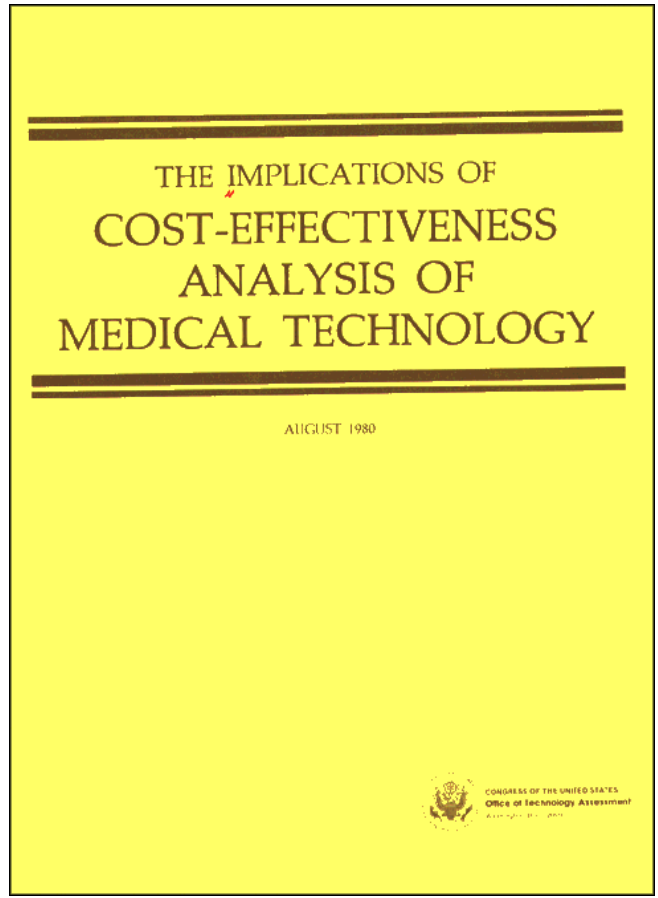
The Implications of Cost-Effectiveness Analysis of Medical Technology

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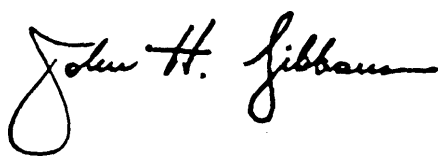
Foreword

In this volume we present the major, policy-oriented report of OTA'S assessment, The *Implications of Cost-Effectiveness Analysis* of Medical Technology. Begun in October 1978, the assessment analyzes the feasibility, implications, and usefulness of cost-effectiveness and cost-benefit analysis in health care decisionmaking.

In addition to this main report, there will be five background papers: 1) one covering methodology and literature issues, 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 study was published in September of 1979: *A Review of Selected Federal Vaccine and Immunization Policies.*

The main report, *The Implications* of *Cost-Effectiveness Analysis* of *Medical Technology,* was prepared by OTA staff. In preparing this report, OTA consulted with members of the advisory panel for the 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 the final report 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 (see Acknowledgments in app. H). We are grateful for their assistance.



JOHN H. GIBBONS
Director

Advisory Panel on The Implications of Cost-Effectiveness Analysis of Medical Technology

John R. Hogness, *Panel Chairman*
President, Association of *Academic Health Centers*

Stuart H. Altman
Dean
Florence *Heller School*
Brandeis University

James L. Bennington
Chairman
Department of Anatomic Pathology and Clinical Laboratories
Children's Hospital of San Francisco

John D. Chase
Associate Dean for Clinical Affairs
University of *Washington School* of Medicine

Joseph Fletcher
Visiting Scholar
Medical *Ethics*
School of *Medicine*
University of *Virginia*

Clark C. Havighurst
Professor of Law
School of *Law*
Duke University

Sheldon Leonard
Manager
Regulatory Affairs
General Electric Co

Barbara J. McNeil
Department of *Radiology*
Peter Bent Brigham Hospital

Robert H. Moser
Executive Vice President
American College of Physicians

Frederick Mosteller
Chairman
Department of Biostatistics
Harvard University

Robert M. Sigmond
Advisor on Hospital Affairs
Blue Cross and Blue Shield Associations

Jane Sisk Willems
VA Scholar
Veterans Administration

OTA Cost= Effectiveness Project Staff

Joyce C. Lashof, *Assistant Director, OTA*
Health and Life Sciences Division

H. David Banta, *Program Manager*

Clyde J. Behney, *Project Director*

Bryan R. Luce, *Senior Analyst*
Lawrence Miike, *Senior Analyst*
Michael A. Riddiough, *Senior Analyst*
Barbara Greenberg, *Senior Analyst*
Karen J. Kohlhof, *Research Associate*
Chester Strobel, *Research Associate*
Leonard Saxe, *Congressional Fellow*
Kerry Britten Kemp, *Editor and Writer*
Virginia Cwalina, *Administrative Assistant*
Shirley Ann Gayheart, *Secretary*
Nancy L. Kenney, *Secretary*

Contractors

Kenneth E. Warner, University of Michigan The Hastings Center
Lewin and Associates Robert A. Derzon

OTA Publishing Staff

John C. Holmes, *Publishing Officer*

Kathie S. Boss Debra M. Datcher Joanne Mattingly

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Summary and Policy Option;

Nature never gives anything to anyone; everything is sold. lt is only in the abstractions of *ideals that choice comes without consequences.*

Ralph Waldo Emerson

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1
Summary and Policy Option;

The rapid and continuing growth of expenditures is a central issue in many policy decisions concerning the medical care system of the United States. Policymakers, health professionals, and consumers are seeking ways to control this growth while simultaneously improving the quality of health care. Increasingly, the use of cost-effectiveness analysis/cost-benefit analysis (CEA/CBA) is being advocated as a possible means of making the medical care system more efficient. In particular, this technique is suggested for use in health care programs—for example, by the medicare program in its reimbursement coverage decisions. Nevertheless, a great deal of confusion and disagreement surrounds the implications and feasibility of applying CEA/CBA in health care. To aid in their decisions concerning the possible use of CEA/CBA in Federal health programs, the Senate Committees on Labor and Human Resources and on Finance asked OTA to explore the applicability of CEA/CBA to medical technology.

In the assessment, three major issues are examined: 1) the general value of CEA/CBA in decisionmaking about the use of 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, the Professional Standards Review Organizations (PSROS), health planning, market approval for drugs and medical devices, R&D activities, and health maintenance organizations (HMOs)—and, most importantly, the implications of any expanded use.

The prime focus of the assessment is on the application of CEA/CBA to medical technology, i.e., the drugs, devices, medical and surgical procedures used in medical care, and the organizational and support systems within which such care is provided. Except in a background paper on psychotherapy, the report does not address psychosocial medicine. Other factors influencing health, such as the environment, are not directly covered either. The findings of this 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.

This **OTA assessment finds that CEA/CBA cannot serve as the sole or primary determinant of a health care decision. Decisionmaking could be improved, however, by the process of identifying and considering all the relevant costs and benefits of a decision. At present, using the approach or *process* of CEA/CBA in decisionmaking may be more helpful than the rigid and formal application of CEA/CBA study results in health care program decisions. It is unrealistic, moreover, to expect that CEA/CBA, in itself, would be an effective tool for reducing or controlling overall expenditures for medical care.**

EVALUATING COSTS AND BENEFITS

All decisions have consequences. Usually, however, in most decisionmaking processes only a fraction of all potential consequences is taken into account. The inherent complexities and uncertainties associated with many decisions make it extremely difficult to identify and weigh all possible consequences. In general, however, the quality or validity of decisions can be increased by analysis that forces a structuring of the decision process—that provides a consistent framework for identifying and considering as many of the relevant costs and benefits as is feasible.

The public, or governmental, sector is called upon to make certain decisions that are imprac-

tical for the private sector to make. Examples of these are decisions concerning national defense or air pollution control, neither of which is amenable to being traded in the marketplace. Other decisions are made by the public sector for social reasons such as assuring equitable distribution of what may be considered essential goods (e.g., health care for the elderly). Because conventional private sector techniques, such as capital budgeting and return-on-investment analysis, are insufficient for these decisions, techniques such as CBA and CEA have been developed. In the medical care area, CEA/CBA is designed to integrate the economic aspects of a decision with the health aspects of that decision. Consequently, **it should not be considered simply an economic tool.**

There are two basic types of health care resource allocation decisions which *in theory* could benefit from a CEA/CBA process. The first are decisions made within **a** fixed or prospectively set budget, such as those made by HMOS. The second are decisions made in the absence of a direct budget constraint, such as those made for medicare reimbursement or in health planning.

In the former—allocation decisions made within a budget—tradeoffs must be made, since not all projects can be funded. The projects that promise to deliver more benefits for the cost should be more attractive than those projects expected to deliver fewer benefits. In these decisions, the function of CEA/CBA would be to illuminate the decision process and to require that implicit judgments be made more explicitly, thus forcing external examination of the assumptions and values placed on decision variables.

In nonconstrained decisions, direct tradeoffs between competing projects often are unnecessary. Consequently, a function of CEA/CBA in these decisions would be to force consideration of economic factors. In health planning decisions, for example, planners would be asked to consider not only whether a service is needed but also whether it is worth the cost.

COST= BENEFIT ANALYSIS AND COST-EFFECTIVENESS ANALYSIS

The terms CBA and CEA refer to formal analytical techniques for comparing the positive and negative consequences of alternative ways to allocate resources. In practice, the comparison of costs and benefits is accomplished through various analytical approaches, which comprise a spectrum ranging from sophisticated computer-based analysis 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. At one end of the continuum are what will be referred to as "net cost" studies. In these studies the emphasis is on costs, and net cost 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 the technologies under study to specific health-related outcomes and to compare the costs of the technologies to the differences in health benefits. CBA and CEA comprise the entire set of analytical techniques—differentiated by the specifics of what costs and benefits are considered and how they are analyzed—on this continuum.

The principal distinctions between CEA and CBA lie in the valuation of the desirable consequences of a decision, in the implications of the different methods of that valuation, and usually in the scope of the analysis. In CBA all costs and all benefits are valued in monetary terms. Thus, conceptually, CBA can be used to evaluate the "worth" of a project and would allow comparison of projects of different types (such as dams and hospitals). In CEA, the health-related ef-

fects of programs or technologies are not valued in monetary terms but rather are measured in some other unit (such as years of life gained), A CEA, therefore, does not result in a net monetary value for a project. Instead, it produces a measure of the cost involved in attaining some desirable health-related effect. Conceptually, CEA permits direct comparison of only those programs or technologies that share similar objectives. This OTA assessment uses the term CEA/CBA to refer to both of these techniques because the findings below apply generally to both.

FINDINGS

General

Most of the specific findings presented below relate to the two major, general findings of the OTA assessment:

- Performing an analysis of costs and benefits can be very helpful to decisionmakers because **the process of analysis gives structure to the problem, allows an open consideration of all relevant effects of a decision, and forces the explicit treatment of key assumptions.**
- CEA/CBA exhibits too many methodological and other limitations, **however, to justify relying solely on the results of formal CEA/CBA studies in making a decision. Thus, although CEA/CBA is useful for assisting in** many **decisions, it should not be the sole or prime determinant of a decision.**

CEA/CBA is viewed by different parties as ranging in usefulness from obfuscating the pertinent issues in a decision process at one extreme to illuminating and synthesizing these issues so well that the technique can be used to make decisions at the other extreme. There is, however, a middle position which maintains that the technique could be helpful in structuring information and that this information should be only one of several components of a decision process. The OTA case study on the artificial heart, for example, lays out many of the factors to be considered in decisions regarding continued funding of R&D of this technology. But those decisions will also be dependent on a number of other political and social factors that are difficult to analyze systematically.

Both extreme positions mentioned above pertain to the use of CEA/CBA as a formal, structured analysis which is oriented toward a bottom-line answer, such as a cost-benefit ratio. Such a bottom line, however, often hides many important value judgments, thus providing a seemingly unambiguous answer that may rest on ambiguous data or assumptions.

Advocates of the middle position propose that CEA/CBA be used within the context of accepted principles of analysis in order to illuminate the costs and the benefits of a decision but not necessarily to aggregate and weigh them.

The findings of this and any other assessments of the past usefulness or current potential of CEA/CBA in health care decisionmaking should be kept in perspective: Because there has been little experience with the use of CEA/CBA, these findings are based on very little definite information. Therefore, any of the findings or projections of the usefulness or implications of CEA/CBA in this report could be proven wrong as more experience accumulates. In fact, one of the priorities for future examinations of the role of CEA/CBA in health care should perhaps be small, controlled experiments, or demonstrations, of its potential use (see Option 10 below).

Methodology

There is no set combination of specific analytical elements that form a standard CEA or CBA methodology. A standard or rigid methodology, however, is to be neither expected nor desired. OTA found a wide variation in the forms of CEA/CBA analyses actually conducted to date. Most of the analyses reviewed seem to be academic exercises, infrequently connected to the policy process. In addition, the literature on CEA/CBA indicates that a great many of the

analyses conducted tend to be on the "net cost" end of the CEA/CBA continuum, that is, analyses in which health outcomes were not taken into explicit account. In part, this may be a reflection of the paucity of data on the efficacy, safety, and appropriate use of medical technology.

OTA'S assessment does find, however, general agreement on a set of 10 principles of analysis that could be used to guide the conduct, evaluation, or use of CEA/CBA studies. OTA believes that use of generally accepted principles is important, largely because of the basic methodological limitations of CEA/CBA. For example, although the results of a CEA/CBA are often dependent on the discount rate chosen, there is no general agreement on what discount rate should be used under what circumstances. Furthermore, as primarily an efficiency-oriented technique, CEA/CBA is weak in the areas of equity and other ethical considerations. Finally, CEA/CBA must often address issues of great uncertainty, such as disease progression, patient compliance rates, differing responses to technology by differing population groups, and so on. These and other limitations must be kept in mind and must to the maximum extent feasible be compensated for by techniques such as sensitivity analysis (see **#8** below). The 10 basic principles of analysis are:

1. **Define Problem.**–The problem should be clearly and explicitly defined and the relationship to health outcome or status should be stated.
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.
3. **Identify Alternatives.** —Alternative means (technologies) to accomplish the objectives should be identified and subjected to analysis. When slightly different outcomes are involved, the effect this difference will have on the analysis should be examined.
4. **Analyze Benefits/Effects.—All foreseeable benefits/effects (positive and negative outcomes) should be identified, and** when possible, should be measured. Also, when possible, and if agreement can be reached, it may be helpful to value all benefits in common terms in order to make comparisons easier.
5. **Analyze Costs.—All expected costs should be identified, and** when possible, should be measured and valued in dollars.
6. **Differentiate Perspective of Analysis.—When private or program benefits and costs differ from social benefits and costs (and if a private or program perspective is appropriate for the analysis), the differences should be identified.**
7. **Perform Discounting.—All future costs and benefits should be discounted to their present value.**
8. **Analyze Uncertainties. — Sensitivity analysis should be conducted. Key** variables should be analyzed to determine the importance of their uncertainty to the results of the analysis. A range of possible values for each variable should be examined for effects on results.
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.**
10. **Discuss Results.—**The results of the analysis should be discussed in terms of validity, sensitivity to changes in assumptions, and implications for policy or decisionmaking.

In addition to conforming to these 10 principles, all quantitative analyses should specify data sources and be written as clearly and nontechnically as possible. They should also be subjected to review—including public scrutiny when appropriate—especially regarding assumptions upon which their outcomes may rest.

OTA found that many of the methodological limitations of CEA/CBA are often hidden by the practice of deriving a cost-benefit or cost-effectiveness ratio—that is, a numerical bottom-line. Therefore, **the possibility of not aggregating the often complex sets of calculations should be investigated.** Instead of aggregating, analyses

might be done by explicitly listing or ARRAYING all the elements which are included in or would be affected by a decision. Where costs and effects could be quantified, that would be done; where they could be combined, that would be done, But no effort would be made to arrive at a single combined value when one or more important nonquantifiable variables that would have to be left out or relegated to a footnote. Such an array method of analysis would mean that decisionmakers would have a greater number of elements to consider, but it would also mean that intangible or nonquantifiable factors would be more explicit and, thus, more likely to be taken into consideration.

The findings of this assessment, especially methodological ones, focus primarily on the practical implications of CEA/CBA for health policy. The report is not written for the academic research community. The general principles above apply to analysis for policy use. In no way, however, should the findings be taken to mean that complex, sophisticated analysis is always unnecessary or superfluous. Advancement of the state-of-the-art of CEA/CBA requires ongoing and sophisticated research. Current research on the development and validation of sets of indexes of the health status of a population, for example, appears very valuable. Use of such complex aspects of CEA/CBA, however, may require greater technical capabilities than most health programs currently possess. Addressing a more limited approach to analysis, then, seems appropriate for the goal of this assessment. But it does not diminish the need for more complex approaches in research or other specialized circumstances.

Applying CENCBA in Health Care

As stated above, CEA/CBA can be more valuable to health care decisionmaking when it serves as a problem structuring process than when it becomes the primary factor, with numerical results, of a decision. **Furthermore, CEA/CBA potentially can be more valuable for decisionmaking under a constrained budget, when tradeoffs have to be made directly, than when constraints are nonexistent or very indirect. In neither case, however, would CEA/CBA necessarily function as an effective cost-constraining mechanism or tool.** Under the budget system, the budget itself would be the constraining mechanism. Under the nonconstrained system, since no direct tradeoffs are required, no direct limit on expenditures is set. CEA/CBA might, however, change the mix of expenditures. Especially under a budget system, technologies might be substituted for one another as a result of analysis.

The context within which a decision will take place must be specified before any judgment of the usefulness or applicability of CEA/CBA can be made. For example, does the decision relate to a technology at an early stage in its lifecycle such as bone marrow transplants? Or does it concern an established technology, such as cervical cancer screening? Is the technology in question a diagnostic technology, such as the CT scanner, or a therapeutic one, such as renal dialysis? The possibility of affecting the course of a technology's diffusion and use might be greater in early stages of its development, but the uncertainties about its health effects and its costs will generally be greater. Thus, it may be possible to do a more valid or certain CEA/CBA later in the technology's lifecycle, but the information gained may be less valuable for public policy. The tradeoff required will vary depending on the specifics of the technology and the policy decision to be made. Similarly, diagnostic technologies are often more difficult to study than other types because of the uncertainties involved in linking their use to health outcomes. Thus, studies of diagnostic technologies often tend toward the "net cost" end of the CEA/CBA spectrum, where the measures of outcome or benefit may be numbers of tests performed or levels of diagnostic accuracy.

Applying CENCBA in Specific Health Care Programs

OTA examined the current and potential use of CEA/CBA or related techniques in six health care programs (see chs. 5 through 10). Although informal and often implicit analysis of costs and benefits frequently takes place in health policy formulation, OTA found very little formal use

of CEA/CBA in the programs studied. In several of the areas, cost itself has played little or no role in policy decisions.

Reimbursement programs such as medicare and Blue Cross/Blue Shield, when deciding what technologies will be covered, concentrate on criteria that generally do not explicitly include costs (e.g., stage of development of the technology and acceptance by the medical community). Under medicare, initial responsibility for identifying questions about whether a technology should be covered lies with a system of local contractors who administer the program. When not resolved at the local level, a question is referred to the Health Care Financing Administration (HCFA), which may seek a recommendation from the Public Health Service (PHS). PHS has traditionally used four criteria in its recommendations: efficacy, safety, stage of development, and acceptance by the medical community. Other health insurance programs, such as Blue Cross/Blue Shield, operate similarly.

The possibility of expanding coverage criteria to explicitly include costs or cost effectiveness is being examined by HCFA and PHS. The first question to be answered is whether there is a legal basis for any such inclusion. Currently, the language of the Social Security Act requires the medicare program to pay for services that are "reasonable and necessary." There is no definitive interpretation of whether that language means that the relative "cost effectiveness" of a particular technology might make it unreasonable or unnecessary.

Most current reimbursement programs are examples of programs without direct budget constraints. Each reimbursement coverage decision does not involve a tradeoff. Approval of one technology does not mean that another will not be covered. In a very real sense, the existing reimbursement system is an open-ended system of financing medical care.

The **PSRO program** was enacted to assure that health services provided under medicare, medicaid, and certain other programs are medically necessary, meet professionally recognized standards of care, and are provided at the most economical level consistent with quality care. CEA/CBA approaches have theoretical applicability in three areas: 1) the development of standards of care against which actual practices are judged, 2) the internal management of individual PSROS, and 3) evaluations of the national PSRO effort. Cost-effectiveness criteria have not been directly incorporated into standards of care except in a few instances (e. g., respiratory therapy), but it appears possible for PSROS to do so, although the studies would most likely have to be conducted elsewhere. PSROS generally do not have the analytical capabilities for CEA/CBA. Net cost techniques have been used to evaluate whether the savings achieved outweigh the costs of administering the review activities of the overall PSRO program. These analyses, which often reach contradictory conclusions, do not, however, examine costs in relation to changes in health outcomes that may result from PSRO reviews. Nor do they address the fact that even the most optimistic reports of savings represent an infinitesimal portion of total medicare and medicaid expenditures.

Because individual PSROS operate under a program management budget, incentives exist for them to use cost-effectiveness-like approaches in choosing areas in which to concentrate their review activities.

In contrast to PSROS, in the area of **health planning,** the National Health Planning and Resources Development Act, with its amendments, explicitly states that resources are to be allocated in a more efficient manner and that health planners should weigh both costs and benefits in their decision processes. The Health Resources Administration (HRA) is emphasizing a more analytical approach to health planning, especially in regard to capital budgeting. OTA found that State health planning and development agencies (SHPDAS) and health systems agencies (HSAS) are for the most part still primarily health "needs" oriented. An OTA survey of planning agencies indicated that few agencies, even those in the vanguard of using economic analysis for allocation of resources, are going beyond the practice of considering only capital costs. There is a slight trend, however, for the most analytically advanced staffs

to consider the marginal (or incremental) costs associated with changes in use of a technology. The analysis that took place around the CT scanner is a good example of that. OTA discovered no HSAS that explicitly balance costs with health benefits in, for example, certificate-of-need recommendations. Thus, although there appear to be no legal barriers to its use, CEA/CBA has not been much applied. In health planning, as in reimbursement, there is no direct budget constraint, i.e., the area served by an HSA is not operating with a fixed or predetermined amount of resources to be spent on health care. Few pressures, therefore, act on planning agencies to force consideration of how to get the most health effect for the fewest dollars.

