temptation "to add the nonadditive and to compare the incomparable." Costs in the analysis presented as an example are classified as primary, secondary, and tertiary, denoting program costs, other health-related service costs, and personal living expenditures, respectively. The authors explain and illustrate the tabular display approach to presenting data, in which effectiveness criteria are presented in columns and alternative programs are presented as row headings. It is unlikely, the authors conclude, that one alternative will emerge as preferred on the basis of all relevant criteria. In their example, day care is preferred on the basis of effectiveness criteria, while home care is preferred on the basis of cost criteria.


This report describes the methods used to analyze the cost effectiveness of alternative cancer screening policy options and the rationale for a recommended insurance benefits program. Five cancer sites—breast, colon, cervix, lung, and bladder—were selected for full analysis. The model used translates the problem of screening-program effectiveness, and many variables that contribute to it, into quantitative terms and logical relationships. Probability formulas relating to the important variables are derived. The model, designed to be programed on a computer, traces the expected fate of a patient under various program options. It will accept information about patient characteristics (age, relative risk, previous history, incidence rates, etc.) and will program options and present information on the costs and effectiveness of a specified program. Different discount rates can be entered into it.

The author notes that creating a cancer screening program that is both medically effective and low in cost requires that many age, sex, and risk categories be used to define the optimal services and screening frequencies for various groups of individuals. Ideally, a program might include several screening protocols, each tailored to different categories. This is not possible, however, for a prepaid benefit program that will be purchased by a large heterogeneous population. Thus, one objective is to design a benefit program in which services do not vary greatly for those covered. Marginal effectiveness, rather than absolute effectiveness, was considered the effectiveness criterion, and on this basis, there is little difference in the cost-effective program for high-risk as compared to average-risk groups. The benefit program designed includes the following provisions: 1) a standard screening program will be provided every 4 years for persons aged 25 to 45; 2) a standard screening program will be provided every year for those over age 45; 3) an impregnated guaiac slide will
be provided every year beginning at age 45; 4) a Pap smear will be provided to women every 4 years beginning at age 25; 5) a mammography will be provided to women covered by the high-option benefit every 2 years beginning at age 50; 6) a proctosigmoidoscopy will be provided every 5 years beginning at age 50.


This study focuses on the techniques that are available to screen for colon cancer—their development, evaluation, use, and cost effectiveness.

The author examines the three basic methods of techniques used in the detection of colon cancer: 1) the digital exam, 2) the sigmoidoscope, and 3) the test for occult blood in the stool. For each method, the author notes, there is either some degree of uncertainty regarding the sensitivity and specificity of the tests, or some degree of risk to the patient involved.

The study points out that there have been few, if any, clinical studies of the digital exam. Its effectiveness has been proven via the “time-honored” method of use and acceptable results at the patient-provider level. The effectiveness of sigmoidoscopes has been examined in a few clinical studies. The Hemoccult test has been through, and is going through, a number of large clinical trials to evaluate its efficacy. To date, the results are inconclusive.

The author discusses the problems that exist in trying to apply CEA to colon cancer screening programs. He also examines a number of factors that affect CEA studies in the health care area in general. One is the need for, but absence of, information from formal randomized clinical trials regarding the effect and value of screening techniques. The information that is available is usually from uncontrolled studies that are burdened with problems of their own. Factors such as leadtime bias, patient-self-selection bias, and length-of-study-period bias also present data problems that must be considered. Another problem for the analyst are the quantitative aspects of trying to measure the costs, benefits, risks, or outcomes of the different colon cancer screening programs.

The author also discusses the special considerations that colon screening programs present to a CEA. These factors include patient characteristics and differences (in terms of effectiveness of screening programs), schedule (or history) and type of testing procedures used, varying accuracy of the different procedures, different origins of the cancer that require separate analysis, order and frequency of testing, and a host of other variables that must be included in a thorough evaluation.

Once, or if, these data and methodological problems are solved, the author feels the central issue can be addressed: What is the value of screening for colon cancer? The author sets out the basic format for addressing the problems involved in a CEA approach. An illustrative example is used to examine the costs and benefits of screening for colon cancer. A screening program for a 50-year-old average risk woman is evaluated using eight different combinations and frequencies of screening tests. The relevant factors (costs, screening regimen, efficacy data, outcome information, etc.) are examined by the author, using a sensitivity analysis approach to determine how the different variables affect the mortality rate and cost of the various screening programs. The result of the analysis is presented as a comparison between the decreasing probabilities of colon cancer’s occurring with more frequent testing, improved life expectancy changes, increases in screening costs, and decreases in lost earnings as a result of the different levels of screening programs.


The study has two major goals: One is to assess the available evidence regarding the benefits and costs of cimetidine in the treatment of peptic ulcer disease; the other is to develop a widely applicable cost-benefit model for evaluation of medical technology. The study combines these two objectives by applying the model to the evaluation of cimetidine and ulcer disease. The authors approach the analysis in three parts: 1) a development and discussion of the cost-benefit model that they feel can be applied to medical interventions in general; 2) an overview of peptic ulcer disease in the United States; and 3) a discussion of the development, diffusion, and use of cimetidine to treat and/or manage peptic ulcer disease.

The foundation of their cost-benefit model is as follows: 1) There are two principal classes of effects—clinical effects and health system effects—and the specific components of these effects depend on the population and intervention being examined; 2) an evaluative model must apply to an identifiable patient population and specific health care interventions; 3) patient population may be defined in terms of a diagnostic category, clinical signs or symptoms, risk factors, or complications of disease; and 4) clinical and health system effects interact to lead to an outcome (health status and/or resource costs).

The authors examine a host of studies dealing with the safety, efficacy, and effectiveness of cimetidine. Among the short-term clinical effects they assess are healing, pain relief, safety and adherence to the treatment plan, complications, recurrence, and recommendations for treating newly diagnosed, uncomplicated ulcers. The long-term clinical effects they examine are recurrence, safety, and complications. They also briefly discuss the pending approval by the Food and Drug Administration of cimetidine for long-term use.

The authors also examine the health system and outcome effects of cimetidine use. Among the variables evaluated are medication, diagnostic tests, physician visits, mortality, morbidity, and resource costs. These three areas—clinical effects, health system effects, and outcomes of
cimetidine use—are the primary elements of the CBA they perform.

The authors also examine and discuss the following findings: Cimetidine promotes healing and provides faster and more complete pain relief for duodenal ulcers; it may be more effective than placebos for patients with gastric ulcers; when used for up to 2 months, cimetidine appears to be a relatively safe drug; most known side effects are minor or reversible; cimetidine plus moderate amounts of antacid costs no more than a therapeutically equivalent course of intense antacid therapy; and maintenance treatment with cimetidine for as long as a year significantly reduced the chance of ulcer recurrence (compared to a placebo) during the period of treatment. Cimetidine, according to a few studies, also appears to have contributed to a sharp decline in surgery for ulcer disease in 1978, as well as to have helped patients to lose significantly fewer days of work than patients given a placebo.

These many findings and conclusions indicate that cimetidine provides a substantial benefit to cost ratio to the peptic ulcer patient and the health care system. The authors cite the findings of two other studies; one by the Netherlands Economic Institute in 1977 and the other by Robinson Associates, Inc., in 1978. The authors conduct an in-depth review and critique of the Robinson study.


This CBA examines the costs and benefits of a public dental care program designed to "maintain the integrity of the natural teeth" in school-age children. Benefits are calculated by estimating the number of teeth "saved" in 5-year-olds that are a result of the program, and then multiplying it by the cost of replacing a natural tooth with an artificial bridge. The current costs of saving a permanent tooth are used as a cost measure. Data from two actual public dental care programs are examined. The authors conclude that public dental care programs must be administered over a relatively long period of time (6 to 7 years) before net benefits begin to accrue on an annual basis. An even longer period of time (11 to 14 years) is required before the programs generate sufficient total benefits to cover total costs. The discounted present values of the program, with use of an 8-percent discount rate, were found to be particularly sensitive to changes in the cost of care and the value of saving a tooth. Extensive sensitivity analysis is performed on the variables involved, making this article an excellent illustration of the use of sensitivity analysis in handling uncertainty.


This article, a general review rather than an analysis, presents an explanation of the rationale behind the use of CEA and CBA in the allocation of health resources and describes an application by HEW. Costs are described as forgone benefits: "The cost of saving a human life is not to be measured in dollars, but rather in terms of alternative lives to be saved or other social values sacrificed." The problem of incommensurability of benefits is discussed. HEW's calculations of the cost per death averted and of productivity and medical treatment savings in various cancer control programs are presented and compared to other health programs (e.g., motor vehicles safety and arthritis). The article illustrates the changes in program priority that can occur when the criterion is changed from deaths averted to savings from avoided medical treatment and loss of productivity (measured as discounted lifetime earnings). The problem of uncertainty is discussed, and a matrix composed of relative payoffs and the certainty of results is presented as one method of handling it. The final section of the article describes in detail the HEW maternal and child health program analysis.


