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Methodological Finding; and Principles

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4. Methodological Findings and Principles

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 curtailment 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.

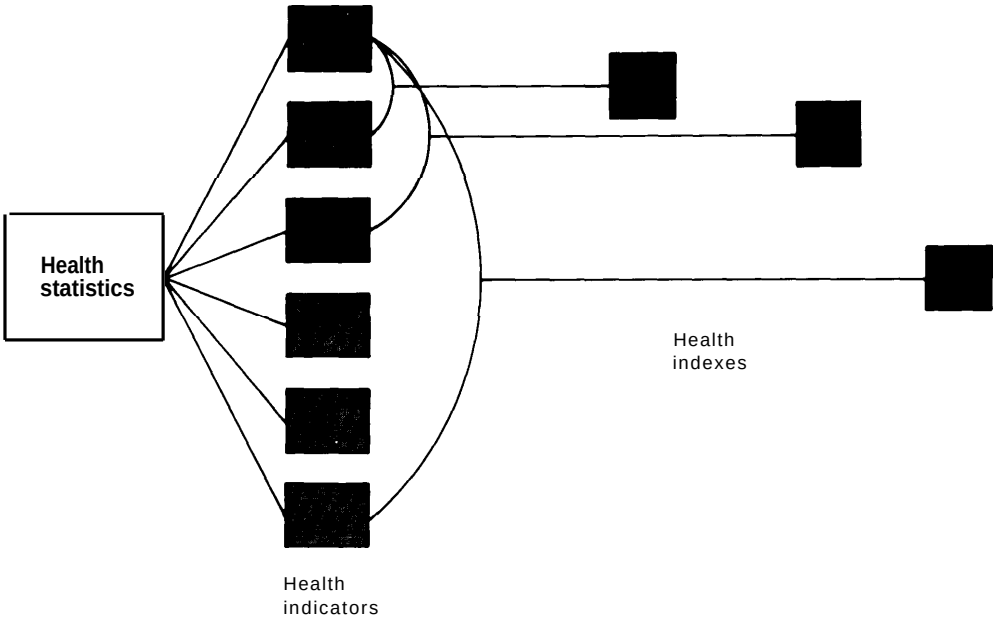
The National Center for Health Statistics (NCHS) is **an agency** within the Office of the Assistant Secretary for Health of the Department of Health and Human Services and is one of the principal health services research agencies of the Federal Government. NCHS has played a major role in the development 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 CEAs. Currently, NCHS staff are coordinating an interagency Public Health Service Cost of Illness Committee which is examining the state-of-the-art of estimating costs of illness and disability.

There is also an expanding literature related to health status measurement. This maturing field is important to health care technology assessment in general and CEA/CBA in particular. It may have the potential to capture, in a very useful format, aggregate measures of, and changes in, health status which are much more inclusive than single health statistics or health indicators, such as mortality rates or disability rates. The relationship between health statistics, health indicators, and health indexes has been characterized by Murnaghan *(738)* and is shown in figure 3. Health statistics can be thought of as basic data such as number of hospital admissions. Health indicators are processed data that aggregate information of similar type and are often expressed in terms of percentages, rates, and ratios. The main methodological issue in regard to health indicators is reliability, not validity (738). Health status indexes (HSIs), however, are considerably more complex and controversial. In general, such indexes attempt to combine multiple indicators such as disability and death into a single expression. Usually an index scale is used (e.g., a scale from zero to 10, where zero represents death and 10 represents perfect health). The methodological issues in regard to health indexes are reliability (i.e., do repeated measurements provide the same information?), validity (i. e., is the relative weighting system correct?), and definitional consistency (i.e., what constitutes "health''?).

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-

Figure 3.—The Relationship Between Health Statistics, Health Indicators, and Health Indexes



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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.
4. Analyze benefits/effectiveness.
5. Analyze costs.
6. Differentiate perspective of analysis.
7. Perform discounting.
8. Analyze uncertainties.
9. Address ethical issues.
10. Interpret results.

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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 *re*-sulting 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-

duced in the professional literature in abstract form. Results of CEA/CBA often have the potential to mislead the reader, a hazard which can be greatly reduced by proper interpretation.

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 programing 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-

[1]See *Background Paper #2: Case Studies of Medical Technologies, Background Paper #3: The Efficacy and Cost Effectiveness of Psychotherapy,* and *Background Paper #5: Assessment of Four Common X-Ray Procedures,* prepared by OTA in conjunction with this assessment.

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 formal CEA/CBAs, however valid and effective potentially, can be inappropriately used by decisionmakers who lack the necessary resources, **skills, or understanding of the inherent limita**tions. Defining a more practical, limited appreach to the methodology seems clearly appropriate 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 calculations should be investigated and considered.* Rather than aggregating, analysts might explicitly list or *ARRAY* all the elements which are included in, or would be affected by, decisions. When costs and effectiveness could be quantified, that would be done; when they could be combined, that would also be done. *Whenever one or more important nonquantifiable variables would otherwise either be left out or be relegated 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 decisionmakers commensurate with the factors' significance. The arraying method can either be highly quantitative and analytical, using multiobjective programing techniques, or when that is not desirable or possible, it can be presented more qualitatively.