Market approval for drugs and medical devices, by the Food and Drug Administration (FDA), is an area where Congress has specified the decision criteria, which explicitly do not include costs. FDA is required to regulate the market introduction of drugs and devices on the basis of effectiveness (efficacy) and safety. FDA has not formally used cost-effectiveness or any other economic criteria to evaluate drug or device applications. The primary purpose of FDA is to protect the public from unsafe and inefficacious products. Although the Agency's processes do have indirect influence on the way resources are allocated, there are several factors arguing against the incorporation of explicit economic criteria in its decision processes. Perhaps the most important of these is that by incorporating such criteria, FDA might be extending its regulatory functions beyond those envisioned by Congress. Further, the administrative burden and demands of doing so would be enormous.

The **Federal health R&D effort** encompasses a broad spectrum of activities and involves several Federal agencies. At one end of the spectrum is biomedical and behavioral research which is supported by the National Institutes of Health, and at the other end is health services research such as that supported by the National Center for Health Services Research, the National Center for Health Statistics, and HFCA. Lying somewhere in the middle of the spectrum and incorporating both end points is research supported by the National Center for Health Care Technology. The Federal agencies involved in health R&D rarely use explicit CEA/CBA considerations to set research priorities, to allocate research resources, or to evaluate the results of research. The uncertain end products of much research makes it difficult to conduct CEA/CBA. Technologies being assessed or to be developed are often at an early stage of the lifecycle. A CEA/CBA-like approach, with no requirement for aggregation of variables, may be more applicable for R&D than is rigid, traditional CEA/CBA. The more basic the level of research, the less applicable the techniques become, owing to the increased uncertainties. Ironically, it may frequently be more desirable to assess a technology, including its cost implications, earlier in its development rather than later.

HMOS are both insurers and providers of medical care. They are an example of a program operating under a constrained budget and have a direct economic incentive to provide "cost-effective" care. However, a preliminary OTA examination of HMO's decisionmaking criteria indicates that, at least in part because HMOS operate in a predominantly fee-for-service environment and must compete for enrollees, these organizations do not commonly weigh health benefit against cost in deciding what medical services to offer. The actual analytical approach used by HMOS seems to be related to "net cost" techniques. Although HMOS do not always consider health benefits, their use of a "net cost" approach suggests that they view the provision of technology in terms of efficiency. They seek ways to provide benefits comparable to fee-for-service medicine at the lowest cost feasible. The current potential for use of CEA/CBA by HMOS, weighing health benefits against costs, does not appear to be as large as the existence of direct budget constraints would predict. That situation may change, however, as more experience is gained with CEA/CBA and as HMOS increasingly encounter competition with each other in addition to fee-for-service health care providers.

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POLICY OPTIONS

Options for congressional consideration fall into two categories: 1) those that relate to the current possibilities for using CEA/CBA in policy formulation and decisionmaking (Options 1 through 6), and 2) those that relate to the development of CEA/CBA techiques in themselves (Options 7 through 10).

Options Relating to Current Possibilities for Use of CEA/CBA

Options 1 through 6 follow a four-stage progression of confidence in the current applicability of CEA/CBA to health care programs—from a perception that its use should be discouraged (Option 1), to a neutral, status quo attitude (Option 2), to cautious encouragement (Option 3), to a positive belief that CEA/CBA can be effectively applied (Options 4 through 6). Options 1 through 3 present general approaches that are mutually exclusive for any given program area. Each of Options 4 through 6, however, relates to a specific program **area.**

OPTION 1

Discourage or prohibit the explicit inclusion of cost-effectiveness criteria in specified health care resource allocation decisions.

The implicit weighing of the costs and benefits of resource allocation decisions cannot—and should not—be legislated out of the policy process. It is inherently a part of that process. What this option does, therefore, is signal that the techniques available to formalize the now informal "process of weighing these costs and benefits are not regarded as sufficiently valid to justify their use.

For those types of decisions where costs have not previously been explicitly considered to any significant extent (such **as** medicare coverage of specific technologies), this option would reflect a positive decision not to include costs explicitly. For decisions made within a constrained budget, where the costs of alternative allocations of resources are automatically, though often implicitly, factored in, this option would be a statement that existing methods of considering costs are either adequate or would not be improved upon by mandatory use of formal analytical techniques.

Reflected in this option are a recognition of both the inherent methodological limitations of CEA/CBA and the weaknesses that result from the fact that CEA/CBA is still an evolving methodology; a belief that other factors in the decisionmaking context are more important than CEA/CBA-derived information; and a desire to avoid the possible misuse of CEA/CBA. These considerations, along with considerations of the country's limited experience with making health care allocation decisions based on CEA/CBA, and the expense associated with conducting large numbers of CEA/CBAs required for decisionmaking, all support this option.

However, if the health care system is in fact operating in an era of limitations on resources and if the concern over rising health care costs continues to lead to pressures for better balancing of costs with outcomes in the use of medical technology, this option becomes much less attractive. It might eliminate most of the current and future developmental work on techniques for comparing costs to benefits. It would force reliance on current methods of making decisions. If Congress is not satisfied with the allocation and use of medical technology and other health care resources and not satisfied with the cost implications of such allocations, some method for more explicitly forcing a weighing of costs with benefits may need to be developed. (Options 7 through 10 below could be a step in that direction.)

Thus, this option should not be adopted if Congress believes that more explicit balancing of costs and benefits is necessary and that CEA/CBA or similar techniques hold the potential to contribute to that process. Rejection of this option does not mean that immediate mandatory use of CEA/CBA should take place; it simply does not shut the door to that possibility *or* to the possibility of encouraging methodological development, experiments with increased use of CEA/ CBA, and increased use of formal analytical techniques for balancing costs with benefits.

OPTION 2

Allow current trends in development and use of CEA/CBA to continue, but make no changes in legislative requirements or prohibitions relating to the use of CEA/CBA.

This is basically a status quo option, but it recognizes that the field of CEA/CBA is undergoing methodological change and that analysts and policymakers are paying increasing attention to the potential uses of the techniques. These trends would continue under this option. (They could be encouraged through implementation of any or all of Options 7 through 10.) But no changes in legislative language regarding the formal, explicit use of CEA/CBA in program decisions would be made. Since OTA found that very little formal use of CEA/CBA currently takes place, that nonuse would most likely continue into the near future.

Under this option, health care programs would be allowed to examine the possibility of using more explicit costs and benefits information. But they would not be required or encouraged to do so by Congress.

In medicare reimbursement decisions, this option would enable HCFA and PHS to continue their investigation into the possibility of adding cost effectiveness to the list of coverage criteria. At the same time, the option recognizes that the negative aspects of adding cost effectiveness as a criterion (discussed below under Option 4) are quite serious. It would allow demonstration projects (as presented in Option 10) to take place before a general decision to change current reimbursement policies is made.

Current legislative language for market approval of drugs and medical devices would continue to specify only safety and effectiveness as primary decision criteria. This situation seems to reflect congressional intent to date and would avoid the problems associated with changing the laws to include cost effectiveness (as examined in Option 6 below.)

Similarly, other health care programs would be allowed to continue investigating the possibility of using more explicit costs and benefits information. But they would not be required or encouraged to do so by Congress. For example, the health planning program could continue to provide technical assistance regarding cost and cost-effectiveness analysis to local HSAS. Also, Congress could encourage or discourage the establishment and expansion of HMOs without mandating or prohibiting their use of cost-effectiveness criteria for decisionmaking regarding technology.

Selection of this option would in part reflect the view that the pros and cons of the other options are too uncertain to permit a definite decision on prohibition or on active encouragement of use of CEA/CBA. But it also would reflect a belief that some method of more explicit consideration of costs and benefits is needed.

OPTION 3

Encourage the explicit consideration of costs and benefits in resource allocation, but do not require it as a formal decision criterion.

Selection of this option requires a more favorable view of the current potential of formal analysis than does the previous option. This option, in effect, says that health care is at the point where costs should be explicitly compared to benefits when decisions about resource allocation are made, and that the techniques that are available to make such comparisons are useful—though not well enough developed to be used as mandatory aspects of the decisions. This option goes beyond the status quo. Congress, by adopting this approach, would be explicitly approving the approach of CEA/CBA. Decision areas such as health planning would receive clear signals that decisions on resource allocation should be *in part* based on formal identification and (where possible) measurement and valuation of relevant costs and benefits.

Should this approach be taken, Congress could, for example, inform HCFA and PHS that it approves of efforts examining the possibility of including cost effectiveness of medical technology as a criterion for reimbursement coverage and that it approves use of such a criterion if that examination finds it to be feasible. Reimbursement coverage is mentioned here only as an example; the difficulties in this area are substantial (see Option 4) and this may not be an area where Congress wishes to encourage CEA/

CBA. Similar statements can be made in regard to encouraging the use of CEA/CBA in market approval decisions by FDA.

There are, however, areas where more explicit consideration of costs and benefits might be encouraged with less negative impact. One is in resource allocation decisions by health planning agencies. Costs and benefits are already listed as criteria for such decisions. Also, quality assurance activities of PSROS might be modified to take costs and benefits into more explicit consideration. Encouragement in these areas might take the form of providing increased analytical capabilities to agency staffs, providing technical assistance based on modified CEA/CBA-derived national data for agency use, and, importantly, signaling a congressional desire that *all* relevant costs and benefits of agency decisions be considered, not just those costs or benefits that may be easily quantified.

The essence of this option is that costs and benefits should be a *part* of resource allocation decisions, but that current methodologies argue against leaning on formal analysis too heavily.

A positive aspect of this option is its direct recognition of the necessity of having some way of making resource tradeoffs. The option may also result in an increase in knowledge about the value of current and, eventually improved, analytical techniques such as CEA/CBA. A negative aspect is the difficulty of encouraging use of CEA/CBA while not requiring it. Substantial thought should go into the specific means of implementing this option in individual health care programs.

OPTION 4

Specify that medicare reimbursement criteria should include consideration of technology's cost effectiveness.

This option would add consideration of costs, which have not been a criterion to date, to the largest Federal health program. The option's desirability depends on philosophical, methodological, and logistical factors. Philosophically, selection of this option would signal a change in the rationale of medicare and perhaps other Government-funded health programs. Currently, medicare operates, with some exceptions, as a program to enable the aged and disabled to have access to the medical services available to the general population. For some very expensive life-prolonging technologies (e.g., renal dialysis), however, medicare has become the vehicle by which those technologies are made generally available to all age groups.

If, under this option, cost-effectiveness criteria are applied to all technologies to be covered under medicare, the aged and disabled might be denied reimbursement for use of technologies that were still available to other insured populations.

Further, major changes in the procedures for raising coverage questions would have to be made. The local contractors might have to refer a great many questions to HCFA for resolution, and HCFA would depend on PHS for a large volume of analysis. Moreover, the data on which coverage recommendations and decisions would have to be made do not exist for many technologies. The administrative expense would be large and the analytical base would have to be greatly expanded at considerable cost. The potential cost savings from more cost-effective use of technologies, if that were brought about by application of CEA/CBA, would have to be compared to the expenses generated by this option. There is, however, no way to estimate confidently either the probability or the amount of medicare cost savings that might result. This option, if implemented in the absence of an overall system to control expenditures, would have little effect by itself on the absolute amount of expenditures.

Consideration might be given, however, to requiring that the possible addition of major high-expense technologies or inexpensive but high-volume technologies to medicare be carefully assessed and their potential impact on the total health budget be analyzed. Tradeoffs between increases in medicare expenses and increases in medicaid benefits or PHS programs could then be considered.

OPTION 5

Require that resource allocation decisions by health planning agencies be based in part on formal cost-effectiveness criteria.

This option implies a belief that the state-of-the-art of CEA/CBA is sufficiently advanced to provide useful and valid information to health planners. It further implies that State and local health planning communities and their constituents will accept the results of CEA/CBA studies as inputs to the decision process. The feasibility of the option also depends on health planning agencies' developing the capability to perform or commission adequate analyses or having access to studies done by others that will be adaptable to the local situations.

OTA finds that, although recent legislation does indicate that Congress intends that health benefits be weighed with costs in planning decisions and that HRA is providing some guidance to State and local planning agencies in this regard, there is no consensus as to an adequate analytical method to which health planners can turn. At the same time, however, OTA finds that helpful information for many decisions can be obtained by following generally accepted principles of analysis, which include explicitly enumerating all the costs and benefits of a given course of action. In general, health planning agencies at present do not have sufficient technical skills to perform formal CEA-type studies.

OPTION 6

Modify the food and drug laws to include cost effectiveness as a market approval criterion for drugs and medical devices.

This option might force FDA to formally compare the safety, efficacy, and costs of a new product with those same characteristics of existing technologies. Such an effort would require an extensive amount of data, much of which does not currently exist. The analytical capabilities of FDA would have to expand and change markedly to incorporate the new criterion. The administrative and analytical demands of this option would be enormous.

Most importantly, the decision of whether to select this option would hinge on current congressional intent regarding market approval. Past intent, as reflected in the statutes, explicitly mentions only safety and effectiveness (efficacy). Approving this option would mean that the intent of Congress regarding FDA's regulatory function has changed. If such is not the case, the option is inadvisable. If intent has changed, then the feasibility of the option depends on the minimization of the administrative and methodological problems.

Options Addressing the Techniques of CENCBA Themselves, or Their Development

OPTION 7

Encourage research on the methods of CEA and CBA, concentrating on general principles of analysis.

OPTION 8

Encourage the conduct of increased numbers of CEA/CBAs of medical technology.

OPTION 9

Encourage the development of a strategy for identifying and collecting information needed for CEA/CBA.

OPTION 10

Require or encourage demonstration projects to test the feasibility of CEA/CBA as an aid in decisionmaking.

These options are not mutually exclusive; any combination of them could be implemented. They are designed to yield information about the future usefulness of CEA/CBA in decisionmaking and to increase CEA/CBA's potential for being useful. None of the options requires an immediate commitment to or decision on the ultimate use of CEA/CBA. All might contribute to advancement of the state-of-the-art of CEA/CBA.

Research as proposed in *Option 7* might concentrate on issues such as the development of: 1) principles of formal analysis, as presented above, that could be used by analysts to conduct studies and by decisionmakers to evaluate and use studies; 2) "array" or nonaggregated methods of presenting and considering costs and

benefits; 3) measurement indexes that attempt to capture and weigh divergent variables; and 4) analytical approaches to various categories of decisions in health care, for example, the use of diagnostic technology, or assessment of new versus established technologies. This research could be coordinated with the studies that would be conducted under Option 8. Those studies, in order to help determine the potential usefulness of CEA/CBA, should be undertaken or at least designed and evaluated in collaboration with agencies making policy decisions relating to medical technology. Option 8 cannot be merely an academic exercise; it cannot be done in a policy vacuum. If it is, few relevant lessons about CEA/CBA will result. Further, because the number of technologies that could be studied is extremely large, the setting of priorities for selecting those to be analyzed is of critical importance.

Option 9 does not mean the collection of additional data. OTA believes that such collection should be postponed until much more thought has gone into a strategy that specifies the most useful kinds and forms of information needed to conduct analyses of costs and benefits. The usefulness of CEA/CBA is critically dependent on the quality of the data available. Currently, the state of many types of data, especially data on efficacy and safety of medical technology, is inadequate. Much work has already gone into examining the state of health data systems and content. The strategy envisioned by Option 9 would build upon the existing studies, but would require consideration of the specific needs of CEA/CBA analysts and decisionmakers.

The need for some method of comparing the effects of health care activities with the resources consumed will remain critical to policymakers. Thus, the potential of CEA/CBA or some derivation should be explored fully. This **may** require, or at least benefit from, limited experiments on the actual formal application of CEA/CBA in program decisionmaking. Three possible areas for the demonstration projects of *Option 10* may be medicare's reimbursement coverage decisions, the resource allocation activities of health planning agencies, and the review activities of the PSROS. Option 10 recognizes that the methods of CEA/CBA may improve, that data inadequacies may be lessened, and that methods of assuring the appropriate consideration of CEA/CBA results (in relation to other variables of the decision) may be developed. Thus, today's judgments of the role and usefulness of CEA/CBA may need modification later. In fact, demonstration projects may help to advance the usefulness of CEA/CBA by contributing to advances in methods, data, and so on.

2
Evaluating Costs anti Benefits in Health Care

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2
Evaluating Costs an; Benefits in Health Care

INTRODUCTION

A substantial share of the health policy community seems to perceive cost-effectiveness analysis / cost-benefit analysis (CEA / CBA) as a potentially significant aid in the attempt to reduce health cost inflation and inefficient resource allocation. Officials of both public and private sector third-party payers have cited their desire for CEA / CBA to assist them in reimbursement decisions. Recent conferences on medical technology issues, attended by experts from academia, Government, and the private sector, have focused on the need for technology assessment, given its presumed significant potential role in reimbursement decisionmaking (427). In addition, the Federal Government is building the institutional machinery to guide, support, and possibly use analysis.

More generally, the health care literature exhibits a growing interest in the cost effectiveness of health care. * Further, a wide variety of health care organizations have demonstrated concern with the resource allocation and cost containment issues that CEA / CBA is presumed to address. Illustrative of the interest and concern are growth in the teaching of health economics in medical school curricula (18), the separate efforts of the American College of Physicians and the Resident Physicians Section of the American Medical Association to promote cost-effective care, and the work of Professional Standards Review Organizations, medical society cost containment committees, and so on.

The potential of CEA / CBA to contribute significantly to cost containment and improved resource allocation seems to be an article of faith to many officials and health policy experts, but both the potential significance and nature of any contributions of these techniques remain to be established. Despite the fact that different observers have different kinds of impacts in mind when they discuss these analytical techniques, the several qualitatively distinct kinds of impacts CEA / CBA might have are rarely articulated explicitly.

The purpose of this chapter is to set the stage for later discussions of findings regarding the usefulness or potential usefulness of CEA / CBA.

The public, or governmental,[2] sector is called upon to make certain decisions that are impractical for the private sector to make. Examples of these are decisions concerning national defense or air pollution control, neither of which is amenable to being traded in the marketplace. The public sector also must make other decisions for social reasons such as assuring equitable distribution of what may be considered essential goods (e. g., health care of the elderly), Although the inherent complexities of these decisions and the uncertainties of the variables involved make it extremely difficult to identify and weigh all the consequences, the quality and validity of the decisions can be greatly enhanced by structuring the process so as to reveal as many of the relevant costs and benefits as is feasible and by evaluating them from a social perspective.

Since conventional private sector techniques such as capital budgeting and return-on-investment analysis are ordinarily insufficient for decisions made in the public arena, special tech-

See Background Paper #1 Methodological issues and literature Review, prepared by OTA in conjunction with this assessment.

[2]This report uses the term "public sector" to refer to governmental institutions and the term "private sector" to refer to all other segments of society. Some people further distinguish between the for-profit "private" sector and the not-for-profit "private" sector (including, e.g., foundations, universities, or institutions such as the Blue Cross Association). The term "independent sector" has been suggested for this latter sector.

niques have been developed. In the medical care area, two such techniques are CEA and CBA. CEA and CBA are designed to integrate the economic aspects of a decision with the health aspects of that decision. Consequently, **they should not be considered simply as economic tools.**

DEFINITION OF CEA/CBA

The terms CEA and CBA have come to refer to formal analytical techniques for comparing the positive and negative consequences of alternative ways to allocate resources. In practice, the comparison of costs and benefits is accomplished through a spectrum of approaches, ranging from sophisticated computer-based mathematical programing 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. At one end of the continuum are what will be referred to as "net cost" studies. In these studies, the emphasis is on costs; net cost 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 the technologies under study to specific health-related outcomes and compare the costs of the technologies to the differential health benefits. Thus, CEA/CBA comprises a set of analytical techniques, differentiated by the specific costs and benefits that are considered and the manner in which they are analyzed.

The principal distinctions between CEA and CBA lie: 1) in the method of valuation of the desirable consequences of a decision, and 2) in the implications of the different methods of that valuation. In CBA, all costs and all benefits are valued in monetary terms. In a CBA (simplified for illustration) of an emergency medical system, for example, the cost of the program would be estimated and compared to the benefits. The latter, which might consist of saving a certain number of lives, would somehow be valued in dollars, and the results of the analysis would be expressed in dollar cost per dollar benefit. Thus, conceptually, CBA can be used to evaluate the "worth" of a project and would allow comparison of projects of different types (e.g., such as dams and hospitals).

In CEA, on the other hand, desirable program consequences are not valued in monetary terms, but rather are measured in some other unit; common measures include years of life saved, days of morbidity or disability avoided, or combination of morbidity and mortality such as quality-adjusted life years (QALYs) saved. The reason for a nonmonetary measure of program effectiveness is either the impossibility or the undesirability of valuing certain outcomes in dollars. Thus, unlike that of a CBA, the "bottom line" of a CEA is not expressed as a net monetary value or a monetary ratio; rather, it is expressed in units such as "dollars per life saved" or "dollars per QALY gained." CEA does permit comparison of cost per unit of effectiveness among competing program alternatives designed to serve the same basic purpose. Unlike CBA, however, it does not allow comparison of programs having vastly 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 question requires a social and political judgment; it is not simply a technical matter. Naturally enough, as the state-of-the-art of developing composite indexes of outcome measures improves, the versatility of CEA also advances, because the technique can then be used to compare increasingly divergent programs.**

In this report, the term CEA/CBA is used to refer to the class of techniques that includes both CEA and CBA. The findings of the following chapters apply generally to both techniques.

IMPORTANCE OF EFFICACY AND SAFETY DATA

As emphasized above, CEA/CBA should not be considered only an economic tool. This point is not negated by the fact that CEA/CBA is described as an efficiency-based technique. Measurement of efficiency depends as much on output as on resources used to produce the output. OTA believes that one of the critical output or outcome measures that are or can be addressed by CEA/CBAs is effect on health status or other health-outcome-related effects. Any CEA/CBA that attempts to analyze such outcomes for evaluation of medical technology will be only as comprehensive and valid as the data on the efficacy and safety of that technology are. Thus, **health-outcome-related CEA/CBAs are dependent on the existence of an adequate efficacy and safety information base.** As OTA has reported previously, however, the status of such information is inadequate for many medical technologies (465).

HISTORY OF CEA/CBA

The commonsense principles of CEA/CBA have been promoted for centuries, but formal application of CEA/CBA is primarily 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 benefit (of projects) to whomsoever they may accrue (must be) in excess of the estimated costs," though the Act provided no guidance as to how benefit 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 that 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 (585).