The costs and benefits of providing routine prenatal diagnosis, with termination of affected pregnancies, are examined. The costs of different age groups are discussed. A discount rate of 10 percent is used. The authors examine the changes in the results of the analysis that would occur if, after genetic counseling, only half of the women accepted amniocentesis and possible termination of pregnancy.


This document describes the steps in CBA as the following: 1) articulation of the problem, 2) enumeration of alternatives to address the problem, 3) identification of their achievable effects, 4) measurement and valuation of the
achievable effects, and 5) application of the economic decision criteria. Objectives are described as cost reduction and/or enhancing of benefits. Costs and benefits are classified as direct, indirect, or intangible. The need to focus on incremental, rather than total, costs and benefits is explained. Discounting to present value and the problem of choosing a discount rate are discussed. Five criteria of preferredness are described: 1) net present benefit, 2) internal rate-of-return, 3) benefit-cost ratio, 4) payback period, and 5) average rate of return. The advantages, disadvantages, and appropriate use of each criterion are presented. Threshold analysis, sensitivity analysis, and probability/risk analysis are described as methods of dealing with uncertainty. Common problems encountered in analysis, such as incomplete data, transitional costs, scope, and externalities, and the issue of equity and distribution are discussed.


The authors attempt to determine the best mix of center dialysis, home dialysis, and kidney transplantation in examining the costs and effects of treating chronic renal disease. A quality-of-life adjustment is made to account for the differences in lifestyle between patients on dialysis and those with effective transplants. (The freedom associated with the latter is valued at 0.25 of a life-year.) The calculations in the analysis are based on survivorship tables for transplant and dialysis cohorts of 1,000 each. The authors warn that, at the time of the analysis, there had not been enough experience with any of the three treatment modes to generate an expected life table with great accuracy. The discount rate used is net of an anticipated inflation rate, resulting in a discount rate of 4 percent for transplant and center dialysis and 5 percent for home dialysis. No sensitivity analysis is performed for the discount rate, the anticipated inflation rate, or life expectancy. The authors conclude that kidney transplantation is more cost effective than the other two alternatives. Choice of the preferred treatment modality is independent of the quality-of-life adjustment because transplantation dominates even without the adjustment.


This study examines elective hysterectomy as it is used for sterilization and cancer prevention. The focus of the study is a review of the literature and the issues surrounding the costs, risks, and benefits of elective hysterectomy. The study does not attempt to establish the cost effectiveness of hysterectomy. The authors examine the significant side effects of hysterectomy, such as change in medical utilization and psychological effects following surgery.

The authors review selected studies that evaluate the efficiency and cost effectiveness of elective hysterectomies. Not taking a cost-benefit approach, these studies do not attempt to value the saving of life in monetary terms. The first two efficiency studies that the authors’ review contrast the direct costs of hysterectomy with the net lifetime costs of gynecological care. Future costs are discounted at rates varying from 3 to 6.5 percent. Another study the authors review examines the use of hysterectomy as a sterilization device where the direct costs of tubal ligation plus the expense of future gynecological care which would have been averted by hysterectomy.

The effectiveness of hysterectomies in preventing pregnancy and cancer is not an issue; but the health risks of the procedure are. Efficacy/effectiveness of alternative means to accomplish these objectives are assessed, but not in the cost-effectiveness studies reviewed. Additionally, the cost-effectiveness studies which are reviewed do not attempt to identify, measure, or place a value on the side effects of surgery.

Costs are distinguished from charges and issues of equity are discussed. The authors do not employ a sensitivity analysis. Conclusions are drawn with respect to the cost effectiveness of elective hysterectomies as they are used for the separate purposes that are examined.


This study reviews the literature on the cost effectiveness of nurse practitioners to provide primary medical care services. Only limited data are available, and much of the information deals with other types of physician extenders. In addition, many of the data have been gathered in the developmental stage of introducing nurse practitioners; the relevance of these data for present policy purposes is unclear.

At least theoretically, nurse practitioners offer the potential to reduce the costs of health care and improve access to the health care system. Nurse practitioners can perform basic and routine medical care tasks, allowing physicians to focus their efforts on serious illness problems. Training costs and pay are less for nurse practitioners than for physicians, so costs should be lower for routine care if nurse practitioners are used. There are a number of problems in directly extrapolating to lower costs, however, and, depending on the system within which nurse practitioners operate, cost savings may or may not be realized.

A key question examined by this study deals with the nature of the services nurse practitioners perform and how they affect costs. In general, they provide complementary and substitute services, although the nature of these services is difficult to document because data often indicate only “office visit.” Complementary services would include

● With the technical assistance of Mitchell LaPlante
treatment such as “well baby care,” while substitutive services refers to such treatment as “physicals.”

In terms of quality of care, nurse practitioners appear to provide care that is of as high quality as that of physicians (with whom they usually work and are compared). There is some evidence that nurse practitioners, working in close conjunction with physicians, provide superior care when compared to solo practitioners. Productivity is more difficult to assess and depends on how nurse practitioners are used. There seems to be clear evidence that the use of nurse practitioners improves physicians’ productivity, but it is not clear how this improved productivity affects costs. Supervisory time, duplicative work, and the fact that nurse practitioners spend more time per patient must be considered.

The data needed to conduct a CEA of nurse practitioners include employment costs, training costs, and medical care costs. Unfortunately, each of these factors may be subject to changes as a result of alterations in another part of the system. The employment costs of nurse practitioners, for example, is a function (in part) of the demand for their services. Even more difficult to determine is price. Because they are most often hired by physicians or health institutions which have already established fee systems, any cost savings may be absorbed by the physicians or institutions and may not be reflected in the price of services delivered.

The case concludes by cautioning against the use of current data to determine new policy. Based on changes in the way nurse practitioners are used, costs could vary widely. This is a case in which an actual CEA may provide misleading policy advice, although the identification of variables required by the CEA may be very helpful.


This analysis was one of a series of federally sponsored efforts to assess the costs and benefits of alternative approaches to the problem of kidney disease. The approaches included screening, prevention, and three treatment modalities (home dialysis, center dialysis, and transplantation). Employing a variety of assumptions (e.g., risk population for the screening programs, size of treatment facilities), the authors concluded that early detection dramatically dominated the treatment approaches with respect to economic benefits and costs. Depending on the population screened, the former had benefit-cost ratios of 30:1 and greater. By contrast, the treatment alternatives produced benefit-cost ratios in the vicinity of 1:1. This ratio varied according to: 1) the treatment method (transplantation producing the highest ratios); 2) the scale of operation; 3) the allocation of research costs; and 4) high, low, and best cost estimates in the two instances of dialysis. To estimate indirect benefits (i.e., productivity losses avoided), the authors assumed that 70% percent of the dialysis patients would be capable of resuming a normal earning capacity; the remaining 30 percent were assigned half the expected income of a comparable but healthy individual.

The qualitative findings of this analysis were supported by other studies undertaken at the same time. Despite the consensus that prevention and early detection were the most cost-effective approaches to dealing with the kidney disease problem, Federal policy was directed toward the alternative which appeared least economically desirable, center dialysis.


This study examines the many factors that have played a role in the development of the artificial heart: factors that are affected by, and in turn affect, three areas of public policy—R&D, reimbursement, and regulation.

The authors provide a backdrop of the history of the artificial heart development program. They also examine the safety and efficacy determinations that have been arrived at through experiments and clinical trials. The current and potential technological developments that are or will be part of the artificial heart are described, and the numerous R&D needs that must be met before an artificial heart can be successfully used are examined.

The authors examine the economic aspects of the artificial heart from the patient’s perspective and from a societal view, focusing on the costs of diagnosis, implantation, and postoperative care. These costs are compared to the costs associated with related procedures: cardiac pacemakers, aortocoronary bypass surgery, and heart transplants. The renal dialysis program is used to illustrate the possibility of the Federal Government’s financing artificial heart procedures and the distribution of services to the population.

The authors also examine four social cost areas: increased social expenditures, potential distributional inequities, effects of nuclear radiation if a nuclear energy source is used, and the opportunity costs. They also examine the efficacy, potential benefits and costs, and likelihood of saving lives by cardiac disease prevention programs.

Quality of life issues are addressed for both the short- and long-term effects. The authors draw on the experiences of those who have had heart and kidney transplants to illustrate the types of impacts on the patient and the family that can occur. The potential effects include personal, marital, family, physical, medical, and psychological problems that can occur after a person undergoes major surgery. The authors also discuss the added burdens/impacts that will result if a nuclear-powered energy source is used.

