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INTRODUCTION

OTA found no consensus among analysts and practitioners as to a standard set of methods for cost-effective analysis/cost-benefit analysis (CEA/CBA). Although there is still some disagreement as to which variables should be considered and how these variables should be treated—problems which may be lessened as the state-of-the-art develops—there is agreement that at present 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 methods is due more to the inherent nature of the analysis, the nature and stage of development of the technology being analyzed, and the general social and political environment of decisionmaking than to the immaturity of CEA/CBA methods. 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 did find full agreement on the paucity of, and consequently the need for, improved data, without which good analyses are impossible. Efficacy and safety information for many technologies is generally not available. Health care utilization data are often either not available or not in standard format or accessible form, and cost data are often inaccurate and also nonstandardized. Better routine data collection, although desirable and possibly necessary, would probably not be sufficient for better analyses, however, because each specific analysis often requires a unique data set that will not be available in even the best of routine data collection systems. Consequently, an optimum mix of routine data collection and study-specific data collection needs to be defined, and when specific studies are funded, monies for necessary data collection should be included.

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 type can be expected to diminish as more experience accumulates. The 10 principles for analysis presented later in this chapter are directly relevant to lessening this type of shortcoming, which will be called "weaknesses due to immaturity." Limitations of the first type, those that are inherent, however, are likely to remain significant barriers to advances in the usefulness of CEA/CBA in health care.

Weaknesses Inherent in CEA/CBA

Inherent weaknesses of the methodology of CEA/CBA,[1] especially in its more formal or sophisticated forms, may prove to be of greater long-run consequence than those of immaturity (617).

Examples of such weaknesses are the difficulty of predicting with precision the costs and benefits of new or not yet existing programs or technologies, fundamental problems in quantifying or valuing certain important but less tan-

[1]Inherent methodological weaknesses of CEA/CBA are discussed in detail in *Background Paper #1: Methodological Issues and Literature Review*, prepared by OTA in conjunction with this assessment.

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gible health benefits, controversy over the appropriate discount rate, the inability of analysis to adequately incorporate equity and political considerations, and the inevitability of significant sensitivities or uncertainties even in many perfectly managed studies. The rapidity and profundity of technical change in medicine exacerbate analytical difficulties, a problem that is 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 evaluation uncertainties are most dramatic. Sensitivity analysis sometimes can demonstrate that inherent technical analytical problems do not affect qualitative conclusions, but frequently these difficulties preclude a definitive assessment of the desirability of competing programs. Ultimately, research may resolve some currently intractable problems, but for the foreseeable future, most such limitations seem likely to remain inherent barriers to the direct application of the findings of many CEA/CBAs to policy decisionmaking. In particular, the uncertainties which pervade analysis severely restrict the potential of a study, however high quality, to resolve definitively the "close calls" in which alternative programs are similar in both cost and effectiveness.

Weaknesses Due to Immaturity

As noted in the previous chapter, there are relatively few examples of technically high-quality CEA/CBA studies in the health literature. As the state-of-the-art of CEA/CBA matures and as analysts and decisionmakers gain more experience with CEA/CBA in health care, however, there may be a reduction in the number of problems such as inappropriate or inaccurate specification of production relationships; [z] inadequate identification, measurement, or valuation of costs or benefits; lack of discounting of future costs and benefits; and failure to examine sensitivities. Though one should never downplay the difficulty of producing a technically high-quality study, in principle problems such as these can be resolved; clearly the practice of analysis can and should improve over time. Thus, the current restriction on the usefulness of CEA/CBA caused by weaknesses of immaturity seems likely to recede in importance over time. The 10 principles of analysis presented below are suggested as one method of minimizing these weaknesses.

[z] *See Background Paper #1: Methodological Issues and Literature Review.*

TEN PRINCIPLES OF CEA/CBA METHODOLOGY

There is widespread agreement that the 10 basic principles below are generally applicable to CEA/CBA analysis, (See table 1.)

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, may 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. For instance, "excess deaths" could lead to comparing any preventive or therapeutic program that decreases mortality, whereas "excess deaths due to cancer" would limit the scope of study considerably, and "excess deaths due to cervical cancer" would limit it even 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 or improve health status. Some studies, however, must necessarily focus on the efficient use of a technology. This is particularly true of studies 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 ob-

Table 1.—Ten General Principles of Analysis
(for CEA/CBA methodology)

1. Define problem.
2. State objectives.
3. Identify alternatives.
4. Analyze benefits/effects.
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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jectives 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 lower these deaths 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/Effects

All foreseeable benefits/effects should be identified and when possible should be measured. The relevant effects 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 or effects 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/effects, such as alleviated pain, reduced risk of sickness or death, enhanced quality of life, lowered anxiety, 2) health resource benefits/effects such as increases and decreases in health care expenditures, 3) other economic benefits/effects such as increased productivity, and 4) social benefits/effects 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 that 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; the primary private objective (i.e., expected benefits) of the patient, however, 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.

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 commonsense 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 policy of 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 introduced 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 interpretation.

OTHER FINDINGS

In addition to conforming to the aforementioned 10 general 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 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 is 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,* this analytical method could often be too complex, expensive, and time consuming if used as a routine method for decisions by public policymakers. In fact, the cost-effectiveness case studies conducted as part of this assessment serve to highlight the immaturity of the technique itself.[3] 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 methods, the tenuousness (or error) in the key assumptions, 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 sets of indexes that describe the health status of a population at any given time (79). That type of work should continue and perhaps should receive more emphasis. .Nevertheless, formal CEA / CBAs, however potentially valid and effective, can be inappropriately used by decisionmakers who lack the necessary resources and skills. Defining a more practical, limited approach to the methods seems clearly appropriate and does not diminish the worth of or need for more sophisticated approaches under different circumstances.

[3]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.

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. Instead of aggregating, analysis might be done by explicitly listing or **ARRAYING all the elements that are included** in, or would be affected by, decisions.

When costs and effects can be quantified, that would be done; when they can 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 help force consideration of **these factors by decisionmakers commensurate** with the factors' significance.

A more detailed examination of this arraying possibility, along with a discussion of circumstances leading to OTA's suggesting it, is found in *Background Paper #l: Methodological Issues and Literature Review.*