Early in the Kennedy administration, the Defense Department, under Secretary McNamara, adopted a program budgeting system that 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 Government. CEA/CBA represented both the spirit and the letter of the new initiative to rationalize Government resource allocation decisionmaking (547).

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 (256, 352). 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 (516). The philosophy and logic of CEA/CBA and PPB have been reincarnated during the Carter administration in the form of zero-based budgeting.

In this assessment, OTA found relatively few sound applications of CEA/CBA in health resource decisionmaking. There are, however, increasing numbers of such studies being performed, particularly in the applied research field, and the results of these studies are increasingly being disseminated. Although it is difficult to know how much effect this type of information has, there is ample evidence that both the private and public health sectors are increasingly cost conscious.[3]

[3]See ch. 2 of *Background Paper #1: Methodological Issues and Literature Review*, prepared by OTA.

OTA found two divergent trends in published CEA/CBA studies. One trend, based largely within the academic community but slowly diffusing to research-oriented practitioners, is leading to rapid changes in the state-of-the-art of CEA/CBA. This trend is toward CEA/CBA studies characterized as interdisciplinary, of high quality, and advancing the state-of-the-art of the methods. These studies are also becoming increasingly oriented to concerns peculiar to the health field, such as health status measurement, equity considerations, and the value of health and of life itself. Since the studies are gaining wide readership—being published in the leading journals—and are becoming more understandable to the lay public, they are enhancing acceptance of CEA/CBA.

The other trend, which is occurring outside the academic community, is characterized by a rapidly expanding CEA/CBA literature base. Many of the articles are written by practitioners who are increasingly concerned about the general concept of cost effectiveness in medical practice. For the most part, this body of literature is found in general health and medical journals. Although the analyses are not as methodologically advanced or complex as those in the former group—probably because the authors often do not have an economic or other quantitative background—their impact upon physician practice may be substantial. The increasing number of studies, in any case, certainly can be considered an index of practitioners' concerns about health costs.

LEGAL STATUS OF CEA/CBA

Currently, the law explicitly authorizes only one health-care-related agency, the National Center for Health Care Technology, to support CEA/CBA studies, although parts of the National Health Planning and Resource Development Act (Public Law 93-641) require that cost effectiveness be considered in some decision processes. Furthermore, no court rulings or pending cases directly relate to the use of these techniques in health care agencies. In areas other than health (e.g., environmental regulation), however, there are immediate and significant judicial and legislative pressures to use CEA/CBA in decisionmaking. In the last two Congresses alone, more than 65 separate bills have included provisions for Federal agencies to use CEA/CBA or risk-benefit analysis in the decisionmaking process. See appendix E for a brief discussion of the legal status of CEA/CBA.

USE OF CEA/CBA IN HEALTH CARE DECISIONMAKING

There are two basic types of health care resource allocation decisions that *in theory* could benefit from CEA/CBA. The first are decisions made within a fixed, constrained, or population-based budget, such as those made by health maintenance organizations (HMOS). The second are decisions made in the absence of a direct budget constraint, such as reimbursement decisions by medicare or certificate-of-need recommendations by local health planning agencies.

In the former—allocation decisions made within a budget—tradeoffs must be made, since not all projects can be funded. The projects that promise to deliver more benefits for the cost should be more attractive than those projects expected to deliver fewer benefits. In these decisions, an economic constraint already forces costs to be considered. The function of CEA/CBA in budget-constrained decisions, therefore, would be to illuminate the decision process and to require that implicit judgments be made more explicitly, thus forcing external examination of assumptions and of the values placed on the decision variables. Note that even though costs are normally taken into account in these budget-constrained situations, the types of CEA/CBA or related analyses undertaken can still range from analyses on the net cost end of the continuum to analyses where effectiveness is ex-

plicitly related to health outcomes or some equivalent measure.

In non-budget-constrained decisions, direct tradeoffs between competing projects often are unnecessary. Consequently, a function of CEA/CBA in these decisions would be to force economic factors to be considered. In health planning decisions, for example, planners would be asked not only to consider whether a service is needed but also to compare the cost of the service with the expected benefits and perhaps to compare the costs and benefits of the service under study to the costs and benefits of other services that could be assigned higher or lower priorities.

CEA/CBA is viewed by different parties as ranging in usefulness from obfuscating the pertinent issues in a decision process at one extreme to illuminating and synthesizing the issues so well that the technique is used to make decisions at the other extreme. There is, however, a middle position that maintains that the technique could be helpful in structuring information and that **this information should be only one of several components of a decision process. Both extreme positions mentioned above are associated with the use of CEA/CBA as a formal, structured analysis that is oriented toward a bottom-line answer,** such as a cost-benefit ratio. Such a bottom-line, however, may avoid or even hide many important value judgments, thus providing an unambiguous answer which may rest on ambiguous data or assumptions. Advocates of the middle position propose that CEA/CBA be used within the context of accepted principles of analysis in order to illuminate the costs and the benefits of a decision, but not necessarily to aggregate and weigh them. Warner mentions a similar perception of CEA/CBA as a consciousness-raising exercise: CEA/CBA would have "no direct influence on policy decisions, but its presence in the literature and in policy debates (would serve) to raise the general awareness and understanding of the economic side of health care, particularly among members of the medical profession" (615). When properly conducted, CEA/CBA can serve as a means of raising value and equity issues related to the subjects under study.

Most of the specific findings of this report relate to two major general findings of the OTA assessment. The first of the general findings is that **performing an analysis of costs and benefits has the potential to be very helpful to decisionmakers, because the process of analysis structures the problem, allows an open consideration of all relevant effects of a decision, and forces the explicit treatment of key assumptions.** The second general finding is that CEA/CBA ex**hibits too many methodological and other limitations to justify relying solely or too heavily on the results of formal CEA/CBA studies in making a decision. Thus, CEA/CBA could be useful for assisting** in many decisions, but is probably not appropriate as the sole or prime determinant of a decision.

3
Methodological Finding; and Principles

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3
Methodological Findings and Principle;

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.

SOURCE Office of Technology Assessment

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.*

4. Uses and Usefulness of CEA/CBA: General Findings

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4. Uses and Usefulness of CENCBA: General Findings

INTRODUCTION

Substantial disagreement and confusion surround the question of the potential usefulness of cost-effectiveness analysis/cost-benefit analysis (CEA/CBA) in decisions regarding medical technology and the health care system. With the continuing concern over health care expenditures, and with the advocacy of CEA/CBA by many people and groups as a means to ameliorate cost-related problems, this confusion and disagreement take on a significance that is far more than academic.

OTA believes that the potential usefulness of CEA/CBA depends very critically not only on the feasibility but also on the implications of its use. Accordingly, in this assessment, OTA examined three major issues: 1) the general usefulness (past and potential) of CEA/CBA in decisionmaking regarding medical technology; 2) the methodological strengths and limitations of CEA/CBA; and 3) the potential for initiating or expanding the use of CEA/CBA in health care decisionmaking regarding medical technology, especially in six health care programs—reimbursement coverage, health planning, market approval for drugs and devices, Professional Standards Review Organizations (PSROS), R&D activities, and health maintenance organizations (HMOS). A major aspect of the examination of the third issue is the potential implications—to the programs, to decisionmaking quality, and to society's values–of CEA/CBA use in the six program areas.

The primary focus of this assessment is on the application of CEA/CBA to medical technology—which OTA defines as 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 CEA/CBA and psychotherapy, [1] the assessment does not directly address psychosocial medicine. Other aspects of health, such as the environment, are also not covered. OTA believes, however, and it was the consensus of the advisory panel, that the findings presented in this report, and in the background paper on methodological issues,[2] may apply also to other areas such as health care resource allocation in general. With modification, the findings may also apply to areas such as environmental health regulation, occupational safety and health, and education resource allocation.

Furthermore, although the subject of the assessment was CEA/CBA, the findings should also be examined with an eye to their applicability to other types of formal analysis. Risk-benefit analysis, decision analysis, systems analysis, technology assessment, and social impact assessment, for example, are all techniques used to examine various policy questions in both public and private organizations. The usefulness and implications of each of these techniques will vary according to many of the same factors that affect the usefulness of CEA/CBA. In fact, there are only hazy distinctions between these other forms of analysis and the forms of CEA/CBA.

[1] *Background Paper #3: The Efficacy and Cost Effectiveness of Psychotherapy*, prepared by OTA.
[2] *Background Paper #1: Methodological Issues and Literature Review*, prepared by OTA.

GENERAL FINDINGS

OTA found few examples of well-conducted, sophisticated CEA/CBAs conducted for and used in decisionmaking in health care. It is likely, however, that the extent of use of CEA/CBAS in health care decisionmaking OTA found in its survey (see app. B) understates actual usage—of informal CEAS in particular, but of formal, relatively sophisticated analyses, as well. OTA'S survey was not exhaustive. The effort that was undertaken to ascertain the amount of use, though, does seem to indicate that the level of use is not substantial. Use of formal CEA/CBA in decisionmaking in health care is the exception not the rule.

It is safe to say, however, that most decisions made take into account only a subset of the potential consequences of those decisions. The inherent complexities of many decisions and the uncertainties of decision variables make it extremely difficult to identify and weigh all the consequences. In general, OTA found, the quality and validity of decisions **can be** increased by analysis that forces a structuring of the decision process, that provides a framework for identifying and considering as many of the relevant costs and benefits as is feasible.

This finding supports the two major general findings of the assessment that were presented at the end of chapter 2. **The process of CEA/CBA may be more helpful generally than would be the rigid and formal application of CEA/CBA study results in health care programs.**

Chapter 2 also set out two broad classes of health care program decisions: constrained budget ones, and nonbudget or nonconstrained ones. **CEA/CBA potentially can be more valuable for decisionmaking under a constrained budget where tradeoffs have to be made directly than when constraints are nonexistent or very indirect.** Under the budget system, the budget itself would act as a cost containing or controlling factor. Under the nonconstrained type of system, since no direct tradeoffs are required, no direct limit on expenditures is set or forced. Thus, **in neither case would CEA/CBA necessarily function as an effective cost-constraining mechanism or tool.** Advocacy of CEA/CBA as such a tool, therefore, should be regarded skeptically. CEA/CBA might, though, change the mix of expenditures. Technologies might be substituted for one another on the basis or partially on the basis of analysis—especially under a budget situation. In this regard, there is potential for CEA/CBA to help increase efficiency, even in terms of health outcome, without necessarily lowering total expenditures.

Related to this last point about the possible use of CEA/CBA in improving resource allocation within a constrained budget is the observation that this country's health care system might move closer to an overall constrained budget. This is mentioned only as a possibility. Perhaps in the future, health care expenditures may be limited (or constrained) to a fixed or specified percentage of gross national product, or to some specified absolute amount of dollars. If this situation ever comes about, or even as an increasing number of individual institutions and programs operate under budget constraints, the appropriateness of CEA/CBA may increase. In such a possible future situation, most resource allocation decisions would require explicit tradeoffs. It is conceivable, therefore, that efforts devoted to the development of a CEA/CBA-based approach to decisionmaking (not necessarily tied to numerical study results) will represent an investment in future social policymaking. The lack of direct applicability of formal CEA/CBA to many of today's decisions may then be offset by future applications of CEA/CBA.

Various sources consulted and numerous people interviewed by OTA for this assessment provided information yielding several seeming contradictions or paradoxes concerning CEA/CBA. For example, one common argument is that use of CEA/CBA may often be unethical if it does not take values and distributional issues into account adequately. There is validity to that statement. But there also seems to be validity to the argument that not considering costs and benefits in decisions on society's resources, especially in an area so basic as health, is unethical, because in the absence of the explicit consideration of consequences and of the parties on whom those

consequences may fall, inequities will very likely occur.

Another example of a seeming paradox concerns the "power" of CEA/CBA results. Some people argued that because many decisions are made in a political context, the results of any "objective" analysis would be heavily criticized and overwhelmed by other factors. Yet others argue that one of the factors in the potential misuse of CEA/CBA is its quantitative nature, allowing those involved in the decision process to "anchor" their arguments to what appear to be hard numbers. Are the results of CEA/CBA powerless? Or overly powerful?

The resolution of both these examples may lie in the distinction between the process or approach of CEA/CBA and the quantitative results of formal studies. As indicated by the two general findings of this assessment, many of the negative perceptions of CEA/CBA are based on the possible misuse or inappropriate use of formal study results. **Viewed as a method of structuring the decision process, CEA/CBA need not hide or avoid questions of ethics or values, and it need not provide a deceptively quantitative answer to** complex problems.

As an example of the difficulty of concentrating on quantifiable variables and how investigations of decision possibilities might be enhanced by thinking in CEA/CBA terms, consider the cost effectiveness of CEA/CBA itself. OTA was frequently asked whether a CEA/CBA of CEA/CBA might not be what is needed. And for a given decision situation that type of analysis might be very valuable, Approaching a CEA/CBA of CEA/CBA in order to arrive at a quantitative, traditional bottom-line result, however, might lead analysts to list as a primary "cost" of CEA/CBA the resource costs involved in conducting and interpreting the studies. Thus, resource costs such as those identified in appendix C would be included, with dollars being the measure used. If, however, the analysts were less interested in a bottom-line figure for the CEA/CBA of CEA/CBA, they might consider the opportunity costs of analyses. That is, the more important aspect of the costs of CEA/CBA may not be the dollars it takes to conduct it, but rather the alternate uses of those dollars and the alternate types of analysis and other activities that might occupy the attention of those concerned about more rational allocation of medical technologies (617). Would the funds and attention that could be devoted to CEA/CBA be more productive if applied to efficacy and safety studies? To education or consciousness-raising of physicians? To more dissemination of existing knowledge of the costs and benefits of various technologies? To regulation of the use of technology? These are the questions that probably should be asked.

FACTORS AFFECTING THE USE AND USEFULNESS OF CEA/CBA

One of the key factors affecting the uses and usefulness of CEA/CBA has already been discussed in chapter 3: the technical, methodological feasibility of the technique. These methodological factors can be inherent aspects of CEA/CBA, or they can be due to the state-of-the-art of CEA/CBA and thus more tractable.

The manner in which both types affect the usefulness of CEA/CBA, however, should be analyzed in the context or the environment of current or potential uses of CEA/CBA. In other words, the questions should be asked what is the decisionmaking context and how does it affect the strengths or limitations of the methodology, and *vice versa ?* For example, does the decision relate to a technology at an early stage in its lifecycle, such as bone marrow transplants? Or does it concern an established technology, such as appendectomy? Is the technology in question a diagnostic technology, such as the CT scanner, or a therapeutic one, such as renal dialysis?

The possibility of affecting the course of a technology's diffusion and use might be greater in early stages of its development, but the uncertainties about its health effects and its costs will generally be greater. Thus, it may be possible to

do a more valid or certain CEA/CBA later in the technology's lifecycle, but the information gained may be less valuable for public policy. The tradeoff required will vary depending on the specifics of the technology and the policy decision to be made. In addition, diagnostic technologies are often more difficult to study than other technologies because of the uncertainties involved in linking their use to health outcomes. Thus, studies of diagnostic technologies often tend toward the "net cost" end of the CEA/CBA spectrum, where the measures of outcome or benefit may be numbers of tests performed or levels of diagnostic accuracy.

In sum, the stage of development of the technology under study and the type of technology (or function of the technology) are two of the factors that will affect the specifics of analysis to be used, the uses to which analysis can be put, and the usefulness of resultant information. Other factors are the relative strength or importance of nonanalytical factors, such as politics or equity, in the decisions to be made; the ability of the sponsors of analysis to implement the results; the familiarity of sponsors and decisionmakers with formal analysis; the existence of adequate data relating to the technology, to the disease or other problem addressed by the technology, or on other possible effects of interventions based on analysis; the existence of economic incentives that match or run counter to the results of analysis, the types of decisions to be made (e.g., budget-based decisions or nonbudget-based decisions); and so on. Some of the factors that affect the use of CEA/CBA are listed in table 2.

One of the factors listed above is of particular importance: the quality and availability of data. Obviously, without data or estimates of data, there would be no CEA/CBAs. The quality of a CEA/CBA is directly related to the accuracy of the data used in it. For example, when good epidemiological data on the effects of a technology or the existence of disease are present, analysis will have a greater potential for being relevant and useful. A specific example of where epidemiological data have permitted analyses of high quality is in the area of smoking and its effects on health. Good data do not guarantee good analyses, however, because the quality of analysis is also affected by the other factors mentioned above. Similarly, the usefulness of analysis is dependent on those factors affecting quality as well as on a number of other factors (see table 2) relating to the decisionmaking and analytical contexts or environments.

Table 2.—Factors Affecting the Use of CENCBA

Stage of Development of the Technologies Under Study. —Tradeoff required between availability/validity of data and ability to affect the future use of the technologies. Both the type of analysis and the usefulness of analysis will be affected.

Nature of Technologies Under Study and Function of Tech. nologies Under Study.— In terms of function, diagnostic technologies, for example, often have indirect connections to health outcome and often lend themselves to the net cost type of CEA/CBA. In terms of the physical nature of technologies, surgery, for example, may involve additional uncertainties due to varying skills of surgeons and surgical settings. Both type and use of analysis will be affected, but especially the type or specific methodological elements.

Social, Ethical, or Value Influences in the Decision Environ. ment.—Very similar, often overlapping with the above factor. Will affect both the type and uses of the analysis. The example of renal dialysis applies here. Abortion would serve as another example.

Quality of the Analysis.— Can be of at least four types:

Analysis Subject to Inherent Methodological Limitations. —e.g., inability to adequately deal with equity concerns; influence of discount rate chosen on outcome of analysis.

Analysis Subject to State-of-the-Art Limitations.—e.g., difficulties in identifying and measuring many costs or effects.

Analysis Containing Errors of Omission or Commission. —These are errors not due to the state-of-the-art, e.g., failure to discount or perform sensitivity analysis when appropriate.

Ana/ysis Subject to Data Limitations. —This factor will affect quality even though the other factors might have been adequately dealt with. Much cost and health outcome data are uncertain, difficult to retrieve, or simply nonexistent.

All four of these factors can affect the quality of analysis, which in turn affects the usefulness of the results.

Ability of Sponsors or Users of Analysis to Implement Results.—The usefulness of analysis will naturally depend on the amount of control the user has over the particular technology or situation studied.

Experience/Familiarity of Users With the Type of Analysis Conducted.—This factor will affect usefulness in two ways: it will be a direct influence on the acceptability of results, and it will affect the ability of the users to appropriately apply the results.

Existence of Economic Incentives in the Decision Environment.— If the economic incentives relating to the use of the technology under study are in accord with the results, their acceptability will be great. If they run counter to the results, the usefulness will be limited, depending on the strength of the economic incentives.

SOURCE: Off Ice of Technology Assessment

There are many gaps in the data available for CEA/CBA, owing to such factors as methodological constraints, inadequate resources for data collection and interpretation, lack of communication between the users of data and those collecting it, and the sheer impossibility of collecting and analyzing all the data that could be used by someone, somewhere. The principal Federal agency charged with collecting and analyzing health data is the National Center for Health Statistics (NCHS). NCHS is currently involved in several developmental projects intended to clarify certain methodological issues related to the provision of data for CEA/CBA, especially in relation to cost-of-illness studies (see app. B).

POTENTIAL USERS OF CEA/CBA

Health care policies and other decisions are made at a variety of levels and in a variety of situations by an extremely broad range of individuals and groups. In theory, CEA/CBA results or approaches might be useful to any or all of these decisionmakers. Table 3 lists many of the decisionmakers—the list is not exhaustive but should provide an idea of how diverse and numerous the types of decisionmakers are. Three general classes of decisionmakers or potential users of CEA/CBA information are discussed in this assessment: individual medical practitioners, nongovernmental institutions, and governmental /quasi-governmental institutions.

Table 3.—Partial List of Individuals and Groups Making or Influencing Resource Allocation Decisions

Individual physicians and other health care professionals
Individual patients
Medical professional societies and boards
Consumer groups
Health industry representatives and organizations
Hospitals, clinics, other health care institutions
Labor organizations
Businesses
Health maintenance organizations
Medicare and medicaid
Other governmental health care programs
Health systems agencies, State agencies
Professional Standards Review Organizations
Blue Cross and Blue Shield Associations
Other health care insurers, third-party payers
Other quality assurance or utilization review groups
Food and Drug Administration
Ratesetting commissions
Voluntary health organizations
Public health departments
Other State and local health agencies
U.S. Congress, executive agencies, State legislatures
Health care systems, such as the Veterans Administration's and the Department of Defense's
Medical schools
Biomedical and health services researchers
Other health-related associations

SOURCE Office of Technology Assessment

Individual Medical Practitioners

Despite the fairly small amount of empirical research on the subject, it seems safe to say that CEA/CBA has had little direct impact on individual physicians' behavior.[3]

Discussions with academic physicians indicate a consensus regarding the above point on CEA/CBA's lack of impact. Beyond that point, however, the consensus dissolves. There is disagreement, for example, concerning whether CEA/CBA has, and if so the extent to which, significantly affected physicians' consciousness of economic issues. Explanations for the lack of impact on practice are numerous, with emphasis on their relative importance varying dramatically from one observer to the next. And the consensus on current practice impact does not translate into agreement on the future role of CEA/CBA in influencing individual physician behavior: Opinion seems to be split roughly in half between those who believe that CEA/CBA will cause many physicians to alter their medical practices and those who anticipate continuation of the current absence of significant effect.

The principal explanations for CEA/CBA's lack of impact on physicians' behavior to date can be grouped under two headings:

1. **The novelty of CEA/CBA in health care.** Until very recently, the Literature on health care

[3]The following discussion is taken from work done for OTA by Kenneth Warner of the University of Michigan (615).

CEA/CBA was sparse. As indicated in Background Paper #l, this has been particularly true in the medical literature. Relatively few physicians read the nonmedical health care literature; hence their exposure to the concepts and practice of CEA/CBA was minimal prior to the last few years. Needless to say, lack of exposure correlated highly with (and presumably caused in part) a lack of understanding of the technique and meaning of CEA/CBA.

The novelty of CEA/CBA in health care accounts for some of the quality problems in the published literature. While poor analytical quality certainly could be a barrier to application of the results of analysis, few observers cite it as a significant factor in the failure of physicians to apply findings to their practices.