On the benefits side, although the authors briefly discuss the technological spinoffs of the artificial heart program, their primary focus is on two areas: the potential for patients returning to an active life, and the estimated years of life that may be gained. Morbidity, mortality, and added years of life are examined and estimated via a best case and worst case analysis if the artificial heart is implanted.

This author believes that one important reason for the reluctance to fund disease prevention/health promotion programs is that benefits often do not accrue sufficiently to those who pay for the programs or who are expected to fund such programs. Consequently, traditional CEA's which are performed only from the societal perspective may not be applicable for public policy.

This author performs a CEA of cervical cancer screening for a given risk group from different perspectives. Screening for cervical cancer is used to demonstrate the cost effectiveness of disease prevention programs. The disease process is modeled by the author using a Markov Chain technique to “age” a simulated population of 30- to 39-year-old women for 10 years (using disease transition probabilities reported in the literature). The cost effectiveness of screening is then calculated at different intervals—ranging from annual screening to no screening for the 10-year period. The effects are evaluated for: 1) different migration patterns, 2) different risk groups, 3) different modes of administering Pap tests, and 4) joint production considerations. The author also tests the sensitivity of the results to various discount rates and to the range of error rates for Pap tests.

The results indicate that a private party always has a financial incentive to postpone screening, whereas society finds it more cost effective to screen, but only at infrequent intervals. In addition, the author notes, the cost effectiveness of screening is markedly affected when a more efficient (i.e., less costly) delivery mode is simulated. Screening is significantly affected when joint production effects are considered. The cost effectiveness of screening, however, is not very sensitive to small changes in the discount rate, initially set at 10 percent, nor to varying assumptions regarding error rates.

The author concludes that if society wants the private sector to screen for cervical cancer at a socially determined optimal rate, then society must be willing to subsidize the cost of the program. The study also concludes that the cost effectiveness of cervical cancer screening is much more affected by the cost assigned to screening than by different assumptions of the precise error and discount rates.

The cost effectiveness of screening at each simulated interval was compared to no screening for a 10-year period. Efficacy information was addressed and different test error rates were used. The production of the Pap test was simulated, for cost purposes, at two levels: an expensive university hospital clinic using specialists, and an inexpensive health clinic using licensed nurses. Only lives and years of life saved were identified as benefits.

Costs were distinguished from charges, marginal costs were considered, and indirect costs are used. Discounting of costs and benefits was done (rates tested: 0, 5, 8 to 12 percent), and sensitivity analysis was performed; however, issues of equity were not directly considered in the analysis.


The author argues that conventional CBA and CEA should be extended to include a predictive analysis of the implementation phase in order to determine whether and how the project will be done. The predictive analysis involves three steps: 1) a standard CBA to determine whether the project should be undertaken; 2) a CBA from the perspective of each decisionmaker or interest group capable of influencing the success of the project to determine the likelihood that the project will be undertaken; 3) a redesign of the project or the development of incentives to improve the likelihood of success for socially desirable projects.

In a case study of a surgicenter, it is noted that the resultant shifting of a revenue from one set of providers to another, though only a pecuniary externality in standard benefit-cost analysis, has a substantial impact on the likelihood that a surgicenter will actually be implemented. The importance of identifying decisionmakers and their respective power to influence the success of the project is discussed. The author points out that in the second step of the analysis (the “interest-group analysis”), transfer payments, taxes, and pecuniary externalities should be explicitly considered, so that the financial flows as perceived by the relevant interest groups are adequately represented. In addition, it may be appropriate to use substantially different discount rates for each interest group. The final step in the interest-group analysis is to estimate each group’s utility function and the group’s relative power to either promote or block implementation of the project.

Luft presents an application of predictive analysis to the use of a work evaluation unit for ascertaining functional work capacity following a myocardial infarction. The relevant interest groups in this analysis include the patient, family, physician, employer, insurer, and society. Luft estimates both the likely net effects on each interest group of using the work evaluation unit and each group’s relative weight. The author concludes that this extended, positive form of benefit-cost analysis can improve the allocation of resources by helping to promote the implementation of desirable and feasible programs and “to prevent the adoption and implementation of proposals that appear promising in theory but are likely to be sabotaged in practice.”


The authors measure the value, in terms of sensitivity and specificity, of intravenous pyelography and radiopharmaceutical renography as diagnostic screening methods for hypertension caused by renovascular disease. Costs associated with both diagnosis and subsequent surgical treatment are also calculated. Financial costs of the diagnostic procedures are based on the Massachusetts Relative Value Scale; hospital and operation costs are based on 1974
but the risk that the hernia will recur and require additional operations (with a high mortality rate). Using data obtained a calculation of the average effects of 1) having an average number of life-years lost. The results of the analysis data are attempted. The magnitude of the costs involved in the elective operation is noted, but a detailed analysis is not presented. On the basis of this study, the author observes that medicare funds expended on elective herniorrhaphy serve, if anything, not to increase life expectancy, but rather to improve the quality of life. He asks, therefore, if these funds might better serve to improve the quality of life for the elderly in some other way (such as in reducing subway fares for the elderly).


This article examines the costs and effects of the sixth sequential stool guaiac for screening asymptomatic colonic cancer. An analysis of the expenditures concludes that costs rise exponentially, so the marginal cost of the sixth test may be 20,000 times the average cost. In addition, data indicate that there is little gain in the true positive rate from testing beyond the second guaiac examination. Thus, the costs per true positive become gigantic. The marginal cost is decreased with lower test sensitivity and increased with lower prevalence of colonic cancer. The authors conclude that defining a high-risk group, which would serve to lower marginal cost, is essential to justify such screening programs in a world of constrained resources.


This article presents a quantitative approach to the costs and benefits associated with the “interventionist” and “noninterventionist” management of suspected appendicitis. The assessment considers lives, postoperative disability, and economic costs. Since the author relied on the rather scanty data from the available literature and on many simplifying assumptions, however, he cautions that the analysis should be viewed as “paradigmatic rather than definitive.” The analysis addresses the question of when to operate, not alternative strategies, such as a dietary prevention program or antibiotic therapy.

On the basis of two symptoms (location and severity of pain) and two signs (presence of right lower quadrant rebound tenderness and rectal tenderness), an “appendicitis risk score” was developed. Twenty-four symptom combinations were developed and the probability of appendicitis for each combination was determined and ranked.

For example, the highest rank (24) corresponds to the combination of right lower quadrant rebound tenderness and rectal tenderness, an “appendicitis risk score” was developed. Twenty-four symptom combinations were developed and the probability of appendicitis for each combination was determined and ranked.

15 deaths for every 100 surgical cures. The cost-effectiveness calculations are not sensitive to varying the assumptions regarding the prevalence of renovascular disease in hypertension patients from 10 to 5 percent.


This CEA reviews available data in order to see what effect the choice of elective herniorrhaphy v. truss has on the life expectancy of a 65-year-old person. The analysis includes a calculation of the average effects of 1) having an immediate elective herniorrhaphy (with its low mortality, but the risk that the hernia will recur and require additional operations); and 2) using a truss (with its attendant risk of obstruction, followed by an emergency operation with a high mortality rate). Using data obtained from the relevant literature, the authors estimate: 1) the mortality rates associated with (a) elective and (b) emergency surgery, 2) the probability of recurrence of the hernia after an operation, 3) the yearly probability of strangu- lation, and 4) the life expectancy of the patient. Two sets of numbers are used in the analysis. The first set of numbers serves as a conservative test of the hypothesis that the truss prolongs life, because the values in this set are those which systematically place the benefit of the doubt in a direction favorable to the elective operation. The numbers in the second set are based on what seem to be the most reasonable and reliable data. (The author notes that there are insufficient data to consider a “do nothing” third alternative.)

The model takes the form of a decision tree, which is designed so that the “payoffs” equal the expected value of the average number of life-years lost. The results of the conservative test (used because it makes the strongest case for the elective operation, which is standard of surgical practice uniformly proposed by current surgical literature) indicate that the elective operation has a higher loss of life associated with it for the 65-year-old than the truss does. The test using the “most reasonable” estimates indicates that the elective operation has a mortality rate 5.5 times greater than the truss. This large relative difference, however, translates into an absolute difference of only 14.29 days. The author notes that in view of this small absolute difference in mortality, the issue of quality of life becomes important. The article concludes with a discussion of this type of adjustment, but no quality-of-life adjustments on the analysis data are attempted. The magnitude of the costs involved in the elective operation is noted, but a detailed analysis is not presented. On the basis of this study, the author observes that medicare funds expended on elective herniorrhaphy serve, if anything, not to increase life expectancy, but rather to improve the quality of life. He asks, therefore, if these funds might better serve to improve the quality of life for the elderly in some other way (such as in reducing subway fares for the elderly).


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...and noncases, and 3) the net costs of the false negatives and false positives in terms of mortality, convalescence, and direct hospital costs. Two analyses are performed, one assuming that 100 percent of the appendicitis patients on whom surgery is not performed will perforate, the other assuming that 30 percent will perforate. The results indicate that a surgeon can ensure an acceptable mortality rate by taking an “interventionist” approach, but only at the cost of increasing convalescent days and hospital costs. Relaxing the indications for surgery to include patients who lack the most obvious symptoms saves lives, but at an ever diminishing rate. The few lives saved by operating on patients with minimal symptoms are purchased at great costs in convalescence and dollars associated with the removal of large number of normal appendices. The author suggests a solution to this dilemma—namely, increasing discrimination by using very complete diagnostic information and careful clinical interpretations. Increased discrimination can reduce the removal rate of normal appendices. Medical care costs and health effects associated with a preventive program are explored from the perspectives of society and of a third-party payer such as medicare.


This study includes an examination of the cost effectiveness (CE) of applying a primary preventive technology—vaccination against pneumococcal pneumonia—to different age groups. Medical care costs and health effects associated with a preventive program are explored from the perspectives of society and of a third-party payer such as medicare. A CEA was used to calculate the expected change in health effects and medical costs from vaccination against pneumococcal pneumonia—an alternative compared to continuing the present situation in which pneumococcal pneumonia is treated if it occurs. In the analysis, costs were limited to expenditures and savings within the medical care sector, and changes in health status were expressed in years of healthy life. Thus, the cost-effectiveness ratio represented the net medical cost per year of healthy life that would be gained by a vaccinated person. The calculations were based on a single hypothetical vaccination program conducted in June 1978. The analysis used a simulation model to estimate the costs and effects that would result from 1978 to 2050 for two closed populations, one vaccinated and the other unvaccinated. Costs and effects were discounted at 5 percent per year. Separate cost-effectiveness ratios were calculated for five different ages:

- 2 to 4 years,
- 5 to 24 years,
- 25 to 44 years,
- 45 to 64 years,
- 65 years and older.

The analysis employed a sensitivity analysis to test the effect on the results of varying the values of several uncertain parameters over reasonable ranges.

Net health effects were expressed in quality-adjusted life years (QALYs). Mortality rates for pneumonia as an underlying cause of death provided by the National Center for Health Statistics (NCHS) formed the basis for estimating 1978 pneumonia mortality among the unvaccinated. Unpublished age-specific data from the Health Interview Survey conducted by NCHS was used to estimate the days of pneumonia morbidity among the unvaccinated.

Medical care costs, expressed in dollars, included additional expenditures for vaccinations and for treatment of vaccine side effects; reduced expenditures for treating pneumococcal pneumonia that would be expected to occur without vaccination; and additional expenditures for other illness in the extended years of life gained by vaccinees who avoid death from pneumococcal pneumonia. Unpublished age-specific data from the Hospital Discharge Survey and the National Ambulatory Care Survey of NCHS was used to construct estimates of the costs of treating pneumonia.

The study found that, given the range of factors involved, vaccinations would entail positive medical expenditures for every age group and would be most cost effective for those 65 years or older. The cost-effectiveness ratio was about $4,800 per QALY gained for all ages and $1,000 per QALY for ages 65 and older. The analysis found that vaccination of 21.5 percent of the population 65 years and older would result in a net cost to society of about $23 million and would yield about 22,000 QALYs over the lifetimes of those vaccinated. The study also concluded that vaccination for all age groups in the population would have a net cost of about $150 million for a gain of 31,000 QALYs.

The study also examines policy implications of these findings, including a possible change in the medicare law to permit Federal payment for pneumococcal vaccine for the elderly.


In this article, a mathematical relationship is derived between the benefits and costs of a treatment in a given disease and the threshold level of clinical suspicion of the disease. When the probability of a patient’s illness exceeds this threshold level, the better choice is to administer treatment; when the probability is below the threshold, the better choice is to withhold treatment. The benefit equals the net benefit of appropriate treatment and is calculated as the difference between the utility of administering treatment and the utility of withholding treatment from patients who could benefit from it. The cost is the net cost of unnecessary therapy and is calculated as the difference between the utility of avoiding treatment and the utility of administering treatment to those who do not have the disease. Using probabilities, the authors develop equations expressing the expected values of treatment and no treatment. The point of indifference as to course of action is where the expected value of treatment equals the expected value of no treatment. The probability value at the indifference point is the threshold level. Using this concept in a clinical setting requires assessing the probability of the disease in a given patient and determining whether it is above or below the threshold level. A unique threshold value must be calcu-
lated for each disease and its treatment in a given cohort of patients (defined as having common risk characteristics). Sensitivity analysis may be employed when significant uncertainty surrounds the probabilities and utilities involved in the calculations. In addition, if the clinical status of the patient or if the circumstances of administration of the therapy differ notably from the typical case, the benefits and/or costs must be adjusted appropriately.


This paper examines the role of formal analysis in Federal decisionmaking related to end-stage renal disease rather than that of CEA per se. The study places special emphasis on institutional factors encouraging or inhibiting the use of formal analyses. These are defined as "any explicitly analytical means of systematically examining the social costs and benefits of alternative policies for the purpose of choosing a preferred alternative in light of an a priori normative decision rule. " CEA and CBA fit this definition, as do risk-benefit and cost analyses.

The case study presents information on patients with end-stage renal disease. The author notes that the proportion of men in the total patient population on dialysis declined between 1970 and 1976. The average age of dialysis patients increased, and the proportion of home dialysis patients declined from 40 percent in 1972 to 24 percent in 1976. The number of dialysis patients in the Medicare program has risen from 14,000 in 1973 to 50,000 in 1978.

The paper deals primarily with the impact of two formal analyses of end-stage renal disease issued in 1967: 1) the "Gottschalk report," prepared by an expert advisory committee for the Bureau of the Budget; and 2) the "Burton report," prepared by a Public Health Service task force for the U.S. Surgeon General. (The paper mentions several other formal analyses but focuses on these two.) The author describes policy-related and institutional bureaucratic factors that led to the conduct of these formal analyses and that affected the form the analyses took along with many of their methodological assumptions. The author also describes and summarizes the results of the CEA in the Gottschalk report and of the "costs and benefits" analysis in the Burton report.

The author then addresses the effects of both reports. The Gottschalk report, for example, led the Bureau of the Budget to fund a Veterans Administration (VA) administered hemodialysis program that included a substantial portion of the VA dialysis patients. The Burton report, according to the author, had no direct program effects.

On the whole, this study suggests that formal analysis "did not affect the fact that the policy choice was a basic political choice." Yet the paper also notes that the analyses may have raised the consciousness of high level policymakers as to cost implications. The paper also mentions some of the factors that limit the effect of analysis such as inadequate data, lack of access of analysts to decisionmakers, and difficulties in making assumptions that frame the problem.


This case study prepared by Leonard Saxe, was based on a document prepared for OTA by Brian Yates and Frederick Newman. It describes a variety of methodological and substantive problems that arise in assessing the effects of mental health treatments. The report both summarizes the existing literature and attempts to present the divergent perspectives within the research-policy community concerned with psychotherapy. As described below, it deals with four issues that are centrally related to the evaluation of psychotherapy.

Definition. Psychotherapy is not a simple intervention, and part of the confusion about its effectiveness has to do with reviewers' use of different definitions. The present report uses a relatively broad definition of psychotherapy in order to best represent current therapy practice. This definition includes treatments based on Freudian ideas about psychodynamics, as well as newer therapies based on theories of learning and cognition. The report also notes that psychotherapies are not distinguishable only by their theoretical bases. In addition, patient variables (e.g., intelligence), therapist variables (e.g., empathy), and the nature of the treatment setting affect the nature of psychotherapy. Although the inclusion of such factors makes the analysis of psychotherapy more difficult, there seems to be ample evidence as to the importance of these factors on the outcome.

Assessibility. Although psychotherapy itself is complex and there is no clearly agreed upon way of viewing it, the methods for assessing psychotherapy seem better established. The report describes the variety of experimental and quasi-experimental designs that have been used in assessing psychotherapy, along with an analysis of what types of information can be obtained by application of these techniques. The report also describes and analyzes various methodological strategies for measuring the outcomes of psychotherapeutic treatment and the ways in which the reliability and validity of measures are established. Unfortunately, research practice does not always meet these standards. Some explanations offered in the report include the difficulties of withholding treatment and the problems of assessing effects over time. The report also considers the recent development of systematic procedures for synthesizing the findings of multiple investigations. The problems of such techniques, as well as their promise for detecting valid trends in the research literature, are analyzed.