In a similar vein, the uncertainties in analysis frequently prevent determination of an unequivocal conclusion in an analysis. Even when a firm "bottom line" is presented, nonquantified factors—for example, the distribution of costs and benefits—can make the conclusion far from definitive. Thus, one could argue that even high-quality analyses frequently do not produce findings that can or should be translated directly into practice by individual physician decisionmakers. This seems an attractive explanation for physicians' nonresponse to analysis, particularly combined with whatever bewilderment they may feel as a result of their unfamiliarity with CEA/CBA. It is not, however, an explanation often noted in discussions on the subject. Most likely, this explanation presupposes that other, preliminary barriers to application of analysis have been surmounted; the evidence is to the contrary. Thus, one might anticipate that such inherent technical limitations of analysis will grow in importance as other barriers fall.

2. The irrelevance of much of CEA/CBA to medical practice decisionmaking. There are two basic sources of irrelevance, one substantive, one structural. In the substantive category, many CEA/CBAs have involved assessments of the desirability of social programs where social, and not individual, decisionmaking was at issue. Examples include the several studies of communicable disease control programs (e.g., measles, swine flu, etc.), community (or industry) based screening programs (e.g., hypertension), and fluoridation of municipal water supplies. The subject matter of such studies precludes a direct practice response by individual physicians.

While this too may serve as a useful partial explanation of the absence of behavioral response by individual practitioners, it cannot explain the total absence of such response, since much of the health care CEA/CBA literature is clearly relevant to individual practice decisionmaking. Nor is this an often-cited explanation. A more cogent argument concerns structural irrelevance: According to a strict economic interpretation, most physicians' interests in "cost-effective care" deviate significantly from those of society. All physicians share an interest in understanding the efficacy and safety of medical technologies—technologies whose risks outweigh medical benefits are undesirable—but concerns with the economic side of cost effectiveness are either nonexistent or dependent on the physicians' economic environment. In general (619):

> Cost data are psychologically remote. (The physician's) one-on-one relationship with the patient is not in the context of the cost to society.

The physician's economic circumstances, however, can produce in the physician an often subconscious reaction to costs. To a fee-for-service physician whose patients are well insured, the cost of a technology may be irrelevant, at least immediately. If the physician works within the context of prepayment, however, the professional concern with cost effectiveness begins to approach the social concern. In all cases, the patient's economic wherewithall often will be a major consideration: In an environment of prepayment or adequate insurance coverage, high costs of technologies do not translate into direct economic burdens on patients; hence the high costs are something of an abstraction to both the immediate patient and the physician.

This economic interpretation—emphasized by many knowledgeable observers—attributes the lack of effect of CEA/CBA on medical practice to its irrelevancy and even inconsistency

with medical norms, irrespective of the quality or quantity of the literature. Accordingly, unless the reimbursement system is changed, this argument suggests, the future will auger little change in the application of CEA / CBA to individual practice decisionmaking. According to this explanation, physicians' nonresponse to CEA / CBA is not necessarily a reflection of physicians' selfish monetary interests, or their indifference to economic considerations. Rather, nonresponse to CEA / CBA perhaps reflects physicians' fulfilling their roles as agents of their clients—patients. A physician's major responsibility may be to weigh all the costs and benefits to the patient and to his or her medical practice—i. e., the aggregate of all the patients of the physician.

This argument is not an entirely economic one, because the ethics of the doctor-patient relationship are involved. If a patient is not harmed economically by performance of a certain procedure, even though only a small medical benefit might be expected, what are the ethics of the individual physician's denying or recommending against the procedure in order to represent society's cost and benefit priorities? The differences between social and individual economic and ethical considerations constitute the only frequently advanced explanation for physicians' nonresponse to CEA / CBA that does not imply a brighter future for the ability of analysis to alter individuals' medical practice policies. Systemwide changes in the economic environment, such as growth in HMOs or major reimbursement reforms, might more closely aline the practice of medicine with the precepts of analysis. The strength of the explanation does not depend on lack of understanding of CEA / CBA within the medical community; hence anticipated increases in familiarity with analysis need not promote the direct application of findings. Accordingly, barring external pressures, the economic incentives and ethical norms of medicine may very well continue to preclude widespread application by practitioners of the findings of health care CEA / CBAs, with the exception of the "easy" cases in which one procedure is demonstrated to be both more effective and less costly than an alternative.

Nongovernmental Institutions

A variety of nongovernmental institutions are potential consumers of CEA / CBAs. Insurers have a direct economic incentive to find and promote cost effectiveness in the provision of health care services; officials of major insurers, including Blue Cross / Blue Shield, have expressed their interest in the development of more and better CEA / CBAs to assist them with reimbursement decisions (see ch. 5). In an era of increasing restrictions on reimbursement, hospitals' interests in enhancing efficiency are obvious. HMOs also have a direct economic interest in cost-effective care: Greater efficiency translates into lower, more competitive membership rates and / or higher incomes for member physicians. Large business firms and unions have several reasons to be interested in CEA / CBA: Greater efficiency in the provision of medical services to employees implies lower business costs or room for negotiation of other fringe benefits; health promotion and disease prevention among workers may increase productivity and reduce other costs of disability and morbidity; and so on. As major financers of the costs of illness, each of these organizations has not only an interest in promoting cost-effective care, but also has the market power to translate judgments concerning cost effectiveness into changes in health practice.

Another group of nongovernmental institutions, not directly involved in the financing of care, is evidencing interest in CEA / CBA: professional associations. Among such groups are the Association of American Medical Colleges, the American College of Physicians, the Resident Physicians section of the American Medical Association, and the cost-containment committees of several State medical societies. In part, the interest of such groups reflects concerns about the social implications of inefficient medical resource allocation; in part, interest may reflect a perception that if the health care community does not control cost inflation, Government regulators may attempt to do the job for it. Regardless of the motivation, the demonstrated interest suggests a receptivity to information that CEA / CBA in theory can pro-

vide. This interest extends beyond the medical community. The American Dietetic Association has recently completed a study of the costs and benefits of nutrition care services; dentists have discussed the relative efficiency of alternative methods of preventing caries (78); and so on. Whether the efforts of such groups will ever translate into significant practice changes remains to be seen. But the "cost consciousness-raising" function of CEA/CBA seems well served by such efforts.

To date, direct application of CEA/CBAs to institutional policies has been limited. Some organizations have identified themselves as being in the market for specific analyses—for example, several businesses want to learn more about the costs and benefits of certain disease prevention/health promotion programs for employees (e.g., control of alcoholism and drug abuse, hypertension screening and treatment, executive exercise programs, antismoking programs)—and analysts have responded with CEA/CBAs tailored to the specific institutions' needs (275). Recent policy decisions of other organizations reflect a CEA/CBA mode of thinking, though the decisions have not derived from formal CEA/CBAs. For example, the national Blue Cross and Blue Shield Associations have recommended that member plans not reimburse for institutionally standardized batteries of laboratory tests on admission to a hospital.

The question remains whether, and if so how, nongovernmental institutions will use CEA/CBAS. Certainly, by virtue of its ability to clarify issues and collect and organize information, CEA/CBA could assist planning and decisionmaking within many of these organizations. Some kinds of findings might lend themselves neatly to policy decisionmaking. For example, persuasive evidence that a certain diagnostic procedure is both more expensive and less accurate than an alternative procedure could serve as solid grounds for nonreimbursement or nonacquisition of the former. A large cost differential between two equally effective procedures might also serve as support for a use-constraining policy decision, though opposition might be substantial if significant elements of the medical community questioned the procedures' equality of effectiveness. Indeed, whenever significant technical disagreement on efficacy exists within the medical community, CEA/CBAs seem unlikely to overcome opposition to the policies they might recommend, possibly barring the case of a truly dramatic cost difference.

This point deserves emphasis because of a major implication: Clear-cut, unobjectionable CEA/CBA results probably will be an exception, not the rule. Furthermore, they seem likely to reflect reasonably obvious differences between the alternatives being studied. When a CEA/CBA is undertaken out of genuine interest in evaluating alternatives, without significant prior expectations as to the outcome of the analysis, that outcome is less likely to be definitive. Competing professional opinions on technical issues (e. g., diagnostic accuracy) exacerbate the problem. Thus, definitive CEA/CBAs may *support* policy decisions, but their potential to *shape* such decisions seems limited by technical and political factors.

Governmental and Quasi= Governmental Institutions

A limitation of the preceding discussions is that they deal with the decisionmakers as classes of decisionmakers (e.g., nongovernmental institutions, not a specific institution). The discussion and arguments will vary according to individual circumstances. For this reason, and because its mandate is related to Federal programs, OTA analyzed the potential use of CEA/CBA by several individual Federal programs.

As an example of a reimbursement program, medicare is used, but Blue Cross and Blue Shield are discussed briefly (ch. 5). The drug and device market approval processes of the Food and Drug Administration are also covered (ch. **8).** There are two examples of programs that are federally sponsored, with national policies and administration to a degree, but primarily carried out by quasi-governmental organizations at the State and local levels: the health planning program (ch. 7) and the PSROS' programs (ch. **6).** The usefulness of CEA/CBA to the health care R&D activities of the Federal Government

is examined, using four Federal agencies as examples (ch. 9). Finally, a federally promoted program that is carried out by private sector organizations, HMOs, is discussed (ch. 10).

Each of these programs, or decision areas, uses somewhat different approaches to problems and decisionmaking. Specific approaches have been developed to address divergent and diverse issues. Distinct mechanisms to analyze decisions have been evolved in the various programs. Although informal and implicit analysis of costs and benefits seems to be a frequent aspect of policy formulation in most of the programs, however, OTA found very little formal use of CEA/CBA. In several of the areas, one being market approval, cost itself has played little or no role in decisions. Figure 1 provides a view of the relationship of the six programs to each other and to the lifecycle of medical technologies. Table 4 is a narrative explanation of that figure. An organization chart of the Department of Health and Human Services (DHHS), as seen from a perspective of interest in medical technology, is presented in figure 2 in order to show the organizations of chapters 6 through 10 in relation to other elements of DHHS.

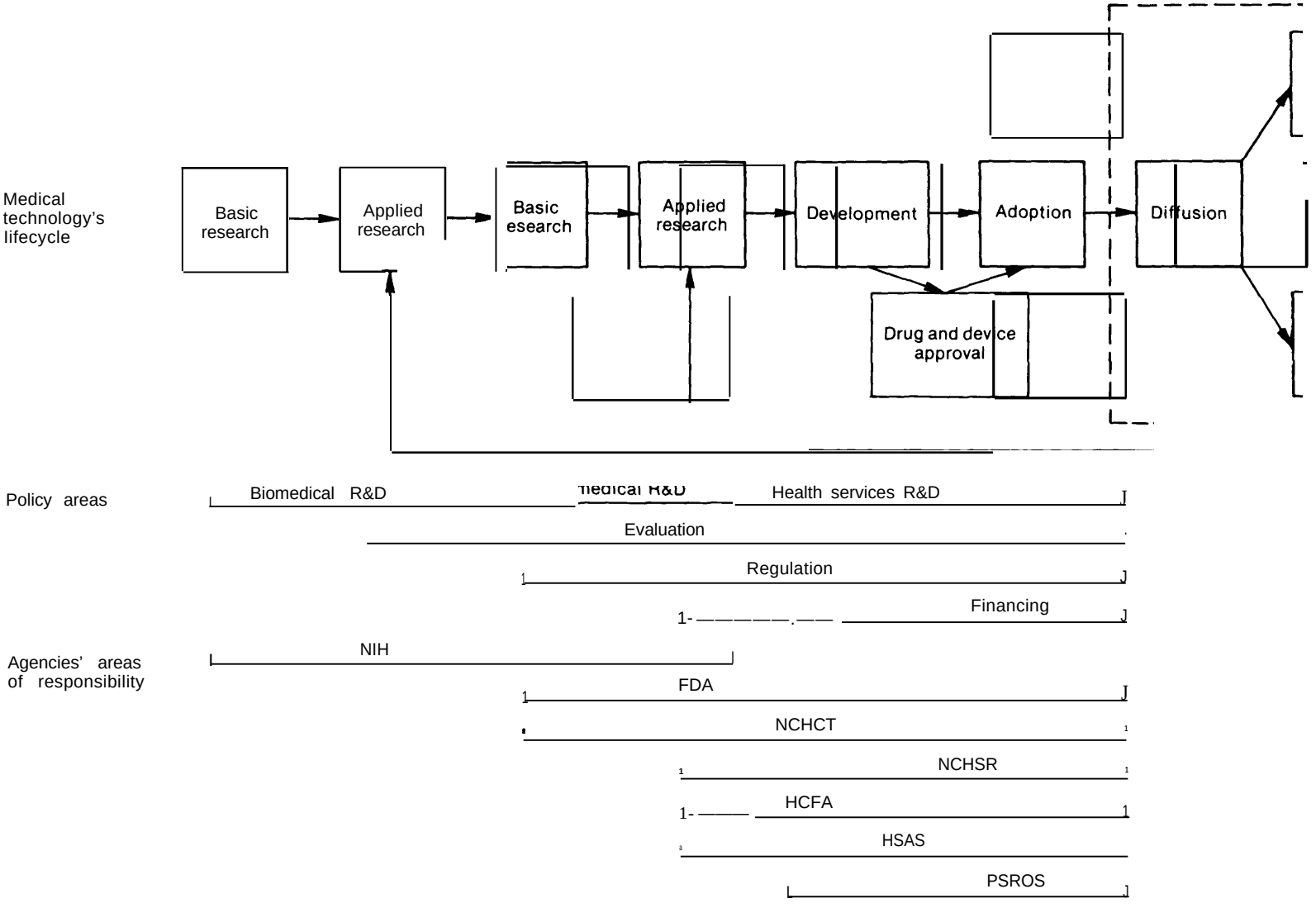
The Federal Government is not the only public institution interested in analysis. Given medicaid and other health care finance programs, States share the Federal Government's concern with health cost inflation. Local governments have also invested in analytical capability (see app. B). Indeed, one of the largest and most productive government analysis staffs was housed in the New York City Health Services Administration.

To date, Government has been the principal consumer of analysis. Still, most observers of CEA/CBA consider Government's past *use* quite limited. One reviewer, however, has concluded that "there is evidence to suggest that such studies have played an important role in public policy determination" (16). According to Dunlop, two examples support this conclusion, First, Enke's mid-1960's finding that the benefits of birth control exceeded costs by a factor of 100 contributed to Congress' significant expansion of Agency for International Development funds to assist developing countries in implementing birth control programs. Second, Dunlop says, CBAS of water fluoridation have "nearly always preceded" dental health program development, with the studies being "widely disseminated to the public" prior to a public vote on fluoridation,

Other analysts are less sanguine about the effects of past analyses on policy. Jeffrey Weiss, who headed the Program Analysis staff of the New York City Health Services Administration, has concluded that, owing to political and budgetary factors, his staff analyses had little impact on broad strategies. Analyses initiated by the staff lacked a political constituency and tended to be ignored. Of analyses requested by city officials, a few were followed by policies consistent with their findings, but these tended to support policy makers' predetermined biases on the issues. Only when administrators had not worked through problems on their own, and strong political constituencies were not involved, could analyses affect the decisionmaking framework; and in most such cases, the effects were "suboptimizing," for example, resolving narrow management issues. Weiss has cited a couple of successes, however—a critical analysis of the neighborhood family care program resulted in constriction of the program; and a study of methadone treatment of heroin addicts created a strong intellectual rationale for drug detoxification efforts in New York City (355). Weiss noted that the city administrator might have gone ahead with the latter efforts in the absence of the analysis, but the study provided support for the policy action (633).

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Figure 1.—Policy Levels in the Lifecycle of Medical Technology



SOURCE: Office of Technology Assessment

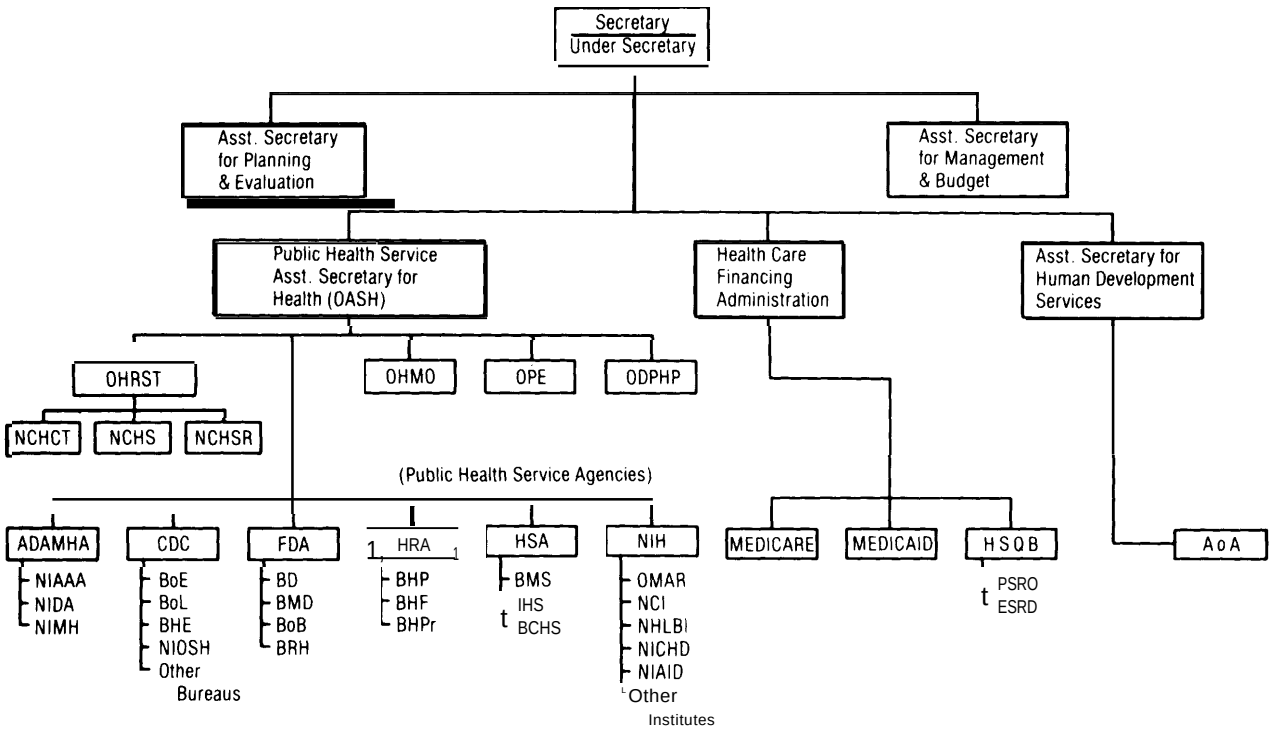
Table 4.—Overview of Agency Activities in Decisions Concerning Medical Technology

Agency/mission	Decision areas and activities: R&D	Evaluation	Regulation	Financing
NIH Improve human health by increasing medical knowledge and encouraging the development of safe and effective medical technologies	Supports and conducts basic and applied research	Establishes standards for research, clinical trials, and human experimentation Supports and conducts clinical trials Sponsors Consensus Development Conferences to evaluate medical technologies Evaluates outcomes of sponsored research		
FDA Protect the American public from unsafe and inefficacious drugs and medical devices and unsafe foods and cosmetics		Establishes regulatory requirements for demonstrations of the safety and efficacy of drugs and medical devices Evaluates safety and efficacy data from clinical trials, etc.	Regulates market approval of drugs and medical devices Regulates drug and medical device manufacturing processes Monitors the safety of marketed drugs and medical devices	
NCHCT Undertake and support assessments of health care technologies		Evaluates social, ethical, legal, medical, scientific, and economic aspects of new, emerging, and existing medical technologies Compiles and disseminates information concerning the safety and efficacy of medical technologies		Makes recommendations concerning reimbursement issues submitted by HCFA
NCHSR Support health services research on a variety of health care issues	Supports health services research, primarily on new and existing technologies			
HCFA Administer the medicare/medicaid programs, PSRO program, and support research and statistics efforts for these programs	Supports health services research on a variety of health care issues, primarily focused on areas of programmatic responsibility		Establishes reimbursement criteria for new and established medical technologies	Establishes benefits packages for medicare Makes reimbursement decisions concerning medical technologies
HSAs[a] Develop and implement local health plans and monitor the dissemination of health services			Review major capital expenditures for certificate of need Review use of Federal funds for certain programs	
PSROs[b] Assure that health care services paid for under certain Federal programs are medically necessary, meet professionally recognized standards of care, and are provided at the most economical level, possible consistent with quality care	Conduct research on quality of medical care through medical care evaluation studies and profile analyses on physicians, patients, and institutions	Review and evaluate the appropriateness of health care provided to Federal beneficiaries at acute care hospitals and long-term care facilities		Support certain Federal programs financing medical care

[a]At the national level, the Health Resources Administration's Bureau of Health Planning is responsible for providing the community or regionally based HSAs with technical assistance and guidance regarding the planning and delivery of health care services.
[b]The Health Standards and Quality Bureau of HCFA, DHHS, is the Federal level organization that provides general guidance and technical assistance to the locally controlled and operated PSROs.

SOURCE Office of Technology Assessment.

Figure 2.—Department of Health and Human Semices-Organizational Components Involved in Medical Technology



NOTE: See app. G for acronyms.

SOURCE: Office of Technology Assessment

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Reimbursement

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5
Reimbursement

INTRODUCTION AND BACKGROUND

Medicare and Blue Cross/Blue Shield, the largest government and private health insurance programs, respectively, pay for a substantial share of the Nation's total health care outlay. In 1978, for example, medicare financed 24 percent of all hospital care and 16 percent of all physicians' services. In fact, its payments represented more than 15 percent of all personal medical expenditures in the United States. In the same year, Blue Cross/Blue Shield paid for nearly 11.5 percent of all personal health care expenditures **(84).** Through financing, both programs can affect the rates at which new technologies are developed, diffused, and utilized, and at which inefficacious, outmoded, and unsafe services are phased out.

This chapter focuses on the medicare program and its reimbursement coverage process. Also included is a discussion of Blue Cross and Blue Shield and its reimbursement coverage process. The latter discussion is included because the national Blue Cross and Blue Shield Associations and the affiliated plans (the actual administrative units, who are relatively autonomous and who receive guidance but not mandatory procedures from the national associations) represent the largest nongovernmental third-party payer in the Nation and because their processes of deciding on coverage of medical technologies for reimbursement are similar to medicare's.

Medicare is a nationwide, federally administered health insurance program authorized in 1965. It provides benefits for people over age 65, for certain individuals eligible for disability payments, and for certain individuals who need kidney transplantation or dialysis. The medicaid program is a Federal program that is administered individually by each participating State government. Each State can use its own procedures for coverage decisions. Although medicaid is not covered in this chapter, in concept, many of the arguments presented would apply equally well to that program.