Efficacy. The report describes some of the plethora of research which has been conducted on psychotherapy. The focus of the report's efficacy analysis is a discussion of six important earlier reviews of the psychotherapy literature. In addition, many of the evaluative studies themselves were reviewed. Despite some fundamental differences,
both in the criteria they develop for assessing psychotherapy and the studies they include for review, the reviews all seem to support the findings that (under specified conditions) there is evidence as to psychotherapy's effectiveness. In fact, with the exception of reviews that focus on psychoanalytically oriented therapies, there seems to be little negative evidence as to efficacy of such treatments. Although it is difficult to make global statements, the evidence seems more supportive of psychotherapy than of any alternative hypothesis (spontaneous remission, placebo effects). However, there is a great need for well-conducted research which evaluates psychotherapy for specific disorders under specified treatment conditions. This research would need to be carried out in actual delivery settings.

Cost effectiveness. The application of CEA/CBA to psychotherapy is much more recent, and hence less developed than efficacy research. Nevertheless, a number of models are available for conducting such analyses. In general, the models are based on those used in other applications of CEA/CBA, and the problems engendered by their use are similar. A particular concern with such psychotherapy assessments is whether costs and benefits can be comprehensively measured. Thus, for example, although the costs of psychotherapy treatment are relatively easy to measure, it is more difficult to determine and quantify what type of benefit has been achieved. Much of the CEA/CBA research to date has involved a comparison of psychotherapy treatments. Although such research indicates the potential use of CEA/CBA to improve the functioning of clinical settings where psychotherapy is given, its usefulness is less clear. Such work seems possible, however, and may potentially be incorporated as part of large-scale efficacy assessments.


This study is an examination of the scientific and technical issues that are part of the debate over the appropriate approach to detecting and treating breast cancer. The major focus of the analysis is devoted to the overview, discussion, and evaluation of the various types of surgical and nonsurgical procedures used to treat breast cancer. Cost-effectiveness considerations, however, are not totally ignored. The authors note that the resolution of the detection and treatment issues will have major cost and benefit implications. The authors also perform a hospital cost analysis of two different treatment strategies—inpatient versus outpatient tissue biopsy.

The background of the study is established by a brief overview of the extent and effects of breast cancer in America. A history of cancer of the breast is presented, as is a description of the development and popularization of the Halsted method of performing radical mastectomy procedures to treat breast cancer. Developed in the late 1880's, the Halsted method remained the generally accepted “treatment of choice” for over 80 years—in 1970, 80 percent of breast cancer patients in the United States received radical mastectomies.

Variations of the Halsted method and completely new approaches to treating breast cancer (both surgically and nonsurgically or a combination of both techniques) over the last two decades have challenged the traditional Halsted technique. In this paper, the authors examine the evidence regarding the efficacy, safety, mortality, and morbidity of these new techniques, as well as that for the Halsted method.

The six treatment procedures they examine are: 1) radical mastectomy, 2) extended radical mastectomy, 3) modified radical mastectomy, 4) simple or total mastectomy, 5) partial mastectomy, and 6) local excision, lumpectomy (or tylectomy). Special emphasis is placed on reviewing the status of the nontraditional methods of treating breast cancer, i.e., those procedures that run contrary to the Halsted approach (radical mastectomy). Also discussed are the roles of three American surgeons—Dr. Leslie Wise, Dr. George Crile, Jr., and Dr. Oliver Cope—who have long advocated and practiced a more limited surgical approach to treating breast cancer. Their investigations and results regarding the success of using non-Halsted procedures to treat patients are examined.

The authors summarize the debate by discussing the results of the National Cancer Institute's consensus panel meeting on the topic of breast cancer treatment held June 5, 1979, at the National Institutes of Health. In essence, the conclusion was that much work is left to be done in evaluating the various techniques. The conference recognized the potential of the nontraditional procedures and the value of the total mastectomy as used in place of the Halsted radical procedure for certain women. More information is needed regarding the efficacy and safety of the alternative procedures; segmental mastectomy, primary radiotherapy, etc. Over the last few years, the modified radical procedure has become more popular than the Halsted radical technique, but there is still no general consensus on what procedure(s) should be the treatment of choice.

According to the authors, there is good evidence that survival rates are no better for the radical procedures than for the less severe techniques available. Why then is there still adherence to the more drastic approach? The authors set out a number of micro and macro issues that may help explain the continued reliance on the Halsted method: cultural and traditional reasons, economic incentives, individual personalities and reputations, existing logic of cancer treatment, structure of the medical specialties, burden of proof requirements on innovators and traditionalists, medical conservatism, and the scaling of evidence.

The authors' cost analysis, as mentioned above, is a comparison of the cost differences of inpatient versus outpatient tissue biopsy. The authors consider these alternative strategies in light of the number of cases of breast surgery at Massachusetts General Hospital in 1976 and the total number of procedures for the United States in 1975. Their calculations and extrapolations determined that $185 million (excluding radiation therapy) or a 45-percent reduction in total costs would result per year if outpatient biopsies were used uniformly and radical surgery were re-
placed with more limited surgery. However, as the authors note, the reader must realize the very approximate nature of cost analysis. Nevertheless, the authors feel that the magnitude of the cost differences warrant a more complete investigation.


This study is basically a cost analysis of alternative methods to deliver respiratory therapy. The authors describe the technology of respiratory therapy, the indications for the use of each type of therapy, and the substitutability of different modalities. The authors also review the literature on effectiveness and conclude that respiratory therapy’s efficacy and effectiveness has not been adequately proven and is still in dispute.

The paper describes an empirical survey which the authors undertook in the metropolitan Washington, D.C., area. Using data from that survey, the authors chart the utilization of respiratory therapy techniques by type of hospital and by number of beds. They also chart the trends in use from 1976 to 1979, noting a shift from the more expensive high-technology oriented therapy (IPPB) to the less expensive simpler aerosols and spiroimeters.

In their cost analysis, the authors compare each type of therapy with another. Cost savings of the shift in technology are estimated. By focusing on a cost comparison analysis, the authors implicitly assumed that efficacy and effectiveness across therapies are constant. The costs of one therapy are compared with those of the others.

The adequacy of efficacy and effectiveness information is addressed (and found to be inadequate). Specific benefits and effectiveness are not identified, measured, or valued. Costs are distinguished from charges, and “avoidable,” or incremental costs are identified. The indirect costs (lost production) are not identified. Discounting is not used (costs are incurred in the present, future benefits are not projected). The resultant complications, and intangibles. Both private and public policy considerations are discussed.


The authors estimate the costs and benefits of various rubella vaccination strategies, each at 100- and 80-percent compliance. Benefits are the savings that result from the prevention of both acute rubella and congenital rubella. The direct costs of rubella (and hence the direct cost savings from prevention) are the costs of medical care, medication or special devices, and special education or rehabilitation. Indirect costs result from temporary disability during acute illness and complications, in addition to deaths from purpura or encephalitis, and from permanent disability that results from congenital rubella syndrome. The costs of rubella vaccination were estimated on the basis of the cost of measles vaccination. Vaccination at ages 10 to 12 appears preferable to vaccination at ages 1 to 3 for two reasons: 1) because the gap between vaccinating and realizing benefits from prevention of congenital rubella is shorter the closer vaccination is to childbearing; and 2) because the net benefits of preventing congenital rubella are greater than those associated with preventing acute rubella infection. The latter reason was demonstrated by employing conservative assumptions: Only the most obvious abnormalities associated with congenital rubella were included in the analysis, and the number of clinical cases of acute rubella was probably overestimated.

The results indicate that the economic benefits of a rubella vaccination program, assuming 100-percent compliance, are greater if offered once to females at age 12 rather than to children of both sexes at age 6 or younger. If compliance is 80-percent instead, the least number of babies with congenital rubella will be born when vaccination is offered twice, once to children of both sexes at the age of 2 and again to females at the age of 12. Finally, the analysis indicates that if the vaccine is to be offered to children at or before age 2, it is more effective to use combined measles and rubella vaccine.

A 6-percent discount rate is used throughout the analysis, with no sensitivity testing done. It is assumed that complications of rubella vaccination are under consideration are negligible. The frequency of rubella infection was estimated on the basis of two serologic surveys.


This CBA examines alternative strategies for a swine influenza vaccination program. The benefits of a vaccination program are described as the product of the direct and indirect costs that would be incurred in the event of an epidemic, the probability of an epidemic, and vaccine efficacy. The costs involved in the program include those associated with vaccine production and administration, resultant complications, and intangibles. Both private and public sector programs are examined. The Delphi method is used to obtain information regarding the probability of an epidemic, age-specific morbidity and mortality rates for both total and high-risk populations, vaccine efficacy and side effects, and vaccine acceptance rates. The net benefits for three strategies, which vary by age and risk of the target population, are calculated. The probability of an epidemic, vaccine efficacy, and vaccine acceptance rates are subjected to sensitivity analysis. The three strategies under consideration were found to be sensitive to acceptance rates. The results of the analysis indicate that expected net benefits are not maximized by the vaccination of everyone over 5 years of age. A policy of orienting the program toward the general adult population can be justified with low vaccine-administration costs, high vaccine efficacy, and high acceptance rates (59 percent), assuming further that the flu strain represents a potential pandemic. Otherwise, only high-risk group vaccination is warranted.
A major feature of this study—both in its design and achievement—is demonstration that a sound, useful analysis can be initiated and completed in a matter of weeks.


The study is a CEA of a highly technical and very costly emerging medical technology. The cost and effectiveness (lives and years of life saved) data the authors use were empirically derived from the Bone Marrow Transplant (BMT) Program at the University of California at Los Angeles. Much of the effectiveness data had been previously published. Quality of life data was collected by a single observer, a BMT Program nurse.

Patients with aplastic anemia and leukemia were studied. Since there were insufficient resources to allow all eligible patients into the BMT Program, patients who received transplants were compared to those who were judged eligible but not selected. The sample sizes were very small and survival data was limited to 3 years as a result of the newness of the technology.

Bone marrow transplant procedures are compared to conventional therapy, as opposed to no treatment, even though there is no indication that conventional treatment is efficacious. The cost of transplant procedures is considered to be the incremental—or avoidable—cost above what would have been spent anyway.

Efficacy data is empirically derived from the study of patients admitted to the program, extrapolated to normal life expectancy for “successful” transplants (defined as those patients still living after 3 years), and compared to the group of nonselected patients. The production process described is one currently in place (this is an emerging technology).

A wide range of benefits is identified, and an attempt is made by the authors to value and combine quality of life with projected increase in life. Hospital charges are used for costs, and incremental costs are identified and included in the analysis. Indirect costs are also calculated. Discounting is not used for future benefit (years of life saved) valuations. All costs were assumed to occur in the present. Sensitivity analysis is not used. Bone marrow transplantation is still being employed in a research mode, so equity issues are mainly relevant to the patient selection process; such issues are not directly addressed in this study.

The results of the analysis are expressed as a cost-effectiveness ratio (cost per year of life saved). The authors do not qualify these results by discussing the confidence which the reader can place in them. An extensive discussion on the relevance this study has to public policy is presented. The cost-effectiveness ratios developed for bone marrow transplant procedures are compared to the cost-effectiveness ratios for other life-saving programs.


This report examines the use of the fiberoptic endoscope to visualize the upper gastrointestinal (UGI) tract from the esophagus to the upper portion of the small intestine. The study covers the effectiveness and economic costs of this common form of endoscopy. Issues related to evaluating endoscopy’s benefits and costs are discussed, though no formal comparison of costs and benefits is undertaken.

The authors describe the technique of endoscopy and the device used—the fiberoptic endoscope. They briefly touch on training in the technique and identify the common medical indications for endoscopy’s use.

The report discusses the clinical effectiveness of UGI endoscopy, which is used to diagnose conditions of the UGI tract and to obtain specimens of tissue. The medical indications for use are quite broad and inclusive. Studies of the diagnostic value of the technique suggest that endoscopy significantly contributes to the amount of diagnostic information. Very often, however, the medical condition being diagnosed is such that the information gained does not improve morbidity or mortality for the patient(s).

The authors state that the most common dangers associated with endoscopy are perforation (esophagus or stomach), bleeding, cardiopulmonary effects, and infection. These complications are relatively rare, yet not insignificant, given the large number of endoscopies performed nationally (at least 500,000 each year).

The authors distinguish between the cost of perform in, the procedure and the charges for it. Using data from California, they provide a median charge of $240, and by extrapolation, a total national expenditure of $122 million. Using a hypothetical cost analysis, they then estimate that the average cost to a physician for performing a routine procedure ranges from $41 to $83.

The study addresses issues in evaluating benefits and costs of endoscopies. The authors point out the difficulties of adequately estimating the value of a diagnostic procedure such as endoscopy. They cite the difficulties of conducting a clinical trial ethically when conditions such as gastric cancer are involved. They also cite other difficulties, such as problems in extrapolating from the results of clinical trials in the event that such trials were conducted. The authors maintain that cost-effectiveness studies would be limited in their usefulness because of these difficulties in assessing benefits. Though theoretically possible, measurements of costs and benefits are unlikely since such measures cannot realistically be made sensitive enough to provide an accurate and useful assessment for decisionmakers.

The authors also discuss the use of endoscopy and policy considerations, such as incentives leading to its use and the regulatory issues involved. Finally, the need for increased investigation of more narrowly defined indications for use of endoscopy is discussed.

The objectives of this analysis are to predict the future medical costs and life expectancy of patient cohorts in facility dialysis, home dialysis, and cadaveric transplantation over the next decade and to estimate the cumulative effect on costs and life expectancy of successive 1,000-patient cohorts, changing methods of treatment in each of the 10 years. Three treatment transition options are evaluated: 1) facility dialysis to home dialysis, 2) facility dialysis to cadaveric transplantation, and 3) home dialysis to cadaveric transplantation. Both costs and life expectancy are discounted at a rate of 7 percent, which is not subjected to sensitivity analysis. The 10-year survival and cost estimates are obtained through linear extrapolation of recent data trends. The experience of the cadaveric-transplantation cohort is predicted for two survival-rate assumptions. The low assumption is based on rates reported in 1976, and the high assumption is an estimate of the average survival rates that will be experienced nationally over the next 10 years. The results of the first phase of the analysis indicate that, over the next decade, each of the dialysis cohorts is predicted to have more added years of life than the transplantation group. Though the predicted number of life-years for both forms of dialysis is approximately equal over the 10-year period, treatment for the home-dialysis cohort will cost about $43 million less than that for the facility-dialysis cohort. Transplantation is less costly than both forms of dialysis.

The second phase of the analysis indicates that undergoing home dialysis instead of facility dialysis (the first option) provides approximately the same life expectancy, but at 34 percent lower costs. The second option, moving from facility dialysis to transplantation, also results in a substantial reduction in costs, but there is an accompanying reduction in life expectancy as well. The third option, moving from home dialysis to transplantation, has results similar to those of the second option. The authors conclude that while it is clear that there are potential savings to society from public policies that encourage patients who are able and willing to shift from facility to home dialysis, an evaluation of the two dialysis-to-transplant options is ambiguous. Transplantation is less costly than dialysis over the 10-year period, but attention must also be paid to the impact of the shift in life expectancy. No cost-effectiveness ratios are presented. The authors caution that the intent of their analysis is not to promote any specific form of treatment, but rather to provide information, such as the relative magnitude of the “tradeoffs” between cost reduction and life expectancy in each of the treatment options.


The authors of this analysis examine a considerable range of issues that deal with the recent growth and expanded use of cardiac radionuclide imaging technology in the health care field. The areas they address are the present and potential future characteristics of the technology; the market for and industry involvement in cardiac imaging innovations; the uses and users of these procedures; the clinical efficacy and risks associated with the techniques; the costs and charges of imaging technology use; and the cost effectiveness of these procedures in different service delivery situations.

The authors point out that much of the rapid diffusion and use in this area is taking place without a well-grounded understanding of the benefits and limitations of the various scanning techniques. To date, only very selected patient populations have been evaluated out of a much broader spectrum of uses and techniques available and in use. Adding to the uncertainty are the rapid technological changes that are occurring and the poorly defined target population for cardiac scans.

Using the various suggested clinical indications and uses as a backdrop, the authors estimate that the potential target population for cardiac imaging could be 134 million people per year if all asymptomatic people 20 years old and over were scanned, 70.8 million people per year if routine screening were limited to those 40 and over, and 11.7 million people per year if scans were restricted to people with suspected or established coronary heart disease.

The study looked at these direct nonlabor costs (equipment, maintenance, radionuclides, etc.), direct labor costs (personnel needs, training, support staff), and indirect costs (overhead) to estimate the financial costs of cardiac scanning services. The authors estimate the annual fixed costs of a model radionuclide laboratory to be $112,300 for the complete service, with the costs of the various individual procedures ranging from $258 to $72 (there are nine different types of procedures and two different types of radionuclide testing materials involved in the range of procedures available). Significant variations exist across the country regarding the charges for the various procedures. Nomenclature and billing procedures/listings are not comparable from hospital to hospital. As a result, it is extremely difficult to determine if there is a relative standard or range of charges for these techniques. The authors developed a set of suggested fee schedules for these procedures that range from $405 to $155 per scan.