THE MEDICARE COVERAGE PROCESS

Section 1962 of the Social Security Act mandates that medicare shall pay only for medical services that are "reasonable and necessary" for diagnosis, treatment, or improved functioning. [1] By granting Government officials the authority to determine which new and existing services[2] are eligible for reimbursement, this section of the law involves medicare in technology decisions. Medicare has refrained from establishing a definitive interpretation of the "reasonable and necessary" language of the Social Security Act and relied on a loosely structured and decentralized mechanism to provide coverage guidelines.

The medicare program is administered in two parts: part A, financed by payroll taxes, provides reimbursement for services in hospitals, extended care facilities, and other institutions; and part B, a voluntary supplementary program, pays for physicians' services. Under the present coverage mechanism, the contractors who administer part A, fiscal intermediaries, and part B, carriers, of medicare bear the initial responsibility for identifying coverage

'Sec. 1862.(a) Notwithstanding any other provisions of this title, no payment may be made under part A or part B for any expenses incurred for items or services (1) which are not reasonable and necessary for the diagnosis or treatment of illness or injury or to improve the functioning of a malformed body member.

'The Social Security Act a I so specifically excludes certain services from payment eligibility.

issues and determining reimbursement policy.[3] Through the advice provided by their medical advisors, contractors make coverage determinations about the majority of new services that they identify (98).

When the contractors feel unable to make a judgment on a particular coverage issue, they submit the issue to one of 10 medicare regional offices. As a rule, referrals are made by carriers, who process physicians' bills, rather than by fiscal intermediaries, who process hospital bills. This is because under the cost reimbursement system, hospital bills generally are not examined for questions about the use of a particular technology. In fact, most hospital billing forms provide little specific information about the various elements of service (398,574). Although intermediaries, as well as hospitals, physicians, and the manufacturers of drugs and devices, may occasionally raise a coverage issue, carriers usually perform this function.

Coverage decisions by both contractors (239) and regional offices (398) appear to be based primarily on two related criteria: 1) the technology's stage of development, and **2)** its general acceptance. If a new technology is perceived to have moved beyond experimental status toward full clinical application and to be accepted by the local medical community, then it is deemed "reasonable and necessary. " These criteria, however, lack precise standards, and the contractors' procedures for considering them tend to be informal and highly variable. Thus, for example, a medical advisor may base a decision on immediate personal knowledge of a technology's stage of development and acceptance or may survey the literature and seek out the opinions of other medical consultants, local specialty society representatives, advocates of the procedure, and the advisors of other area insurance programs.

In addition, both contractors and medicare regional offices appear to show considerable variation in the priority they accord to coverage questions and their approach to handling them. Some regional offices will attempt to resolve many of the issues referred to them by seeking out the opinions of local contractors; others will tend to transmit such issues directly to the medicare central office (398,291). Similarly, within a region, some carriers will display far more initiative than others in identifying a coverage issue, pursuing information about it, and making a decision (398). As a result, the specific package of benefits for which medicare will provide reimbursement varies somewhat across the country, and there is no national standard for covered services.

When the contractor and medicare regional office are unable to resolve an issue, it is referred to the Health Care Financing Administration (HCFA).[4] This agency, in turn, may request a coverage recommendation[5] from the Public Health Service (PHS).[6] Historically, PHS generated its responses through an informal and loosely structured procedure. Typically, one, two, or three professional PHS staff members assigned to the task researched a coverage question by attempting to survey the relevant medical literature and consult with appropriate experts. This process has been inherently unsystematic, because the small PHS staff has lacked the benefit of established channels of communications to medical specialty groups and to other PHS agencies such as the National Institutes of Health (NIH) and the Food and Drug Administration (FDA) (98).

Within this ad hoc framework, PHS has traditionally applied four criteria to coverage recom-

[1] Medicare contractors for part A deal with hospital services and contractors for part B deal with physicians' services.

[4] Reimbursement coverage questions are referred to the Health Standards and Quality Bureau (HSQB) in HCFA. Prior to the creation of HCFA, they were referred to medicare's Division of Provider and Reimbursement Policy.

[5] In 1977, the Administrator of HCFA and the Assistant Secretary of Health entered into an agreement which formalized the Public Health Service's role in providing coverage recommendations. Since the late 1960's, the medicare program's lack of medical advisors has resulted in an almost automatic referral of coverage questions to PHS, although occasionally HCFA has tried to group related issues together or resolve a matter which seems to warrant little attention because it has either been previously raised and answered by PHS or it concerns an apparently worthless service (e. g., colonic irrigation) or a well-established and accepted one.

[6] The current PHS unit providing coverage recommendations is the National Center for Health Care Technology (NCHCT). The Center was created by Public Law 95-623 in November 1978. Before the formation of NCHCT, the Office of Health Practice Assessment bore this responsibility. Previously, it was the function of the Bureau of Quality Assurance in the Health Services Administration.

mendations: 1) safety, 2) efficacy, 3) stage of development, and 4) acceptance by the medical community. Although, these criteria have recently been made more explicit[7] through a PHS staff paper on coverage policy,[8] they still lack precise definition.

PHS recommendations generally have not attempted to recommend specific indications for use of technologies. The PHS staff have maintained that coverage policies should be expressed in broad terms and questions of appropriate usage for a set of specific patient indications should be addressed by the practicing medical community and the Professional Standards Review Organizations. Thus, a PHS description of its coverage policy notes (98):

> The coverage system cannot attempt to be an encyclopedic listing of which interventions to use in which circumstances nor serve as a kind of substitute for medical education and clinical training. Rather, the coverage system uses broad strokes to sketch the boundaries of accepted good medical practice, and leaves the fine-tuning of the system to the "back-end" mechanism, the Professional Standards Review Organizations (PSROS).

Recently, however, HCFA has shown some reluctance in accepting PHS recommendations and sought to issue guidelines which cite indications for use more often.

After the PHS staff develop a coverage recommendation, they send a memorandum to HCFA'S Health Standards and Quality Bureau (HSQB). This Bureau has usually accepted the policy suggested by PHS and has issued appropriate guidelines to medicare regional offices and contractors (282). Because of the large volume of services involved, policy implementation depends mainly on the good faith of providers and on the threat of potential audits.

Cost and Efficacy

Although the cost of medical technology has focused attention on reimbursement, cost information has not been explicitly or directly considered in medicare coverage determinations. In a few cases, where PHS has concluded that a new technology is a modification of an existing intervention and offers little additional benefit, it has favored coverage but recommended payment at the same level as that for the established service. This type of judgment represents a weighing of marginal cost and marginal value, so it might be said that PHS has conducted implicit cost-effectiveness analyses (CEAS). The practice of paying usual and customary fees, however, does not easily accommodate such anal yses.

Unlike cost, efficacy is one of the four criteria PHS has traditionally applied to coverage questions. Several recent PHS procedural changes have promoted more systematic examination of efficacy data: 1) the development of communication ties with NIH, FDA, and medical specialty societies; 2) the formal linking of FDA drug and medical device policies to PHS coverage determinations (98); and **3)** the setting of a precedent in the computed tomography (CT) body scan decision to restrict coverage to uses that are supported by current evidence of efficacy (282), although the list of indications for those scans appears to include nearly every possible use (389).

The extent to which these procedural changes will ensure a greater emphasis on efficacy remains uncertain. Despite the CT body scan recommendation, PHS has generally not attempted to specify indications for use, contending it would do so only in the consideration of drugs or in an exceptional case involving a high-risk or a high-cost technology. Although it is committed to reflecting FDA's safety and efficacy rulings by following FDA's decisions on ap-

[7]Historically, these criteria have been developed and utilized implicitly (33).

[8]A PHS staff paper provides the elaboration of these terms (98):

1. Safety: What is the likelihood and severity of risk associated with the technology?
2. Efficacy: What is the likelihood that the technology will produce the desired health benefit? No distinction is drawn here between efficacy and effectiveness, although in other contexts, "efficacy" is used to mean the probability that a modality will produce a desired outcome under ideal circumstances, while "effectiveness" is used to mean that the desired outcome will result under average clinical conditions. The term "efficacy" as used here is intended to convey a hybrid of both meanings, since the data base for coverage decisions is sometimes derived from laboratory or clinical trial environments and sometimes from routine practice environments.
3. Stage of development: Has the technology moved beyond the experimental stage to full clinical application?
4. Acceptance by the medical community.

proved and contraindicated drug use, PHS exercises discretion in considering uses for indications on which FDA has not ruled. Further, drug coverage questions are not usually raised, because medicare only pays for the use of drugs in hospitals, and it is difficult for fiscal intermediaries to identify individual hospital service components.[9] Questions about medical equipment are far more typical, but the impact of relating coverage recommendations to FDA determinations is unclear, because the Medical Devices Amendments of 1976 are still in the process of being implemented (see ch. **8).** Perhaps more importantly, adequate evidence of the efficacy and safety of devices and procedures is often not available (465), and in the absence of such evidence, technologies judged to be nonexperimental and accepted usually have received a positive coverage recommendation from PHS.

Application of efficacy criteria also is limited by the selected number of coverage questions that reach the PHS agenda.l[10] The current system, in which issues may be identified by fiscal intermediaries when they process hospitals' bills and by carriers when they process physicians' bills, is basically reactive. Identification of new services can be difficult, however, because information may be lost or obscured not only by classification into service code categories, but also by translation from provider to intermediary codes, although the tendency to attach a higher cost to a new technology serves as a major screening aid (399,569). Helen Smits, the Director of HCFA'S HSQB has observed (575):

> A consistent method of coding diagnoses and procedures, consistently applied, is essential to any real "capture" of new technologies by reimbursement . . . Even when a single procedural code has been agreed upon and put into use, problems on variable coding are Iikely to persist and to make accurate application of coverage decisions very difficult.

Identification of a new hospital service is particularly problematic. This is because, as noted above, fiscal intermediaries generally do not examine hospital bills for coverage questions and the majority of billing forms[11] provide little information about the use of particular technology. Certificate-of-need requirements, end of the year or prospective reimbursement negotiation, and informal discussions between intermediaries and the hospitals in their area appear to alleviate this problem only partially.

Another way that a coverage issue may be raised is through the interaction of intermediaries and hospital administrators or staff when the hospital is planning to acquire or to offer a new service. Identification of large-scale, discrete, and potentially expensive technologies may be accomplished more easily through this type of interaction than it is through billings. Neither the extent nor the potential usefulness of such an identification method, however, was analyzed by OTA.

Even when a new service is identified, the coverage decision often, perhaps usually, will be made by the contractor or intermediary and will not be brought to the attention of HCFA or PHS unless a negative determination is legally challenged by a physician, hospital, or patient. For example, PHS has never been asked to make a judgment about the coverage of coronary artery bypass grafting (470), an expensive and widely performed procedure. In addition, the PHS coverage agenda is severely circumscribed by an almost exclusive emphasis on new technology. The one major exception to this pattern occurred in **1977,** when PHS issued recommendations about **28** established procedures after Blue Shield had concluded they were outmoded or ineffective and should be excluded from routine reimbursement payments (431,432,433).

Currently, however, both HCFA and PHS are studying a number of possible changes in the medicare coverage process that may increase the

[9] It is also extremely difficult to monitor drug use for approved indications.

[10] While judgments made by contractors and the regional office about acceptance and stage of development may be influenced by efficacy data, the focus appears to be more directly on stage of development and acceptance.

[11] Because of the diversity of elements involved in surgical procedures, identification of a "new" surgical procedure may be particularly difficult. For example, David Eddy notes, "In surgery, it can be very difficult to identify when a procedure is "new" or sufficiently different from other procedures to require a new evaluation . . frequently a procedure can be described only as a set of maneuvers, and these maneuvers can change in subtle but important ways" (167).

weight given to efficacy data, as well as formally introduce cost criteria. HCFA is examining several possible actions: utilizing cost as a coverage criterion, implementing regulations that would formally define the "reasonable and necessary" language of the Social Security Act, [12] establishing a uniform service code, [13] and issuing more guidelines which relate coverage to appropriate indications for use. [14] At the same time, the new PHS coverage-recommending unit, the National Center for Health Care Technology (NCHCT) is considering utilizing three additional coverage criteria: conformity to health planning guidelines, relative efficacy, and cost effectiveness. Reservations have been raised about the introduction of the latter two criteria, however, because of methodological difficulties in measurement. As a result, the PHS coverage staff are currently awaiting the results of NCHCT'S experience with applying CEA in its comprehensive assessment activities. According to a PHS staff paper (98):

> The chief difficulty is how to measure relative efficacy and cost-effectiveness in an operational way. If one is comparing the relative efficacy of two modalities, how much more efficacious must the more expensive or more risky technology be in order to be "worth" the added cost or risk? The techniques for comparing relative efficacy and determining cost-effectiveness are less reliable than the methods for assessing safety and efficacy, and our experience in using the outputs of relative efficacy and cost-effectiveness studies as a basis for policy decisions is *very* weak. It would appear that some additional developmental work is needed before relative efficacy and cost-effectiveness can be applied routinely as criteria for coverage recommendations. One way to demonstrate and test the application of these two criteria would be to use them in the course of the large-scale evaluations to which NCHCT will subject the high priority technologies. If the "bugs" appear to have been worked out in the mega-assessments, consideration would then be given to applying relative efficacy and cost-effectiveness more routinely in coverage decisions.

Coverage Reevaluation

Cost, safety, efficacy, and legal concerns have all contributed to the current reevaluation of medicare coverage decisions. Most important, perhaps, the rising cost of medical care has confronted policy makers with the need to contain health expenditures and rationally allocate resources, Increases in health care expenditures associated with both the enactment of medicare and the rapid diffusion and use of technology have generated additional interest in creating cost control mechanisms.

Although the overall impact of technological innovation on health care spending is unclear (17), it is apparent that economic incentives strongly favor the spread of technology (196, 528). Because medicare reimburses retrospectively, it provides an open-ended commitment to pay for covered services. Under medicare and some other third-party insurance, hospitals (the most expensive element of the health care system) are reimbursed on the basis of costs; physicians are reimbursed on the basis of charges; and patients are partly insulated from immediate actual costs. As a result, services may be utilized even when patient outcome benefits are marginal or uncertain (468).

The tendency toward utilization of services is also encouraged by other factors: competition among hospitals to achieve quality and prestige and attract patients and physicians; public demand for sophisticated technology; practitioners' desire to do the most possible for their patients and to achieve a high degree of certainty in their judgments so as to avoid malpractice suits; physician specialization; and the stress on ancillary services (470,546).

Spiraling medical expenditures and their association with a reimbursement system that promotes technology development and use has encouraged Government officials to reevaluate medicare's traditional social insurance orienta-

[12] The Office of the General Counsel has been asked to determine whether the "reasonable and necessary" language of sec. 1862 and the reference to "reasonable cost" in other parts of the legislation permit introduction of cost criteria into coverage decisions (292).

[13] HCFA's Division of Provider and Reimbursement Policy has drawn up a set of draft regulations defining "reasonable and necessary," and the Office of Policy Planning and Research also is in the process of performing the same task.

[14] A uniform code also may be used to produce a list of services that are covered (399).

[15] In recent months, HCFA has shown greater interest in issuing guidelines that cite indications for use.

tion. At the time of the enactment of the legislation in 1965,[16] and in the years since, there has been an emphasis on accommodation with the existing health care system (191). 1[7] This theme is reflected in the nature of the present coverage mechanism: the heavy reliance on fiscal intermediaries and carriers, the failure to formally define the "reasonable and necessary" language of section 1862, and the tendency for PHS to react favorably to the majority of coverage questions it has addressed. But as cost containment becomes an increasingly important objective,[a] interest in medicare's becoming a more selective purchaser of health services is rising (34). Within this changing perspective, cost-effectiveness information *would seem to* possess substantial appeal.

Reexamination of the medicare coverage system also stems from a growing awareness that technological innovation and health status do not always seem to be directly related and that the safety and efficacy of many technologies have not been adequately evaluated **(238).** There is interest in protecting patients from risky, ineffective, or unproven services. The result has been that coverage decisions are becoming more closely tied to available safety and efficacy information.

In addition, legal requirements have spurred PHS and HCFA review of the current coverage process. As the likelihood of more coverage denials increases, there is a greater need to establish a firmer legal basis for decisions (291,574).

"The Social Security Act states in sec. *1801:*

> Nothing in this title shall be construed to authorize any federal officer or employee to exercise any supervision or control over the practice of medicine or the manner in which medical service> are provialed

For a political history of medicare, see R. Harris, *A Sacred Trust (278);* T. H. Marmor and J. Marmor, *The Poltics of Medicine (381),* and O. W. Anderson, *The Uneasy Equilibrium. Private and Public Financing of Health Services in theUnited States, "Uneasy, ' 1875-1965 (25)* .

[17] 0f course, the medicare legislation encouraged cost containment through physician self-regulation by originally establishing hospital utilization review committees in 1965 and later creating PSROS in 1972.

[a] "Feder suggests that reorganization was necessary for a shift in focus from social insurance to health policy (191). Several HCFA officials who were interviewed possessed a similar perspective.

THE BLUE CROSS/BLUE SHIELD COVERAGE PROCESS

Cost considerations, along with safety and efficacy and legal concerns, in addition to leading medicare to reevaluate its coverage policies, have led nongovernmental insurers to reevaluate their coverage policies. Traditionally, these insurers' interest in escalating health care costs has been moderated by their ability to respond by raising insurance premiums. But as the purchasers of health insurance—management, labor unions, and individual subscribers—have become more resistant to higher premiums **(470),** third-party payers have been increasingly confronted with the need to contain health care costs and become more selective purchasers of health services,

Blue Cross and Blue Shield, the largest non-Government insurers, with over 40 percent of the Nation's subscribers, have called for cost containment initiatives since the early 1970's. Over the years, they have pursued a wide range of policies, including utilization review of length of stay and level of care, health planning, benefit package designs that emphasize low-cost options (such as outpatient surgery), alternative delivery systems, consumer education, and coverage reevaluation (415). Like medicare, however, Blue Cross and Blue Shield traditionally have refrained from issuing many negative coverage guidelines. In part, this probably arises from a historical social insurance orientation, as well as Blue Cross and Blue Shield's desire to: 1) remain competitive with commercial insurers, 2) fulfill the expectations of beneficiaries, hospitals, and physicians that services will be covered by insurance policies, and 3) forestall legal challenges that might result from denial of payment.

Blue Cross and Blue Shield's treatment of coverage issues parallels medicare's in many respects. Like medicare, Blue Cross and Blue Shield make the majority of coverage decisions through a decentralized and loosely structured process that places key emphasis on two cover-

age criteria: stage of development and acceptance by the medical community. Blue Cross and Blue Shield also lack a uniform national benefit package; interpretations of what qualifies as a covered service vary somewhat throughout the country. 19

Most individual Blue Cross and Blue Shield plan contracts exclude "care which is not a part of generally accepted medical practice" and "unnecessary or inappropriate care, " although the specific wording employed in different contracts varies (255). These criteria lack precise definition, however, so the medical advisors and their staffs possess considerable discretion in applying them, although coverage questions that are viewed as particularly important may be brought to the attention of a plan's board of directors. Medical advisors may make judgments based on immediate personal knowledge or may survey the literature and consult with advocates of the procedure, local specialty societies, the county medical society, and other area insurers. If the plan functions as an intermediary for medicare or an agent for medicaid or the armed services CHAMPUS program, the medical advisor will generally review the coverage determinations of these programs. Similarly, the medical advisor will usually study the coverage recommendations of the national Blue Cross and Blue Shield Associations and other member plans, although he or she may or may not follow any previous rulings.

The national Blue Cross and Blue Shield Associations are a federation of 137 State and local nonprofit Blue Cross and Blue Shield affiliates. Power to make policy ultimately lies with the individual plans, but the national organization exercises considerable leverage through the prominence of its leadership and the ability to serve as a public spokesman for member plans (141,255,415). The national associations' coverage recommendations are made by the Medical Necessity Program and the Medical Advisory Committee.

The Medical Necessity Program, developed in 1977 in conjunction with the American College of Physicians, the American College of Surgeons, and the American College of Radiology, was designed to curtail reimbursement for outmoded, duplicative, or unproven procedures. According to a 1977 Blue Shield bulletin (433):

> The Program is an effort to align the objectives of several national professional medical societies and of Blue Shield in improving the quality of care while simultaneously reducing the costs of procedures that are, in many instances, outmoded, redundant in combination with others, unlikely to yield additional information through repetition, or of unproven value.

Since the inception of this program, the Blue Cross and Blue Shield Associations have recommended discontinuation of routine payment for 68 surgical and diagnostic procedures and hospital admission battery tests (58,59,140,431, 432,433). While physicians may still order these procedures, they must provide written justification for their use in order to be reimbursed by plans that adopt the medical necessity proposals. Most plans appear to be accepting these recommendations, and it has been estimated that full implementation may result in annual savings of as much as $200 million from limiting routine hospital admissions tests and as much as $100 million from curtailing the use of 68 surgical and diagnostic procedures. [20]

Moreover, the national Blue Cross and Blue Shield Associations make over 100 coverage recommendations a year through the deliberations of their Medical Advisory Committee. With the advice of medical specialty groups, the Medical Advisory Committee issues suggested coverage guidelines. Usually these recommendations concern new technology and are expressed in broad terms, but sometimes they specify indications for use. In addition, the committee serves as a clearinghouse for information about the coverage policies of each of the Blue Cross and Blue Shield plans.

The national Blue Cross and Blue Shield Associations consider only a limited number of coverage questions. Medical specialty group

'"While benetit package coverage variation allows responsiveness to local patterns of practice, it also raises the issue of equity.

In some areas where Blue Cross and Blue Shield plans are incorporated together, they share the same medical advisory staff; in other areas, they operate as separate organizations with separate staffs.

representatives suggest which established procedures should be examined by the Medical Necessity Program, and the Medical Advisory Committee's agenda is largely determined by questions raised by plans when they seek the national associations advice.

Cost and Efficacy

Blue Cross and Blue Shield, like medicare, tend not to consider cost information directly in coverage deliberations. In a few cases, where a new procedure has been identified as a modification of an existing service which offers little additional benefit, however, payment for the new procedure has been fixed at the same level as that for the established technology. In addition, cost has traditionally been one of the factors examined in benefit package design. Recently, CEA was used in the development of a screening program when the National Cancer Institute contracted with the Blue Cross and Blue Shield Associations in 1976 to develop **a** model prepaid health service benefit package for cancer screening. Although the contents of this package originally were expected to be determined by the consensus decisions of a panel of experts, the program that eventually was designed was heavily influenced by the results of a CEA of cancer screening submitted by one of the consultants hired for the project (168,434). Moreover, the creation of the Medical Necessity Program has introduced an approximation of cost-effectiveness methodology in Blue Cross and Blue Shield coverage decisions, because cost consciousness has encouraged coverage determinations that are more closely related to judgments made by the national medical specialty societies.