The medical literature is examined to determine if there is a proper role for scanning techniques. The authors examine extant studies to determine what types of sample populations have been used, the reference or control groups used, the technical and medical standards against which radionuclide procedures were judged, and the clinical settings in which the studies were conducted. In addition, the authors examine the risks associated with these procedures—both to the health care professionals and the patients—and assess the value of the diagnostic information that the scans provide to the diagnosis or the understanding of the extent of the disease and its response to treatment.

The authors fit the many variables into a cost-effectiveness framework to conduct a limited analysis of cardiac imaging procedures. No discount rate is employed (the benefit, costs, and risks occur in the present), nor is a sensi-
tivity analysis performed. The conclusion is that “decision strategies based on threshold cutoff probabilities of a given disease(s) are cost effective compared to blanket testing . . . and that use of cardiac imaging appears to identify additional surgical candidates at reasonable cost when compared to exercise tolerance testing.” The reasonableness of these additional costs will depend, to a large extent, on the incremental health benefits achieved by coronary artery surgery.

The authors identify many of the policy issues raised by this emerging technology. A few of the areas they discuss are issues of reimbursement, safety and efficacy determination, disposal of the radionuclide wastes, clinical standards and indications for use, allocation of resources, and responsibility for regulation and diffusion of these procedures throughout the medical community.


CEA is applied to the management of essential hypertension to “determine how resources can be used most efficiently within programs to treat hypertension and to provide a yardstick for comparison with alternative health-related uses of the resources.” Costs of treatment consist of the lifetime costs of hypertension treatment, costs of treating diseases that occur during additional years of life gained by antihypertensive treatment, minus the costs that would have been incurred for the treatment of cardiovascular morbid events if treatment had not been given. Effectiveness is calculated in terms of increased years of life expectancy from blood-pressure control, adjusted for changes in the quality of life due to the prevention of morbid events and to the side effects of medication. The analysis is performed under three alternative assumptions concerning the proportional reduction of risk of cardiovascular events and death associated with the reduction of blood pressure due to treatment: 1) full benefit, 2) half benefit, and 3) age-varying partial benefit.

One year of life with side effects is taken to be the equivalent of 0.99 quality-adjusted life years. A 5-percent discount rate is used throughout the analysis. Sensitivity analysis is performed on several critical variables, including the discount rate, medical treatment costs, and the quality-of-life adjustment. In addition, the effects of incomplete adherence to the treatment regimen are examined.

The results of the analysis indicate that in no case does treatment pay for itself. At best, only 22 percent of gross treatment costs, on average, can be recovered from savings in the treatment of strokes and heart attacks. However, the analysis also indicates that, in terms of effectiveness, funds spent to improve adherence may be a better use of resources than efforts to screen a maximum number of subjects.

**Steiner, K., and Smith, H., “Application of Cost Benefit Analysis to a PKU Screening Program,” Inquiry 10:34, December 1973.**

The authors compare and contrast the techniques of CBA and CEA, stating that although equally sound decisions may be reached by either method, one of the two is usually better suited for a particular problem. The authors believe that CBA is the best approach for screening programs, and it is this technique that they subsequently use in evaluating a PKU screening program in Mississippi. (PKU is a hereditary condition which causes mental retardation if not detected and treated with a dietary regimen early in life.)

The costs associated with PKU are classified as direct and indirect. Direct costs are defined in this study as the actual expenditures for medical and other services attributable to the disease. Indirect costs are defined as a loss of economic productivity attributable to the disease. These costs serve to measure the benefits of a successful prevention program. The analysis is performed from both a retrospective and a prospective point of view. The retrospective approach measures the costs of the current population with PKU and estimates what the costs of screening, detecting, and treatment would have been. For this study, the direct costs associated with PKU are estimated using data from three mental institutions in Mississippi. Indirect costs are measured by the loss of income, under the assumptions that the PKU victim remains incapacitated for life. Detection costs are based on estimates of the incidence of PKU, 70:1. The retrospective analysis indicates that the total costs of institutionalization and lost earnings associated with the current Mississippi population with PKU (25 patients) amount to $2,314,595. The costs of detecting and treating the 25 patients are estimated at $1,392,668, yielding a cost-to-benefit ratio of 1 to 1.66.

The prospective method calculates the cost of screening all live births in a given year to detect those found to be suffering from PKU. In this study, these calculations are based on the 1967 live births in Mississippi. Testing the 46,714 live births that year would have detected an average of 1.76 PKU cases. The costs associated with these cases amount to $135,062, if the minimum expected length of institutionalization (30 years) is assumed, or $256,418, if institutionalization is assumed to cover the normal life expectancy of a 1-year-old child born in 1967 (70.8 years). Program costs are estimated at $98,518, yielding cost-benefit ratios of 1 to 1.37 and 1 to 2.6, respectively. The authors state that in all calculations, the detection costs are high and the total illness costs (i.e., possible benefit) are low in order to produce conservative results. A discount rate of 4 percent is applied to the lost earnings data, but not to direct or detection costs. Rather than varying the length of institutionalization in calculating the prospective cost-benefit ratios, the authors do not perform sensitivity analysis.


This article examines the costs and benefits of a Massachusetts program designed to detect inborn errors of metabolism and transport in newborn infants. The costs, based on a survey of all hospitals with obstetric and newborn units in Massachusetts, include those for routine specimen collection, laboratory analysis, the collection of additional specimens, confirmatory testing, and followup care.
and therapy. For fiscal year 1972-73, these costs amounted to $460,638. Benefits are calculated as the estimated savings from the prevention of mental retardation and other complications. For 1972-73, estimated total savings amounted to $825,300, yielding a net benefit of $364,662 or a benefit-cost ratio of nearly 1.8. Indirect costs of metabolic disorders (such as reduced economic productivity due to disability and premature mortality), which would also be averted as a result of a screening program, are not included in the calculation of benefits. Presumably, the inclusion of the present value of such benefits, when considered along with a similar future stream of the other costs and benefits (also discounted to present value), would result in even higher net benefits.


This study examines the appropriate methodology of CEA/CBA for diagnostic procedures. Following the development of a framework for analysis, the author reviews the literature of the cost effectiveness of CT scanning, critically evaluating it in terms of the evaluation model.

The author describes a theoretical “ideal” evaluative model in which the analysis compares alternative diagnostic pathways, each of which begins with the presentations of signs and symptoms and ends with patient outcomes. The purpose of the evaluation is not to examine the technology per se, but rather to evaluate its appropriate use. The author describes the need for an appropriate means to 1) identify homogeneous patient groups, 2) specify diagnostic pathways, 3) measure diagnostic accuracy, 4) measure diagnostic and therapeutic costs, and 5) specify outcomes of the diagnostic and therapeutic process.

In a review of the literature on the economic impact of CT scanning, only one study that attempted to specify diagnostic pathways was identified. Most of the other studies examined the impact CT has on diagnostic costs or examined the cost of case finding.

Efficacy information is addressed both for diagnostic studies in general and for CT scanners in particular. Comments regarding the potential benefits associated with negative findings are also included.

Costs are distinguished from charges; marginal, or avoidable, costs are recommended; the difficulty of capturing true costs is discussed extensively. Indirect costs are not considered. Discounting was not specifically discussed, except within the context of the reviewed case studies; where, in one, future benefits were discounted. Equity issues were not addressed.

Despite major limitations in applying principles of economic evaluation to diagnostic procedures, such evaluations are feasible. For CT scanning, when sufficient demand exists to operate a scanner at full capacity, some specific uses appear to be cost effective.


This paper presents a state-of-the-art assessment of CBA and CEA of medical procedures. CBA/CEAs are defined and distinguished from each other. The author advocates the use of a multiattribute accounting framework, in conjunction with CEA and CBA, in which unquantifiable concerns, such as equity and ethical issues, are considered along with the traditional, measurable impacts. The basic methodological principles are reviewed, including estimation of event rates, sensitivity analysis, choosing a discount rate, measurement of costs, and measurement of benefits. The controversy surrounding the assignment of monetary value to life saving and health improvement in CBA is discussed.

A review of selected applications, classified as treatment, secondary prevention, screening, and immunization, is presented. The author states that diagnostic procedures other than screening have not received much attention, in part because of methodologic obstacles. He predicts that technology evaluation will be the area where the next major advances in CEA and CBA will develop. He then discusses current methodologic problems, classified as 1) the valuation of multiattributed outcomes, 2) the evaluation of diagnostic tests, 3) the evaluation of multifaceted technologies, and 4) uncertainty concerning efficacy, costs, and ultimate uses of evolving technology.

The paper concludes with a generally optimistic assessment of the prospect for CEA and CBA in medical care and for overcoming the current methodological problems. The author recommends a multidisciplinary approach to analysis, including the expertise of physicians, engineers, and economists. He notes that the value of formal economic analysis lies not so much in the actual results, but rather in the ability of such analysis to highlight uncertainty and the most important value tradeoffs involved in alternative policies.


This study illustrates the possible techniques for evaluating the cost effectiveness of automated multichannel chemistry analyzers. The authors also examine and discuss the limitations due to data deficiencies, areas for future research, and influences of clinical practice on the evaluation of such analyzers.

The case study briefly reviews the history of the multichannel clinical chemistry technology and presents an analytical framework for evaluating the cost effectiveness of the multichannel analyzer. The authors review the available data concerning the costs of multichannel chemistry

This CBA of poliomyelitis research uses and expands on the benefit calculations first presented in Weisbrod’s The Economics of Public Health (578). These calculations comprise savings from avoided premature mortality, morbidity, and treatment and rehabilitation costs. The analysis requires an estimation of 1) the time stream of research expenditures directed toward poliomyelitis, 2) the time streams of a number of forms of benefits resulting from (or predicted to result from) the application of the knowledge generated by the research, and 3) the cost of applying that knowledge.

Using this information, Weisbrod calculates internal rates of return on research expenditure. Savings per case prevented, application costs, the time horizon, and research expenditures are all subjected to sensitivity analysis. The internal rates of return were found to be sensitive to application costs, varying from 4 to 14 percent. In approximating present value of expenditures and benefits, Weisbrod uses a discount rate of 10 percent. No sensitivity analysis is performed on this variable. The difficulties encountered in trying to associate specific medical research expenditures with a particular disease are discussed. These include the fact that basic research is often not directed at a specific disease problem and even disease-specific research frequently yields knowledge relevant to the prevention or treatment of other diseases. The data used here are estimates of awards for poliomyelitis research from 1930 to 1956. Weisbrod stresses the need to include the costs in-
volved in the application of new medical knowledge, as well as the costs of generating it, when attempting to comprehensively analyze a medical research program.

The article concludes with an interesting discussion of the impact on private market allocative efficiency when a collective consumption good (e.g., medical research) requires for its application a procedure such as vaccination which is provided individually and from which nonpayers may be excluded. Weisbrod also discusses the effects of externalities on the provision of medical research and its application for contagious diseases. The author concludes that when collective consumption goods require use of individual consumption goods for their application, and where these individual goods produce real external economies, neither the nature nor the extent of private market inefficiency is clear.


For the 10-year period 1963-72, the authors estimate the costs the Nation would have sustained without measles immunization (i.e., the benefits of measles immunization) and the actual costs of measles during that period in terms of illness and associated resources consumed. The research costs of developing and testing the measles vaccine are not included because of the difficulty in identifying them and in determining the share applicable to the United States in the period under consideration.

The benefits associated with the measles immunization program considered in this analysis include 1) savings in medical care costs for services of physicians and for long-term institutional care for those who would have become retarded, and 2) avoidance of production losses due to morbidity and premature mortality. Program costs are those incurred in vaccine production, distribution, administration, and promotion. The analysis concludes that the net benefits achieved through immunization in the United States totaled $1.3 billion for the period 1963-72. A single discount rate of 4 percent is used. The authors assume that the national immunization effort had no significant effect on the demand for medical care or on the size and composition of the labor force.
Appendix D.— Description of Other Volumes of the Assessment

The overall OTA assessment, The Implications of Cost-Effectiveness Analysis of Medical Technology, consists of a main, policy-oriented report plus five background papers. The present volume, Methodological Issues and Literature Review, is one of the background papers. The main report and the other background efforts are briefly described below.

The main report examines three major issues: 1) the general usefulness of CEA/CBA in decision-making regarding medical technology, 2) the methodological strengths and shortcomings of the technique, and 3) the potential for initiating or expanding the use of CEA/CBA in six health care programs (reimbursement coverage, health planning, market approval for drugs and medical devices, Professional Standards Review Organizations, R&D activities, and health maintenance organizations), and most importantly, the implications of any expanded use.

The prime focus of the report is on the application of CEA/CBA to medical technology (i.e., the drugs, devices, and medical and surgical procedures used in medical care, and the organizational and support systems within which such care is provided). With the exception of a background paper on psychotherapy, the report does not address psychosocial medicine, Other aspects of health, such as the environment, are not directly covered either. The findings of the assessment, though, might very well apply to health care resource decisionmaking in general, and with modification, to other policy areas such as education, the environment, and occupational safety and health.

The main report contains chapters on methodology, general decisionmaking, each of the six health programs mentioned above, and the general usefulness of CEA/CBA. It contains appendixes covering a survey of current and past uses of CEA/CBA by agencies (primarily Federal), a survey of the resource costs involved in conducting CEA/CBAs, a discussion of ethical issues and CEA/CBA, and a brief discussion of legal issues.

In order to help examine the applicability of techniques to assess the costs and benefits of medical technology, 19 case studies were prepared. All 19 are available individually. In addition, 17 of the cases are available collectively in a volume entitled Background Paper #2: Case Studies of Medical Technologies. Some of the cases represent formal CEAs (e.g., the case on certain respiratory therapies). Other cases illustrate various issues such as the difficulty of conducting CEA in the absence of adequate efficacy and safety information (e.g., the case on breast cancer surgery), or the role and impact of formal analysis on policymaking (e.g., the case on end-stage renal disease interventions). The 17 case studies in Background Paper #2 and their authors are:

Artificial Heart
  Deborah P. Lubeck
  John P. Bunker

Automated Multichannel Chemistry Analyzers
  Milton C. Weinstein
  Laurie A. Pearlman

Bone Marrow Transplants
  Stuart O. Schweitzer
  C. C. Scalzi

Breast Cancer Surgery
  Karen Schachter
  Duncan Neuhauser

Cardiac Radionuclide Imaging
  William B. Stason
  Eric Fortess

Cervical Cancer Screening
  Bryan R. Luce

Cimetidine and Peptic Ulcer Disease
  Harvey V. Fineberg
  Laurie A. Pearlman

Colon Cancer Screening
  David M. Eddy

CT Scanning
  Judith L. Wagner

Elective Hysterectomy
  Carol Korenbrot
  Ann B’. Flood
  Michael Higgins
  Noralou Roos
  John P. Bunker

End-Stage Renal Disease Interventions
  Richard A. Rettig

Gastrointestinal Endoscopy
  Jonathan A. Showstack
  Steven A. Schroeder

Neonatal Intensive Care
  Peter Budetti
  Peggy McManus

Natal Guideline Review
  Nancy Barrand
  Lu Ann Heinen

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Nurse Practitioners
Lauren LeRoy
Orthopedic Joint Prosthetic Implants
Judith D. Bentkover
Philip G. Drew
Periodontal Disease Interventions
Richard M. Scheffler
Sheldon Rovin
Respiratory Therapy
Richard M. Scheffler
Morgan Delaney

The 18th case study is published separately as Background Paper #3: The Efficacy and Cost-Effectiveness of Psychotherapy. That study assesses methodological and substantive issues relating to the scope of psychotherapy, the evaluation of psychotherapeutic efficacy, and the applicability of CEA/CBA in assessing psychotherapy. It was prepared by Leonard Saxe on the basis of a report prepared for OTA by Brian Yates and Frederick Newman. The 19th case study was prepared by Judith Wagner and is published separately as Background Paper #5: Assessment of Four Common X-Ray Procedures.

Background Paper #4: The Management of Health Care Technology in Ten Countries is an analysis of the policies, programs, and methods, including cost-effectiveness and cost-benefit techniques, that nine industrialized nations other than the United States use to manage the effects of medical technology. The experience of these nine countries in managing medical technology is compared to that of the United States. The paper on the United States and the comparative analysis were prepared by OTA staff, assisted by Louise Russell. The authors of the papers on the nine foreign countries are:

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Australia
   Sydney Sax
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   Joel Broida
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   Rebecca Fuhrer
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A related report prepared by OTA and reviewed by the Advisory Panel to the overall assessment is A Review of Selected Federal Vaccine and Immunization Policies. That study, published in September of 1979, examined vaccine research, development, and production; vaccine efficacy, safety, and cost-effectiveness; liability issues; and factors affecting the use of vaccines. Pneumococcal vaccine was used as a case study, and a CEA/CBA was performed.
Appendix E.—Health Program Advisory Committee, Authors of Case Studies of Medical Technologies (Background Papers #2, #3, and #5)

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