Further, greater emphasis on efficacy data also has been promoted by Blue Cross and Blue Shield's sponsorship of an Institute of Medicine study of the CT scanner and subsequent recommendation that coverage be restricted to uses that the Institute found to be supported by current evidence of efficacy. Yet, the extent to which these changes indicate that more coverage decisions will be tied to efficacy information remains uncertain. Adequate evidence of the efficacy and safety of medical technologies often is not available, and in the absence of such evidence, technologies judged to be nonexperimental and generally accepted usually receive positive coverage recommendations from both the plans and the national associations.

Currently, however, Blue Cross and Blue Shield are studying contract and coding changes that ultimately may facilitate the application of cost and efficacy criteria. Although Blue Cross and Blue Shield have their own procedure code, plans are mandated to use it only when they deal with national account business (group accounts in which some individuals live beyond the boundaries of an individual plan); other use is discretionary and many local affiliates choose to use other codes for their general subscribers. At present, however, the national associations are attempting to revise and update the code, a move which may make it more attractive to member plans and more useful for the identification of questions concerning coverage issues or inappropriate use of a technology. Moreover, the Blue Cross and Blue Shield Associations are considering inserting new exclusionary language in their national account contract and recommending a model contract exclusion clause to its local affiliates in order to develop a firmer legal basis for coverage decision. [21] In recognition of this need, many plans have taken the initiative in recent years and developed more

[21] Thus, the Blue Cross Board of Directors and the Blue Shield Board of Directors will be asked to review and approve the following policy:

To recommend to the Boards that each Plan be encouraged to re-examine its contracts to assure adequate identification of medical necessity and experimental exclusions, and that the Boards offer the following language to each Plan for consideration.

"Medically necessary services or supplies provided by hospital, physician or other provider to identify or treat an illness or injury and which, as determined by the Plan, are:

1) Consistent with the symptom or diagnosis and treatment of the condition, disease, ailment or injury;
2) Appropriate with regard to standards of good medical practice;
3) Not primarily for the convenience of the member, his physician, or other provider;
4) The most appropriate supply or level of service which can safely be provided to the member. When applied to an inpatient, it further means that the member's medical symptoms or condition require that the services or supplies cannot be safely provided to the member as an outpatient.

Services and supplies which are *experimental or investigational* in nature; meaning any treatment, procedure, facility, equipment, drugs, drug usage, devices, or supplies not recognized as accepted medical practice or by the Plan and any of such items requiring federal or other governmental agency approval not granted at the time services were rendered."

specific exclusionary language for their contracts. [zz]

A few plans in California, Pennsylvania, Michigan, Massachusetts, and New York also appear to have taken the initiative in developing a somewhat more systematic coverage mechanism. Perhaps the most sophisticated decisionmaking process has been adopted by California Blue Shield, which addresses a series of questions before making a coverage determination:

1. Is the procedure experimental or investigational?
2. Is it generally accepted?
3. What is its relative safety?
4. What does it cost?
5. Is there a procedure which costs less and achieves the same result?
6. Is it reproducible, i.e, can it be used by someone other than the original creator?
7. Is it generally available?
8. Does it make a difference in the management of patient outcomes or does it just serve an academic purpose?

Although stage of development and general acceptance remain the most important criteria, California Blue Shield has succeeded in formally placing the concepts of cost and relative efficacy on its coverage agenda. In a few cases, where it has denied routine payment for a new procedure that it has determined costs more and achieves the same result as an existing technology, California Blue Shield has applied an approximation of CEA to its decisionmaking. Moreover, ultimate authority to make coverage determinations for the plan lies with the Medical Policy Committee, a 24-member group composed of physicians and 4 or 5 public representatives, which meets in a number of locations across the State and invites representatives of the local medical community and the appropriate specialty societies to attend its sessions. At least 2 or 3 weeks prior to the time the Medical Policy Committee meets, the medical advisor distributes an agenda book containing all the information that has been collected about the procedures to be discussed. As a result, the Blue Shield coverage has achieved a high degree of acceptance within the California medical community, and the meetings of the Medical Advisory Committee often serve as a public forum for the generation of consensus about a procedure. Thus, even when a decision is made not to restrict payment for a technology, the public discussion of its merits may result in discouraging its use. Further, the high visibility of the Blue Shield coverage process in California has augmented the State plan's ability to identify coverage issues, because the developers of new procedures tend to seek the advice of the Medical Advisory Committee before requesting reimbursement (165,537).

[zz]For example, Washington, D.C., Blue Cross/Blue Shield issued the following statement in 1979 (59):

> Group Hospitalization, Inc., and Medical Services of D.C. have always been denied claims for hospital and medical services or supplies which are not medically necessary. We're now adding a contractual provision to all non-group contracts which specifically excludes services or supplies which are not, in the judgment of the corporation, medically necessary for the diagnosis or treatment of an illness.

POTENTIAL USE OF CEA/CBA IN REIMBURSEMENT COVERAGE DECISIONS: GENERAL FINDINGS

Cost-effectiveness analysis/cost-benefit analysis (CEA/CBA) is based on the assumption that resources are limited and should be rationally allocated. Because it makes this assumption of limits and because it forces explicit consideration of alternatives for achieving goals, CEA/CBA would appear to be able to contribute important information to decisionmakers concerned with acting as selective purchasers of health services. Yet its appeal for reimbursement officials seems severely tempered by the small number of well-conducted CEA/CBA studies available, methodological uncertainties of the techniques, and health policy makers' limited experience with their use. Moreover, the economic efficiency value embodied in cost-effectiveness information may conflict with a number of other values prevalent in our health

care system, including: 1) the practitioner's obligation to do the most for the patient, desire for relative certainty in making a diagnosis, and need to possess the freedom and flexibility to respond to the circumstances of the individual medical problem; 2) the patient's desire to receive a full range of medical care, regardless of ability to pay; 3) society's desire to encourage innovation in order to ultimately improve care; and 4) society's goals in terms of equity and other noneconomic values.

On the other hand, the methodology for efficacy and safety analysis is more widely accepted than that for CEA.[23] Efficacy and safety studies form part of a relatively more established health policy tradition, and the value of protecting patients from risky, unsafe, or unproven technology is less controversial (465) than weighing marginal cost and benefit tradeoffs and not reimbursing for some potentially beneficial forms of care. For this reason, an approximation of the cost-effectiveness concept, in which cost-containment goals encourage stricter adherence to safety and efficacy data might be more appropriate and acceptable in reimbursement coverage decisions than would formal CEA/CBA, One of the problems with this approach, however, is that most technologies probably have some efficacy in some circumstances, and rational allocation requires choosing among alternative uses of technologies by considering marginal costs and marginal benefits and relative efficacy for a specific set of patient indications.

[23] For a discussion of the conflict of medical economic efficiency with efficacy, see W.B. Schwartz and P.L. Joskow, "Medical Efficacy Versus Economic Efficiency: A Conflict in Values," *N. Eng. J. Med.*, 1978 (548).

COST EFFECTIVENESS IN RATESETTING

In the last decade, the number of hospital ratesetting programs has increased rapidly. The central feature of this regulatory mechanism is the negotiation of hospital rates in advance of each operating year. Thus, Hellinger points out (294):

> The key difference between prospective rate setting and conventional methods of reimbursement is that hospitals are not paid the costs they naturally incur, nor are they free to unilaterally adjust their charges to cover their costs or their own interpretations of their financial requirements; rather, they are paid at rates that are determined by another body and that are set in advance of, and considered fixed for, the prospective year.

Various ratesetting mechanisms, however, differ considerably in their structure and operations and in the methods and unit of payment they use to determine rates. For example, many programs have the power to set mandatory rates, whereas others rely on voluntary compliance. More than 20 programs are sponsored by Blue Cross, while 12 programs are funded by State governments, either through existing State agencies or newly formed independent commissions (154, 155,294).

Concern with the rapidly rising cost of health care has encouraged the growth of rate review mechanisms. In fact, Federal legislation has directly contributed to their development. Both the 1972 amendments to the Social Security Act (Public Law 92-603) and the 1974 National Health Planning and Resources Development Act (Public Law 93-641) provide for Federal funding of experiments in ratesetting. Moreover, escalating medicaid expenditures have prompted an increasing number of States to consider establishing a rate review system.

Basically, ratesetting agencies address the need to promote cost containment by encouraging hospitals to operate efficiently. By determining in advance the amount of revenue that will be available, they reward hospitals that keep actual costs below the established rates. Thus, Bauer points out (45):

> The advantages seemed obvious; if a hospital could know its payment rate before it rendered its services, it would have the highest possible motivation to see that these services were produced in the most efficient manner, since its solvency would depend on keeping its spending within the limits of its anticipated revenues. The

hospital would have positive incentives for efficiency as well, since if it could produce its service more cheaply than the predetermined rate had allowed, it could pocket the difference.

In effect, then, the intent of rate review is to promote more efficient behavior by forcing hospitals to live within a fixed budget, identify and anticipate the costs of services and facilities, and make explicit decisions about the allocation of resources.

The record of ratesetting agencies thus far, however, is inconclusive (45,293,294). One significant limitation is that the lack of adequate data, performance standards, and methodology makes it very difficult to set rates of payment that promote efficiency. As Bauer notes, "The central issue is how to set rates in a manner that will neither underpay nor overpay, but will encourage each institution to increase the efficiency with which its services are provided" (45). Still another major problem is that ratesetting programs tend to focus on hospital operating costs and do not concentrate on such other important areas as the costs generated by physicians.

Thus, it appears that use of CEA/CBA in rate review systems will have to await the development of more sophisticated ratesetting methods, more adequate data, and performance standards, and closer liaison with other organizations, such as health systems agencies. It may be that analysis concentrating on the net-cost end of the spectrum of analysis (e. g., cost per unit of service) is more appropriate for ratesetting. If so, ratesetting may be an area where increased numbers of CEA/CBA-like studies could be used. An interesting counterargument is that ratesetting could be based on the results of society-based CEA/CBAs and thereby would be less oriented to a narrower efficiency base and more toward social effectiveness.

6.
Professional Standards Review Organizations

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6
Professional Standard; Review Organizations

INTRODUCTION AND BACKGROUND

As noted in chapter 5, the Social Security Act authorizes the medicare program to pay only for services that are "reasonable and necessary," and these criteria have been generally regarded as met when a new technology is perceived to have moved beyond experimental status toward full clinical application and acceptance by the local medical community. The current policy of the Department of Health and Human Services (DHHS) is to use "broad strokes to sketch the boundaries of accepted good medical practice, and (to leave) the fine-tuning of the system to the 'back-end' mechanism, the Professional Standards Review Organizations (PSROS)" (98).

The PSRO program is one of the principal expressions of Federal policy concerning the use of medical services. The PSRO program, established in 1972 by Public Law 92-603, is administered by the Health Standards and Quality Bureau (HSQB), formerly the Bureau of Quality Assurance, of the Health Care Financing Administration. The purposes of the program are to help improve the quality and control the costs of medical services reimbursed through Federal payment programs. The program operates by setting standards and criteria for the desired level and quality of medical services and by evaluating against these standards the services actually provided. This process is designed to ensure that payment will be made only when services are medically necessary.

The PSRO program is based on the concept that medical professionals are the most appropriate individuals to evaluate the quality of medical services and that effective peer review at the local level is the soundest method for ensuring the appropriate use of medical care resources and facilities. The PSRO program is made up of separate and independent organizations covering 195 geographic areas. Each PSRO must be substantially representative of all practicing physicians in an area. The PSRO program is new and is not yet fully implemented. Of the 203 PSRO areas in March 1977, only 120 PSRO agencies had been funded; 100 were in "conditional" status; 20 were in "planning" status. By April 1979, the areas had been consolidated to number 195. Of these, 182 had PSROS in "conditional" status, and 13 were being planned or were inactive.

Currently, PSROS are required to review institutional care—care provided in hospitals and long-term care (nursing home) facilities—and are authorized to regulate ambulatory care. PSRO activities at present are concentrated on inpatient care provided in short-stay hospitals. The PSRO can delegate its review responsibilities to the hospitals, or, if a hospital is not qualified or not willing to conduct the reviews, the PSRO conducts them itself. Review of long-term care can be delegated if such care is a "distinct part" of a short-stay hospital.

PSROS conduct three types of review (42 CFR, part 466):

1. **concurrent review** of the medical necessity and appropriateness of admission to and continued stay in a hospital;
2. medical care evaluation studies to assure the quality and improve the nature of the utilization of health care services; and
3. analyses of health care practitioner, institutional, and patient profiles.

Medical care evaluation studies and profile analyses are used to focus concurrent review on problem areas.

As specified in the statute, PSROS review services to determine whether:

- such services and items are or were medically necessary;
- the quality of such services meets professionally recognized standards of health care; and
- such services and items proposed to be provided in a hospital or other health care facility on an inpatient basis could, consistent with the provision of appropriate medical care, be effectively provided on an outpatient basis or more economically in an inpatient health care facility of a different type.

The law requires that PSROS use norms, criteria, and standards in evaluating medical services. This approach allows nonphysicians to perform many of the reviews and also enhances the objectivity of the review process. Standards are developed by a consensus of physicians, based on typical patterns of practice in the area and on such regional or national information as may be available and considered applicable by the PSRO.

In its early stages, the PSRO program has concentrated on determining the need for hospitalization. Now PSROS are beginning to move beyond the question of necessity for hospitalization to review of surgical procedures and review of ancillary services.

PSRO decisions on medical care utilization and quality can be enforced in several ways. Reimbursement for services provided can be withheld by medicare and medicaid (medicaid regulations are established in each State and vary somewhat). For serious and repeated violations of PSRO standards, a physician's right to be reimbursed through medicare and medicaid can be suspended or revoked.

Each State with three or more PSROS has a statewide Professional Standards Review Council. Among other duties, the statewide councils have the responsibility to disseminate information and data among the PSROS within the State. At the national level, a National Professional Standards Review Council is established by law. This Council has several functions, one being to "provide for the development and distribution, among Statewide Professional Standards Review Councils and Professional Standards Review Organizations of information and data which will assist such review councils and organizations in carrying out their duties and functions." Such information is specified as including regional norms and standards. Local PSROS are not required to accept model standards issued by the National Council. However, the National Council has authority to disapprove local standards that deviate from model standards if the Council determines that the differences are not medically justified. The National PSRO Council has provided general guidance and sample criteria sets developed by several organizations, including the American Medical Association, under contract with DHHS. The main purpose of these contracts has been to develop criteria on medical necessity for hospitalization for different disease categories. HSQB hopes that technical assistance and norms and standards will have an important educational effect, as well as a direct effect on practice through reimbursement policy.

REVIEW CRITERIA

Criteria used in evaluating the quality of medical care are usually grouped according to structural, process, and outcome measures. Structural measures assess both the availability of medical care resources (e.g., facilities, equipment, and health care personnel) and the qualitative aspects of medical care personnel (e.g., extent of educational background, specialty board certification); process measures assess the appropriateness of the medical care that has been provided; and outcome measures reflect the effect of medical interventions on patient health status. PSROS, in their reviews, have relied primarily on process measures and, to a lesser extent, on outcome measures.

Generally, research on quality of care assurance and on the application of quality of medical care techniques has been process oriented, assessing the appropriateness of medical care as judged by the medical profession. PSRO concurrent review of admission to and continued stay in hospitals is also process oriented. In practice, moreover, review activities of PSROS have been even more narrowly focused. The cost concerns of the Federal reimbursement program, together with the controversial nature and the uncertain state-of-the-art of reviewing the quality of medical care, have limited reviews to assessment of 1) whether a diagnosed problem justified hospital admission, and 2) whether continued hospitalization was justified after a specified number of days of inpatient care.

Recently, some PSROS have tried out slightly more sophisticated reviews than those based on length-of-stay indicators. Beginning in 1977, the Delmarva Foundation for Medical Care of the eastern shore of Maryland began to use a technique called "care level and timeliness review" (CLTR) to help hospitals find and eliminate medically unnecessary hospital days (358). By retrospective audit, the Delmarva Foundation has evaluated both the timeliness of services and the level of medical care supplied. As alternatives to weekend and night admissions through the emergency room (which contribute to unnecessary hospitalization) and to lack of full laboratory services on weekends (which can lead to extra days of hospitalization until tests have been completed), the Foundation examined "swing beds" with additional staffing for observation of patients to determine whether or not they should be admitted, and expansion of laboratory services from 6 to 7 days. In both cases, the reduction of unnecessary days was not enough to justify the extra costs.

COST-EFFECTIVENESS APPLICATIONS

In exploring the actual and potential applicability of cost-effective analysis/cost-benefit analysis (CEA/CBA) in the review activities of PSROS, it is necessary to delineate several separate concepts. First, PSROS do not normally take a specific medical problem or diagnosis and determine which of several effective treatment approaches is the least costly. Instead, they focus on reducing or eliminating unnecessary inpatient care. For a given diagnosis, PSROS perform their review with reference to standards based on medical opinion that hospitalization for that diagnosis is or is not needed. They also review against standards of how many additional days of hospitalization are needed. The PSRO evaluation does not, systematically at least, extend to a further determination that days of hospitalization beyond the standard (and the medical care provided during this period) are not needed at all or could be just as effectively provided through outpatient visits. With the explicit goal of current PSRO reviews being to minimize unnecessary hospitalization—not to determine whether hospitalization, outpatient care, or no medical care at all is most effective for a given medical problem—PSROS are focused on reducing medical care that is of almost no value to the patient, but for which reimbursement is nevertheless being provided.

Second, although outcome measures, or the effect of medical interventions on patient health status, are often viewed as the best criteria for evaluating the quality of medical care, these have been the most difficult criteria to develop. Hence the reliance on process, or medical opinion, criteria. For most medical problems, however, alternative avenues of treatment—not clear-cut, single choices—have been the rule. Furthermore, the effectiveness of even established treatments is continually under question. Taken together, the use of several methods of treatment for specific medical problems and the often uncertain correlations between specific medical interventions and the patient's health status make the development of additional criteria to decide which interventions should or should not be reimbursed very difficult. Nevertheless, the development of such criteria, especially for many of the common interventions

that are subject to review by PSROS, is not impossible. Because PSROS in effect concentrate on deviant practice by establishing ranges of acceptable behavior, boundaries can be set to allow reasonable divergence based on the possibilities of individual patient response. In concept at least, the ranges of acceptable behavior by institutions or physicians under review could be based on information that incorporates cost or cost-effectiveness-related data. The ranges could also be based on a more, though only slightly, common form of data—efficacy and safety.

These two aspects of the use of medical technologies—efficacy and safety, and cost effectiveness—together define the appropriate use of technologies. The PSRO legislation established a framework by which appropriate use of medical technologies could be evaluated by physicians acting in organized groups rather than as individuals. PSRO'S decisions, however, are still based largely on traditional sources of information, so customary practice patterns, whether appropriate or not, become accepted as standard. The lack of scientifically derived information on indications for use and, especially, on the cost effectiveness of various technological applications hampers the development of **ap**propriate standards. Provided with such information, PSROS could perhaps become a more effective mechanism for evaluating medical care. In its absence, PSROS are developing local standards for medical services based primarily on prevailing patterns of medical practice, with little comparison of costs to benefits.

On the other hand, cost-effectiveness approaches have been used in managing some PSROS and in evaluations of the national PSRO program itself. Even with the current focus on reducing medically unnecessary inpatient days in short-stay hospitals, cost-effectiveness techniques can be used for PSRO management purposes. For example, a PSRO might use analytical techniques to decide which of a number of diagnoses to focus on to reduce, by a targeted number, the medically unnecessary days in a given year.

Incentives to use cost-effectiveness approaches to reduce medically unnecessary bed days already exist and have influenced specific PSROS in choosing among problem areas. The PSROS' administrative budgets are funded by annual Federal grants, and their hospital review activities for medicare, medicaid, and the maternal and child health and crippled children programs' patients are paid from the medicare trust funds (Public Law 94-182). Congress set a limit on the use of fiscal year 1979 medicare trust funds for such hospital reviews (44 F.R. 26770, May 7, 1979).

Each PSRO is evaluated annually with regard to its impact on reducing medically unnecessary hospitalization and must negotiate formal annual "objectives" with the Federal Government. Objectives are stated in terms of "impact" (e.g., "reduce the incidence of medically unnecessary days in PSRO hospitals to 5 percent or less as measured by CLTR").

Two Maryland PSROS, the Delmarva Foundation for Medical Care and the Baltimore City PSRO, illustrate the effect of budget constraints on seeking alternative approaches to reducing unnecessary hospitalization (358). In late 1977, the Delmarva Foundation for Medical Care decided that its own collection of hospital data did not enhance its capability to reduce unnecessary hospitalization or assist materially in identifying problem areas. By deciding to eliminate data collection as a PSRO activity and accepting discharge abstracts prepared by the hospitals, this PSRO was able to reduce the concurrent review cost per discharge from $6.50 to $5.00.

The Delmarva Foundation also changed its concurrent review process in order to increase its efficiency. Nonessential tasks such as coding, norm assignment, and abstracting are no longer performed by reviewers. Review procedures have been simplified to concentrate only on the clinical indications for the need for continued hospitalization. A patient receiving intravenous therapy, for example, is automatically considered to be appropriately placed in the hospital. By focusing only every other day on the clinical indications for hospitalization, as opposed to assigning a normative length of stay in advance for a particular diagnosis, this PSRO dramatically shortened the amount of time required for review per patient. As a result, the concurrent

review cost per discharge dropped from $5. 00 to $3.25, yet the PSRO'S capacity to reduce unnecessary hospitalization, as measured by CLTR, has not been affected. OTA has no information on whether the quality of the data itself was affected.

In 1979, in an attempt to cut costs further, Delmarva began exempting hospitals with low levels of inappropriate use from admission review, continued stay review, or total review. Consequently, concurrent review costs have dropped below $2.50 per discharge. The impact of this action on appropriate hospitalization has not yet been evaluated.

Recently, the Delmarva Foundation has attempted to improve its utilization review process by employing, on a test basis, the appropriateness evaluation protocol (AEP), a recently developed technique that compares care delivered with an objective ideal. Like CLTR, AEP measures unnecessary days of care created by organizational problems and clinical judgments that are grossly out of tune with mainstream practices. AEP goes one step beyond CLTR, however, because it questions clinical practices that are in local general use. In addition to providing more timely and complete information than CLTR, AEP promises to be less costly.

This same PSRO has begun using cost-effectiveness-type analyses to identify unnecessary days of care and to change modes of physician practice on a diagosis-specific basis. In selecting topics for evaluation, Delmarva focuses on finding relatively low-cost opportunities for making substantial impacts. A model developed by the PSRO'S statistician is used to select topics for regional medical care evaluations that will have the most impact. This model not only identifies differences in utilization between the PSRO area and regional norms, but also identifies differences within the PSRO area in utilization that can be changed through local educational programs.

The Baltimore City PSRO, partially in response to the cap on review costs, has taken a different approach to maximizing its impact under financial constraints. Specifically, it has used its data base and computer capabilities to rank hospital and diagnoses according to excess utilization rates, thereby enabling the PSRO to focus its attention on those hospitals and diagnoses that produce the highest incidence of unnecessary hospital days.

Excessive hospital days may not be true indicators of excessive costs. Nevertheless, it has been the experience of the Baltimore PSRO that hospitals which have problems with diagnosis-specific lengths-of-stay also tend to have problems with high costs and higher than average death rates. Given these facts and the PSRO'S limited budget, the Baltimore organization believes that length-of-stay analysis is an appropriate **way** to identify inappropriate and unnecessary medical care. By identifying problem areas under the length-of-stay analyses, the PSRO is able to focus its concurrent review, medical care evaluation, and profile analysis efforts on those hospitals and diagnoses where the impact can be greatest.

This technique seems to have worked for the Baltimore PSRO. In the first study of this type, that PSRO undertook a very careful review of the hospital that ranked first in excess utilization. Review of the hospital's data indicated potentially serious problems with several specific diagnostic and cost levels. The hospital has since confirmed the existence of these problems and corrected the excess utilization problem.

Evaluations of the national PSRO program have been conducted by the General Accounting Office (GAO) (243), the Congressional Budget Office (CBO) (121), and DHHS (285,288). Currently, GAO is conducting a general review of PSROS, with the intent of focusing future studies on more specific components of the program **(404).** CBO is also currently updating its evaluation (122), **as** is DHHS (138).

The GAO report was a general review of the progress and problems in establishing PSROS. The CBO report more specifically addressed the effect of PSROS on health care costs and whether the estimated savings (if any) derived from the program outweighed the program costs.

DHHS'S 1977 study (288) found no reduction in unnecessary hospital days was achieved

through PSRO activities. Its 1978 medicare rate study (285) concluded that there was a l.5-percent utilization reduction, leading to an estimated savings of $50.0 million. With medicare review costs of $45.9 million, the study concluded, there was a net benefit of $4.6 million (a savings-to-cost ratio of 1.1 to 1) or 10 percent of review costs.

CBO analyzed the same data used by DHHS, but reached a different conclusion. CBO addressed the effectiveness of PSROS by posing the following three questions:

1. How effective is the program in reducing hospital utilization?
2. Are the savings associated with the program large enough to justify the costs of the program itself?
3. Are the program's net-savings large enough to warrant the expectation that PSROS will play a major role in containing health care costs?

CBO concluded that PSROS have brought about a decrease in days of care of roughly 2 percent for medicare beneficiaries in short-stay hospitals. Its analysis of the DHHS data led CBO to the conclusion that utilization savings would be about 30 percent less than review costs, in contrast to DHHS'S conclusion that savings would be 10 percent greater then review costs. (Both estimates, however, were subject to wide margins of error.) CBO further concluded that, even if the 10-percent net savings were accepted, those savings would still be extremely small relative to Federal expenditures for acute patient care—amounting to less than 0.1 percent of medicare reimbursements for inpatient care in short-stay general hospitals.

One problematic issue is the opposite conclusions reached by CBO and DHHS after analyzing the same data, i.e., utilization savings 30 percent less than review costs *versus* savings 10 percent *greater* than review costs. The CBO report lists a number of methodological problems that could shift the conclusion of the cost effectiveness of the PSRO program either to a net loss or to a net savings position, and the persuasiveness of either conclusion might well rest in the eye of the beholder. Perhaps more importantly, however, these analyses do not examine costs in relation to changes in health outcomes that may result from PSRO reviews. Interestingly, these evaluations, which sometimes show a small cost savings and sometimes show a small net cost increase, are critiqued in terms of the value of the national PSRO effect. Implicit in such a criterion is a view of PSROS as a cost-containment mechanism. Yet, if this is indeed the rationale of the PSRO program, what does it matter if the program does cost slightly less than the amount it saves? That net saving is still infinitesimal compared to the total cost of the programs that the PSROS are supposed to be constraining.

USE OF CEA/CBA BY PSROS: GENERAL FINDINGS

The PSRO program has the broad responsibility of seeing that Federal funds are used for health care services and items that are medically necessary, meet professionally recognized standards of care, and are provided at the most economical level possible consistent with quality care. In practice, however, the program has concentrated on weeding out wasteful care. Even in this limited approach, cost-effectiveness approaches (loosely, and not formally, defined) have potential applicability in: 1) setting standards of medical care against which actual practices are judged, 2) the internal management of individual PSROS, and 3) evaluations of the national PSRO effort.

It is theoretically possible that standards based on CEA/CBA or on other analyses incorporating consideration of costs in relation to effectiveness could be developed at the national or regional level and adapted for local use by PSROS. Development of such criteria will be difficult, and acceptance by PSROS may not be gained easily. Criteria for identifying quality medical care have gone beyond gross measures such as length-of-stay norms to (still modest)

criteria such as the timeliness of services and the level of medical care supplied (i.e., CLTR) and objective standards such as the AEP. If usable and relevant data on the appropriate uses of various medical technologies are developed and made available to PSROS, there may be non-siderable potential for applying CEA / CBA in PSRO review activities.

7

Health Planning Program;

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7
Health Planning Program;

INTRODUCTION AND BACKGROUND

Some of the earliest known uses of cost-benefit analysis (CBA) were associated with health planning. In the middle of the 17th century, for instance, a prominent English physician, Richard Petty, favored society's investing more in medicine because the value of saved human life far exceeded its cost (194). In this country, a similar argument was made by Lemuel Shattuck, who in his famous 1850 report used CBA to justify his proposal for sanitary reforms in Boston (559). It is interesting to note that most, if not all, of the early applications of cost-effectiveness analysis/cost-benefit analysis (CEA/CBA) to health care were used by the health profession itself to justify further investment in the public's health. Unlike the main proponents of CEA/CBA today—economists and others who argue that analytical techniques are needed to help society spend its health care dollars more efficiently—earlier proponents and users of CEA/CBA were health professionals who argued that society should spend more money on its health care. These professionals rested their argument on a concept that still dominates the discipline of health planning today—the concept of "medical need. "

"Medical need" is not well-defined. In the discussion that follows, the term "medical need" is used to refer to that health benefit which an individual or a population could and "should" receive from a given health intervention or a prevention program. Thus understood, medical need is subject to change, depending on the state of medical and health knowledge and the availability of resources, and it also carries with it an element of equity. Thus, whereas a heart transplant was not "needed" 20 years ago because the procedure was not possible at that time, the determination as to whether there is a "need" today will depend on the safety and efficacy of the procedure (i.e., the state of knowledge), the personnel, equipment, and money available for delivering it, and a notion of whether it "should" be available. This last consideration—should—is difficult to define because society's underlying values are so intertwined with it. A 95-year-old man, for example, might not qualify for a publicly funded heart transplant under any circumstances. In general, then, the concept of "medical need" is defined as some sort of absolute medical requirement, within some bounds of reasonableness. This concept is inconsistent with generally accepted economic principles which are based on relative values such as price, utility, and alternative uses of resources.

Over the years, whether the issue has been manpower (353), public health departments (175), hospital construction, or comprehensive health planning, the principal consideration in planning has been "need:" If the planner could show a medical need for the resource or the service, the objective was set, the question of financial feasibility often being a secondary consideration.

When funds had to be specifically appropriated to meet an identified "need, " however, an implicit cost-benefit tradeoff must have been made. For instance, the 1933 Lee-Jones Report (353) calling for more physicians would have required additional training funding; the 1945 Emerson Report (175) calling for complete public health coverage required additional Federal funding; and the 1946 Hill-Burton Act calling for increased hospital construction also required Federal funding. Although there is no evidence that formal CEA/CBAs were done in any of these cases, funding decisions were made in a political arena which implicitly weighed benefits against costs: When it appeared that benefits outweighed costs, funds were more likely to be appropriated; when the reverse seemed true, funding was curtailed. In the case of traditional public health measures, the Federal Government

increased funding from the mid-1930's until the early 1950's, at which point it perceived that additional costs (investment) would exceed additional benefits and therefore it curtailed funding even though the original goals had never been met (532). Recently, Federal funding for hospital construction has been similarly curtailed, as has funding for physician training, Although the essential ingredients for a CEA/CBA were present, formal economic analyses of these programs were not ordinarily performed.

Most health care resource decisions today are made in an economic semivacuum. Owing in large part to the third-party reimbursement system and to the relative ease with which the health care system can obtain private capital, the recovery of investment has been all but guaranteed. In other words, the cost-benefit tradeoff has been distorted. Many believe that this distortion has led to the situation in which health care costs have escalated disproportionately to benefits, a situation that began with the advent of private health insurance in the 1930's and 1940's and became even more acute with the 1965 medicare and medicaid amendments to the Social Security Act. Adding to the problem has been the largely unrestrained diffusion of technological achievements of our time. Basically, the unwritten rule has been that if a technological advancement has medical merit it should be adopted.

THE LAW AND CONGRESSIONAL INTENT

Recent legislative measures reflect public concern over the rising costs of health care. Partly in anticipation of the economic effects of the medicare and medicaid amendments and also to help rationalize the health care system, in **1966** Congress passed both the comprehensive health planning (CHP) amendments and the regional medical program (RMP). By that time, scholars including Roemer had already noted a positive correlation between unneeded beds and unnecessary hospitalization (521), and other researchers were beginning to suggest that some health care procedures, especially surgery, were being performed unnecessarily. An underlying premise of the CHP/RMP laws was a belief that the pluralistic and fragmented health care system simply needed a catalyst to help its elements cooperate with one another. Cooperation among these elements, it was believed, would reduce duplication of services and facilities, and future decisions would therefore be "cost effective." Planning and compliance under the CHP/RMP laws were strictly voluntary. These laws envisioned a health planning system based on "need:" If a particular service or facility was "needed," then it must be worth the cost.

As it became apparent that voluntary planning efforts were not effectively constraining the health care system, Congress and individual States began searching for stronger measures to contain costs. First, in 1964, New York State enacted a certificate-of-need (CON) law that empowered its planning agencies to deny reimbursement to hospitals for large capital expenditures unless the agency found that there was a "need" for the service to be provided. Subsequently, several other States enacted similar laws. These State laws were strengthened by Congress in 1972 when it passed section 1122 of the Social Security Act, allowing Federal funds for capital expenditures to be withheld if large capital projects were not approved by State planning agencies.

In 1974, Congress enacted the National Health Planning and Resource Development Act (Public Law 93-641), which required all States to legislate CON laws. The main intent of this Act was to ensure that health resource decisions were based on cost as well as need. Section 1513 of the Act demonstrates concern for health status, cost, and accessibility, and sections 1502 (national health priorities) and **1532** (CON) show similar concerns.

By 1979, Congress had passed amendments to the Act specifying "cost effectiveness" as one of the criteria that local health planning agencies—health systems agencies (HSAs)—must use in their review of the appropriateness of a health

service. Specifically, section 1513(g) was amended to read:

> . . . In making the appropriateness review . . . of a health service, each health systems agency shall at least consider the need for the service, its accessibility and availability, financial viability, cost *effectiveness* (italics added), and the quality of service provided.

Consideration of "cost effectiveness" was also required of the State planning agencies—State health planning and development agencies (SHPDAs)—in their review of appropriateness.

The use of the term "cost effectiveness" in the 1979 amendments suggests a growing emphasis on a cost-benefit type of tradeoff. There is no evidence that Congress intended that HSAS and SHPDAS do anything as elaborate as formal CEA / CBAs, but there is evidence that Congress wanted these planning agencies to consider during the planning process both the effectiveness of the health care system and its cost. The 1979 amendment to section 1502 (national health priorities), for example, specified the following as national priorities:

> The adoption of policies which will (A) contain the rapidly rising costs of health care delivery, (B) insure more appropriate use of health care systems, and (C) promote greater efficiency in the health care delivery systems . . . and the development and use of cost saving technology . . .

and

> The strengthening of competitive forces in the health care services industry wherever competition and consumer choice can constructively serve . . . to advance the purposes of quality assurance, cost-effectiveness, and access.

In addition, other parts of the 1979 amendments severely restrict HSAS' authority over health maintenance organizations (HMOs), in the apparent belief that HMOs have inherent cost-effective incentives, an issue more fully considered in chapter 10 of this volume.

There is evidence, then, that Congress intends that the Department of Health and Human Services (DHHS) consider both effects and costs in its health care resource considerations and that this intent has become more explicit over the years. The National Health Planning and Resource Development Act and amendments have provided five vehicles through which health resource allocation decisions can, theoretically, be affected:

1. CON for large capital expenditures or additions of new services,
2. Federal funds review for certain health programs,
3. appropriateness review for existing facilities,
4. area health services development fund for planning grants for "needed" services, and
5. closure and conversion funding (title XVI) for assisting facilities to close "unneeded" facilities.

Of the five, CON has been the main vehicle available to planning agencies for affecting resource allocation. Appropriateness review carries with it no sanctions, [1] and neither the area health services development fund nor closure and conversion funding (title XVI) has been funded to date **(189).**

The 1974 National Health Planning and Resource Development Act contained several provisions intended to ensure that planning agencies would have technical and analytical capabilities as well as assistance presumably for analyses such as CEA / CBA. First, the 1974 law authorized funding the HSAS at a significantly higher level than the old CHP agencies and also specified that HSA staff are to have expertise in administration, in the gathering and analysis of data, in health planning, in financial and economic analysis, and in the development and use of health resources. Second, the law provided for the use of consultants. Third, it provided for Regional Centers for Health Planning to provide technical assistance to agencies. Fourth, it provided for a National Health Planning Information Center.

The law makes it clear, however, that HSAS are not supposed to gather data, a restriction which is significant because available data are not sufficient to perform most CEA / CBA-type studies. In addition, it should be noted that HSA funding has been appropriated at a significantly

[1] Federal Register, Dec. 11, 1979.

lower level than was authorized, which in itself could preclude HSAS' ability to perform valid studies. Finally, it should be noted that since HSAS have only 90 days for the entire CON process, another factor that may limit HSAS performance of CEA/CBA studies is time.

REGULATIONS, GUIDELINES, AND ASSISTANCE

Like Congress, DHHS is explicit in its desire that health care resource costs be balanced against benefits. In 1979, the Secretary issued planning guidelines to State agencies (SHPDAS), State Health Coordinating councils (SHCCS), and local planning agencies (HSAS) (287). Contained in these guidelines was a message to planning agencies that their assessments of the costs of implementing goals and objectives should be made in context with the expected effects of such implementation both on the health status of the area residents and on the health care system and its costs.

This message is a clear departure from previous ones, those messages, in effect, being that planning should be based strictly on "need" first and "efficiency" second. The departure from previous policy does not neglect the concept of need in the planning process; indeed, identifying and planning for the health needs of the local population is still an important theme in the new guidelines. What distinguishes the new guidelines is that cost containment is apparently as dominant a theme as need (287).

> Cost containment is one of the fundamental purposes of the health planning program. Section l513(b)(3) of Public Law 93-64 requires comparison of cost and benefits.

Previously, the concept of cost containment was generally expressed in terms of eliminating "unnecessary" costs, referring to those costs that produce little or no benefit. The nuances of the latest guidelines suggest that other costs are not justified because they produce insufficient benefits.

To make judgments concerning the adequacy of benefits in relation to costs, agencies must first be able to identify changes in health status indicators that result from health care investments. The Secretary states **(287):**

> The HSA should describe in its HSP (Health Systems Plan) the expected effects of health systems goals upon the improvement in the population's health status . . . as measured by "Indicators" (which) are quantifiable measures chosen to reflect the health status of the population or to represent how well the health system is performing. Direct indicators (such as infant mortality or disability days, and cost per patient day, or time, or distance from primary care) measure the level and/or change in community health and in health systems performance. Indirect indicators (such as percent of the area population with income below the poverty level or the number of houses lacking adequate plumbing) indicate social or environmental conditions which have been attributed to affecting the health of the area's residents.

Then, goals are assessed in terms of expected payoffs **(287):**

> The development of health systems goals is based on an assessment of the health service needs of the present and projected population, and on an assessment of health services in terms of *availability, accessibility, cost, continuity,* acceptability, and quality. The HSP summarizes the HSA'S assessment of health service alternatives. The summary (ies) should focus on possible tradeoffs between characteristics, such as accessibility and cost, which will affect the selection of goals, and priorities among selected goals.
>
> The HSP summarizes the major alternative actions considered for accomplishing each objective. The choice between alternative actions should obviously be predicated on technical, political, and administrative feasibility, as well as cost effectiveness analysis.

In discussing the importance of cost, the Secretary states **(287):**

> The HSA must consider costs when it sets goals and objectives in the HSP, as well as when it develops the recommended actions in both the HSP and AIP (annual implementation plan).

Costs of goals and objectives also should be described to the extent feasible. Such costs will be derived from detailed estimates of the costs of recommended actions in the HSP and AIP.

In addition to analyzing the costs of potential goals, objectives, or actions, the WA should also analyze and compare their expected benefits. The expected effect of any objective or action upon the area's health status and its economic effects as well as any anticipated improvement in health system performance should be specified. The benefits of each alternative should be compared to the costs of that alternative.

Furthermore, the Secretary specifically refers HSAS to the results of a Health Resources Administration (HRA) contract product which provides the conceptual and technical framework for a formal CBA in health planning (287).[2]

HRA has funded numerous other contracts to assist in developing a framework for resource allocation. At least one is a formal CEA/CBA-type analysis (68), but most are more financially oriented, providing guidelines on capital budgeting (particularly lifecycle cost analysis, which includes not only direct capital financing but also future operating expenditures) (11,363, 424). In February 1980, HRA, through its Bureau of Health Facilities, sponsored a conference on health care capital in which one of the principal topics was the relationship between capital and operating costs (286). It is generally conceded that such comprehensive costing is important for CEA/CBA-type studies. Also important is estimating health impacts, and for that, HRA has funded the development of methodologies such as the health status impact study by Kisch, et al. (315).

HRA appears to be making a deliberate and orderly attempt to introduce, interpret, and expand the concept of the balancing of costs and benefits in health planning. At the same time, there appears to be a justified realization at HRA that the state-of-the-art of CEA/CBA may be too immature for formal, routine use of these analytic techniques. HRA's emphasis at present, therefore, is on organizational development at both the State and local agency level, on "need" assessment, and on HSP development (211). Meanwhile, the more sophisticated elements of CEA/CBA methodology (e.g., lifecycle capital budgeting and health status assessment) are being studied and slowly introduced and disseminated to the agency level.

[2]The paper, "Consideration of Benefits and Costs: A Conceptual Framework," was written by D. A. Dittman and K. R, Smith (146), In it, the authors provide guidelines by which planners can set priorities, choose between them, and perform formal cost-benefit analyses.

CURRENT USE OF ECONOMIC ANALYSIS

The cautious approach that HRA has adopted with respect to the use of CEA/CBA-type techniques for planning is reflected in an analysis of HSA activities. An OTA-funded study found no evidence that HSAS have used formal CEA/CBA to assist in making resource allocation decisions (358).[3] In this study, HSAS most likely to employ formal economic analyses during the planning and decisionmaking process were identified through an intensive interview process.

Cost= Benefit Analysis

OTA found one example of the use of CBA by an HSA (Miami Valley HSA, Dayton, Ohio) for the majority of its recommendations in the AIP. For the 1980 plan, the Miami Valley HSA ineluded 54 CBAS. In general, the planning agency's staff contrasted the costs and cost savings of a given health program with the productive value of the estimated years of life saved by that program, thus valuing life in terms of lost wages averted (i. e., the human capital method of valuing life[4]). The HSA'S analyses were presented as

[3]This study, conducted for OTA by Lewin and Associates, examined the use of CEA/ CBA by HSAS and by PSROS.

[4]This method of valuing life is discussed in ch. 3 of *Background* Paper #l: *Methodological issues* and Literature *Review*, prepared by OTA in conjunction with this assessment.

supporting evidence for actions that had already been taken and objectives that already had been set. The methods of the analyses were simple and did not require large amounts of staff time. The HSA staff estimated that between 2 and 10 person-days were spent on each analysis, and indicated that most analyses fell at the low end of this range.

Although the HSA'S analysts had reviewed the pertinent literature for appropriate parameters for the analyses, in most cases the judgment applied to these parameters was solely that of the analysts. For example, **a** local health education organization had estimated that a health education program could save family medical bills up to $290 per year; since the HSA analysts intuitively felt that a savings of $290 per family per year was too high and could not be corroborated, they arbitrarily chose to use in the analysis a savings of only $50, which they discounted at 2.5 percent over a 15-year period. Even with this major modification of benefit structure, the benefit/cost ratio in the analysis was calculated to be 26:1. On the strength of this analysis, the Miami Valley HSA included in the AIP a recommendation to expand the health education program to include 6,000 low-income families.

The supporting information for other similar analyses in the AIP section of the HSP was very sparse, too. Although elsewhere in the plan document there was a general introduction to CEA and CBA concepts, nowhere in the presentation of the analyses was there a discussion of the assumptions required by each of the analyses or the levels of uncertainty associated with them. It was clear that the HSA staff had neither the time, the resources, nor the expertise to carry out valid CBA studies. (Indeed, for many of the programs which they evaluated, sufficient efficacy information is probably not available for anyone to perform good studies). Yet, the studies that were done have been incorporated into the AIP and apparently have gone virtually unchallenged.

This example is indicative of the bind in which the planning agencies find themselves. They are encouraged, and even mandated, to do more than they are perhaps capable of doing. The skills, data, and funds that the agencies need to perform high-quality CEA/CBA-type studies are not available. It is important to emphasize here that OTA found no indication that the Miami Valley HSA or any other has used or intends to use CBA in its resource allocation decisionmaking process. CON deliberations and other resource allocation decisions are not subjected to CBA.

Financial Impact Analysis

Although, for resource allocation decisions, OTA found no evidence of the use of CEA/CBA by HSAS (in the sense that health effects were estimated and compared to costs), it did find evidence of the use of related analyses. These "financial impact analyses" or "net cost studies" can take one of several forms, some of which are broader than others.

One financial impact analysis, for example, addressed the impact of terminating /consolidating a hospital service. That analysis addressed the following issues:

- the cost savings from closure or consolidation of health care services;
- the financial impact on the hospital's terminating/losing the service and the possibility that the hospital's rates would have to be increased to replace "needed" revenue;
- the possibility that an offsetting service could be found to replace lost revenue;
- the impact on established physician practice patterns;
- the impact on existing physical plants; and
- the possibility that other facilities could be used for "needed" services which will no longer be provided by the facility facing the closure.

Note that this analysis, a typical financial impact analysis, does not consider changes in health status, although it does consider both public and private costs (a point which is discussed more fully later), and is used to measure changes in accessibility to services.

Another example of a financial impact analysis was an analysis an HMO submitted with its application for a CON for an open heart surgery unit. That analysis demonstrated that the HMO would save money by building and operating its

own unit rather than contracting for the service as it was then doing. In its review of the HMO's application, the HSA countered with its own study showing that net societal costs would be increased by the addition of the HMO's unit, because there would be excessive capacity in the area and existing facilities would lose business. Again, this finding may represent the clash of private versus public costs and benefits.

Most HSAS undertake much more traditional analyses, generally attempting to determine whether a given service meets minimum utilization standards, standards which Kristein has characterized as resting more on equity than efficiency (332). The national planning guidelines or similar standards are often used for these determinations. The assumptions are that meeting these standards satisfies basic medical "needs" and guarantees at least a minimal level of efficiency, and is therefore cost effective. Seldom, if ever, though, are costs weighed against expected health benefits.

POTENTIAL FOR USE

Discussions with leading health planners, examination of the literature, and review of the findings of the OTA-sponsored empirical study mentioned above (358) yield the impression that capital budgeting procedures—specifically, lifecycle cost analysis—are one aspect of decisionmaking in planning which is receiving substantial emphasis. As noted previously, lifecycle cost analysis includes the notion of operating costs throughout the expected life of the investment, as well as the capital depreciation schedule. Analysts argue that the total cost of a purchase includes the cost of operating it as well as buying it. Sophisticated financial and accounting techniques are required to estimate depreciation schedules, opportunity costs, and the costs of secondary effects such as might be caused by displacement of personnel or by increased/decreased utilization in another part of the system.

In effect, HRA and its Health Planning Centers appear to be concentrating on the cost side of the ledger, as opposed to the effectiveness side. They appear to be telling the HSAS that societal cost is much more than initial purchase price, and that as far as effectiveness is concerned, national, regional, or local standards and guidelines will have to suffice for now. This appears to be a reasonable approach, although even without good efficacy information, secondary systems' effects (e.g., lost revenue to the provider, changes in reimbursement rates, changes in physician practice patterns) could at least be described and their magnitude estimated, as was done by one of the HSAS interviewed (see earlier discussion).

Considering these secondary effects raises again the question of private versus public costs and benefits, and how these variables should be treated. The resolution of this issue has enormous implications regarding reimbursement rates, competitive systems development, and public responsibility vis-a-vis financial solvency of nonprofit as well as public health care institutions. As an example, if an HSA denies a community hospital the right to operate a service on the grounds that certain utilization standards **(e.g.,** national guidelines) are not met, thus causing the hospital to lose significant revenue, how should cost changes be treated? First of all, some utilization will be shifted to other facilities, which will now operate at a higher capacity and thus, presumably, more efficiently. But the losing facility will find itself with one less cost center with which to allocate overhead and will have to absorb the now unallocated fixed costs elsewhere. What responsibility does the HSA (or society) have to the losing facility with respect to assuring that it recover its fixed costs? And how should the costs be treated in the analysis?

Furthermore, revenue-changing decisions inevitably affect reimbursement rates. In Maryland, for instance, the Rate Review Commission has been at odds with that State's HSAS, because the HSA-approved projects 1end legitimacy to requested rate increases (15). This class

of problems may always be present as long as facilities are reimbursed on a cost basis. Since one of the purposes of the HSAS' activity is to ensure efficient utilization as measured by standards such as the national guidelines, the question becomes whether the HSAS are actually capable of ensuring that such standards are met. The HSA'S control of utilization is indirect at best, and HSAS cannot easily fine-tune their approach. For that fine-tuning, Professional Standards Review Organizations (PSROS) monitor physician activities in the facilities, but even they only or primarily monitor certain Federal beneficiaries. [5]To help ensure that valid CEA/CBAS are done and that their results are effectively used, therefore, it would seem desirable to coordinate the planning process (i.e., HSAS), the quality assurance/utilization review process (i.e., PSROS), and the reimbursement/rate review process.

Earlier it was noted that the time available to an HSA staff for a CON analysis is very short, probably considerably less than 1 month. One recommended method to overcome this time constraint is to require the applicant to do the analysis **(362).** For a renovation replacement project, for instance, the planning agency might require the institution to provide an analysis of five alternatives: 1) reparation, 2) partial replacement, 3) a decrease of capacity (or a smaller project), 4) an increase of capacity (or a larger project), or 5) no change. This technique does require tradeoffs to be considered, and it might allow the planning staff time to respond to an analysis in an appropriate manner and often to anticipate issues.

CEA and Competition

The private versus public cost controversy takes on great significance when the HSA and the HMO interact. In the example cited earlier, the HMO contended that since its costs would be lowered by providing the service itself rather than by contracting for it, the HMO would be at a better competitive advantage, thus forcing the fee-for-service sector, ultimately, to lower its prices or to face the possibility of losing subscribers. This argument—what is good for the firm is good for society—is the classic argument for the free market firm. The HSA countered with its own analysis showing that the additional service would result in overcapacity in the HSA area resulting in higher societal costs—a classic social welfare argument.

Congress has apparently resolved this controversy with the passage of the 1979 Public Health Service Act Amendments. These amendments contain a provision forbidding HSAS to deny a CON to an HMO as long as the HMO can effectively demonstrate that the investment is beneficial to its own subscribers and that it is more efficient for the HMO than other investments. That provision is coupled with the law's requirement that HSAS must consider "the strengthening of competitive forces in the health services industry wherever competition and consumer choice can constructively serve . . . to advance the purposes of quality assurance, cost effectiveness, and access" (15).

The new mandate contained in these amendments sets up potential forces that run counter to the conceptual basis for using CEA/CBA. The rationale underlying CEA/CBA methodology is that the market is not operating properly. The role of CEA/CBA in planning within the context of a competitive environment is unclear.

CEA and HSA Orientation

Other problems with the use of CEA/CBA in health planning—apart from the conceptual difficulties of using CEA/CBA within the construct of a partially competitive system—arise because it is unclear whether the HSA is (or should be) an agent of the community for which it plans, or an agent of the larger society. That distinction is important because the orientation which an analysis takes is very much dependent on the orientation of both the sponsor and the consumer of the analysis.

OTA found that not only is there no standard, generally accepted set of CEA/CBA methods for all technologies, but also that many of the parameters chosen, as well as how they are

[5]See ch. 6 of this volume for further discussion of PSRO activities.

valued, depend on the purpose of the analysis and for whom it is done. Consequently, the results of even a technically superior CEA/CBA by an HSA could vary significantly depending on the importance which the agency attached to saving Federal tax dollars *or* to ensuring that its local community received all the health care which was needed, subject to prevailing guidelines.

One HSA, for example, may set its priorities in large part on the basis of total societal economic payoff rather than health "need" alone. Thus, an analysis by this HSA would include not only health benefits, but also economic benefits such as decreased health care costs and increased wages. This HSA may reject programs such as large hospital capital investments because they seem to weigh too heavily on the cost side without sufficient demonstrable or proven benefits, opting instead for programs such as alcoholism programs that increase working hours. Another HSA may take the attitude that since its community health care system is not operating under a budget, it should consider "need" and efficiency in meeting that need alone. The board of that HSA may feel that increasing the productivity of the local working force is not relevant to its decision process, that is, including productivity variables will bias the selection process away from programs for its nonworking constituents.

There is some conflicting evidence as to how the HSAS do view themselves, but generally it is believed that the more local orientation holds. HRA Administrator Foley stated that despite the law's clear mandate to control costs, "from the perspective of HSAS and State agencies, it is not clear that their priority is on 'reasonable costs', " generally because of the third-party reimbursement system, Foley believes that "the major proponent of giving priority to the goal of reasonable cost is the Governors' Offices and States' legislatures" (211).

Luft and Frisvold, on the basis of an intensive review of CON decisions of two CHP agencies, stated that, at least prior to 1974, "need" was the primary criterion on which decisions were based, and that an applicant could easily convince the agency that the proposed service was "needed" even in the face of a surplus, as defined by established standards (375). Thus, Salkever and Bice were able to show that CON did not constrain overall investment in capital budgeting, although it did alter the types of investments made (530), the latter point possibly providing a clue to the potential role of CEA/CBA.

Most of the studies cited above analyzed the effects of the CON process immediately following its introduction. Recently published reports based on surveys completed by the American Health Planning Association, however, state that the CON process has yielded substantial savings (417). Although the surveys and thus their results have been questioned, this information indicates a continuing controversy over the effectiveness of CON. Altman, in attempting to describe incentives of planners, noted that the constituents of the local HSA were the local providers and consumers, both of whom have economic incentives to increase health care resources in their own area, particularly, since such resources are ordinarily paid for on a much broader basis; the constituents of the State planning agency, however, are the Governor and the State legislature, whose economic incentives are to constrain costs (15). Consequently, it is not unusual for State and local officials to disagree on health resource policy issues (100). Interestingly, however, roles are sometimes reversed, with local agencies being surprisingly cost conscious—recommending against CON–and State agencies being surprisingly insensitive to cost—awarding CON over an HSA'S objections (15,189,499).

Above it was noted that according to some studies, CON has resulted in a shifting of capital expenditures, but not in a decrease in total capital expenditures. The purpose of CEA/CBA is not to constrain costs, but to assist in more efficient allocation of resources, and if properly applied, the CEA/CBA technique possibly could play a valuable role in the CON process. The first attempts to evaluate the effectiveness of CON initially focused on its superficial objectives (e.g., restraining increases in hospital beds). These studies indicated success. A second generation of more sophisticated analyses assumed that the real objective of CON was to al-

locate or to constrain capital investment and thus costs, and these analyses indicated failure (530). A third generation of analyses, which has not yet been performed to OTA'S knowledge, could attempt to determine whether the shift in resources caused by CON activity was cost effective. That is, is society better off (more healthier, happier) now with, for example, sophisticated diagnostic technology than it would be if more beds had been built? CEA/CBA in theory could shed light on this issue; the possibility of its being used in the future for this purpose, therefore, may merit consideration.

A key question remaining is how should CEA/CBA be used in the planning process? To constrain costs or to obtain the best buy for the money? The evidence indicates that CEA/CBA is most effective when it is used within a constrained budget, that is, when choices have to be made. As was previously noted, HSAS currently plan in an economic semivacuum. Although they are told to consider costs when they plan for need, HSAS plan for a health system which is under no budget constraint. In some cases, for instance, HSAS are urged to consider the "financial feasibility" of a project; the stamp of approval (i.e., CON), however, is a ticket for financial feasibility, since once a CON is awarded, reimbursement (full cost recovery) is ordinarily guaranteed (100). This circular situation had led to calls for either external regional budget controls or for the less stringent measure of requiring the agency to evaluate alternative projects to accomplish the stated objective (375). The former would require a major restructuring of the country's health care financing system. [b]The latter, requiring alternative proposals, is therefore advocated as a more feasible solution, and indeed, is practiced by some HSAS interviewed for the OTA study **(358).** The latter mechanism by itself, however, is not sufficient as a cost-containment strategy since it allows the possibility of the acceptance of an unlimited number of objectives. In this case, a CEA/CBA would simply help the planner to ensure that each objective is accomplished in an efficient manner, albeit helping to ensure that more benefit is obtained per dollar expended. By itself, however, use of CEA/CBA is not sufficient for containing overall health system costs.

The resource allocation decisionmaking process of HSAS is basically a political process, rather than a technical one. The law envisions this implicitly by requiring that the controlling board be representative of local constituents. Nevertheless, there are some indications that, at least during the CON process—which is at present the primary means of affecting the system—the planners and their analysts are becoming more sophisticated at the capital budgeting process. There is little indication, however, that HSAS have attempted to estimate the health implications of their decision process; there failure to do this is understandable given the lack of knowledge among the medical and health community about this. In general, decisions are based on either the national guidelines or on similar standards, both of which **are** themselves often based on "efficient" utilization rates and ordinarily set by consensus of experts -without information on marginal costs and benefits.

There does appear to be a serious effort in some instances to compare the cost of programs to the cost of alternative programs which accomplish the same objective (e.g., to compare the cost of a surgicenter to the alternative cost of performing inpatient surgery). In these instances, benefits (or risks) are usually assumed to be equal across alternatives. If that assumption is true, then the study in which it is made can legitimately be called a CEA/CBA, on the net-cost end of the analytical spectrum. If not true, however, the study is simply a financial impact analysis. Most of the studies that have been done appear to be examples of financial impact analysis, because although equality of benefits across alternatives is often claimed, in fact, the analysts seldom have any hard data to prove it.

[b]Altman believes this would further politicize the decisionmaking process (15); if such is the case, it would not be conducive to objective analysis.

[c]See app. B.

USE OF CEA/CBA IN HEALTH PLANNING: GENERAL FINDINGS

The potential for increased use of CEA / CBA in health planning hinges on several elements:

- the ability of HSA staffs to upgrade their technical skills and fundamental understanding of CEA / CBA methods, and to increase the amount of time and financial resources available to conduct analyses;
- the establishment of budgetary bounds within which investment decisions are competing with one another;
- the extent to which market forces operate and are encouraged;
- the ability to measure the health effects of technology and the availability of such data;
- the availability of standard health care utilization data; and
- the state-of-the-art of the CEA / CBA techniques themselves.

Meanwhile, health planning agencies could perform analyses by explicitly listing or arraying all elements which are included or will be affected by an investment. At the most basic level, this would be a descriptive process: When costs and effects can be quantified, that would be done; when they can be combined, that would also be done. If there is insufficient evidence but some probability of a cost or effect resulting from the decision, it would be described and commented on, but not necessarily quantified.

This approach would require systematic analysis but would not require as sophisticated analytical skills. Since the health planning decision-making process is political in nature and ultimately rests on intangible factors anyway, finely tuned studies with valid, aggregated, and quantified variables are not essential for the process to be assisted by CEA / CBA methodology. Moreover, as analysts become more familiar with formal CEA / CBA techniques, analyses could evolve toward increased sophistication.

In this manner, the intent of the health planning law might be met without requiring an immediate herculean effort to upgrade the technical skills of planners and their analysts. This suggested approach could also assist in rationalizing the planning process and provide the foundation for the time when, and if, budget controls are ever imposed on the health care system.

8
Market Approval for Drug; and Medical Devices

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Market Approval for Drugs and Medical Devices

INTRODUCTION AND BACKGROUND

The Federal Government has been authorized to regulate various aspects of drugs in the United States since President Theodore Roosevelt signed into law the Pure Food and Drug Act of 1906 (113). That law was in part intended to help prevent adulteration and misbranding of drug products, In 1912, Congress enacted the "Sherley amendment," which prohibited companies from making false and fraudulent curative or therapeutic claims on the labels of their products. In 1927, Congress established the Food and Drug Administration (FDA).

The 1938 Food, Drug, and Cosmetic Act, passed by Congress in response to a tragic event in which over **100** people died from ingesting a lethal drug product, [1] increased the Federal Government's regulatory control over the marketing of drugs, devices, foods, and cosmetics. In addition to establishing several labeling requirements for drugs, the 1938 Act prohibited interstate commercial shipment of new drugs until they had been adequately evaluated by the Federal Government to show that they were safe under the conditions of use listed on their label. It also authorized FDA to remove from the market any drug it could prove to be unsafe. The Durham-Humphrey Amendment of **1951** defined criteria and categories based on levels of drug safety for restricting a drug to legend (i.e., prescription only) status.

Again stimulated by a disaster, this one involving fetal abnormalities caused by the drug thalidomide, [2] Congress enacted the Drug Amendments of 1962 (Kefauver-Harris amendments). These amendments required drug manufacturers to provide "substantial evidence" that their products were efficacious as well as safe. The amendments also required drug manufacturers to report promptly to FDA information concerning the safety and efficacy of their marketed products and strengthened FDA's authority to remove unsafe or ineffective drugs from the market. In addition, the 1962 amendments authorized the notice of claimed investigation for a new drug (lND) process—a legal mechanism used by FDA to regulate human investigations of drugs.

The Federal Government was given some authority to regulate medical devices in the original 1938 Food, Drug, and Cosmetic Act. Congress substantially increased this Federal regulatory authority, however, by passing the Medical Device Amendments of 1976 (116). The 1976 amendments authorize FDA to require device manufacturers to demonstrate acceptable levels of safety and effectiveness for their products. They establish three categories of products, and each category has a different level of Federal control. Medical devices that are implanted, used in supporting or sustaining human life, of substantial importance in preventing impairment of human health, or that pose a potential unreasonable risk of illness or injury, for example, usually are subjected to premarketing evaluation of safety and efficacy. The 1976 amendments permit FDA to ban or remove from interstate commerce unsafe or ineffective devices. They also allow the Agency to restrict the use of a device to persons with specific training or experience or to those in specified facilities if the device's safety and effectiveness cannot be assured in general use.

[1] Elixir of sulfanilamide made with the lethal solvent diethylene glycol.

[2] Use of the sedative thalidomide by pregnant women in Europe and Japan resulted in several cases of phocomelia, a fetal abnormality characterized by flipper-like limbs. The drug was not approved by FDA for marketing in the United States.

FDA'S PRESCRIPTION DRUG MARKET APPROVAL PROCESS

Since 1962, FDA has used its statutory and regulatory authority to establish an extensive system for evaluating virtually every new prescription drug prior to its release into the general medical marketplace. By law, FDA must base its evaluation of prescription drugs on two criteria: safety and efficacy.[3] The procedures used by FDA in this premarket approval process, described in detail elsewhere (119,144), are briefly summarized below. To initiate human investigations involving agents legally defined as "new drugs, " the drug "sponsor" (e.g., a manufacturer or an independent investigator of a new drug) must obtain FDA's approval. To obtain such approval, the sponsor submits to FDA's Bureau of Drugs (BOD) an IND, describing the qualifications of the investigators and the design of the planned trials. In the IND review process, BOD also examines data regarding the pharmacology and toxicology of the applicant drug collected in animal studies and in human studies that were not subject to FDA approval (e.g., those conducted in foreign countries). If the sponsor's IND is approved, the sponsor may proceed with clinical testing (i.e., testing in human subjects) in the United States, After completing clinical testing under IND, the sponsor files with BOD a new drug application (NDA) that describes in detail the results of IND clinical trials. The applicant drug has usually been tested in **500** to **3,000** human test subjects **(306).** By filing an NDA, the sponsor is requesting FDA's permission to market the new drug in interstate commerce.

According to Dr. Marion J. Finkel, Associate Director for New Drug Evaluation, BOD, FDA, the review process for INDs and NDAs proceeds as follows **(206):**

> Each IND and NDA application is reviewed by a team of FDA scientists: a physician, a pharmacologist, a chemist, a pharmacokineticist, usually a biometrician, and when applicable, a microbiologist. Important NDA's are then presented for consideration to advisory committees, of which the Bureau of Drugs has 13, consisting of extramural [mostly nongovernmental] experts in, principally, the subspecialities of medicine, clinical pharmacology, and biometrics. The committees recommend to the FDA whether or not an NDA should be approved for marketing and, if so, under what labeling, and whether the sponsor should be requested to perform additional studies in the postmarketing phase. When recommendations are made against approval, the committees provide advice on new studies to be done by the sponsors to explore the drug's safety and effectiveness further. The use of advisory committees is the FDA's primary method for broadening the decisionmaking process.

During the NDA review process, FDA reviewers analyze the sponsor's summaries of data—and when needed the actual raw data—generated from clinical investigations. A major task for BOD during this review process is to ensure that clinical experimental data support the sponsor's claims for the drug's safety and efficacy that appear in the drug's labeling. The NDA review process usually entails deliberations between FDA and a drug's sponsor regarding claims for safety and efficacy. If FDA concludes that an NDA is deficient, it usually will require the sponsor to generate new data, modify its NDA with additional information, and resubmit the application to the Agency. Once a sponsor obtains an NDA approval from FDA, it is authorized to market the drug in interstate commerce for only the specific indications (uses) that have been authorized by FDA.

'Food, Drug, and Cosmetic Act S505, 21 U.S. C. S 355 (1976) part 314 (1979). The Act uses the term "effectiveness" instead of "efficacy. "

FDA'S MEDICAL DEVICE EVALUATION PROCESS

The Medical Device Amendments of 1976 (Public Law 94-295) require FDA's Bureau of Medical Devices (BMD) to classify each medical device—on the basis of the level of regulation necessary to ensure safety and efficacy—into one of three regulatory classes. Products placed in the class I category—those requiring the least controls to ensure their safety and efficacy—are subject only to general controls, including premarket notification, registration of the manufacturer, prohibition of product misbranding or adulteration, adherence to FDA-promulgated good manufacturing practices, and compliance with various recordkeeping requirements. Class II products—those for which general controls are deemed inadequate to ensure their safety and efficacy—must comply with performance standards either established or recognized by BMD when existing information permits development of such standards. General controls also apply to class II devices unless superseded by a standard. Class III devices—those for which neither general controls nor performance standards alone are sufficient to ensure their safety and efficacy—are subject to premarketing approval. (BMD can also require premarketing approval of devices for which insufficient information is available for the development of applicable performance standards.) Class III devices are subject to general controls.

The process BMD uses to evaluate the safety and efficacy of new class 111 medical devices is similar to that used by BOD in its NDA reviews. BMD, however, is required by law to use its advisory panels during a product's review, whereas BOD'S use of advisory panels is discretionary. BMD is also authorized to use another premarketing approval process, the product development protocol (PDP). PDP was designed to encourage the development of, and to streamline the approval process for, innovative medical devices. During a PDP process, investigation of a device and the development of information necessary for its premarket approval are merged into one regulatory mechanism. BMD works closely with the manufacturer while information to support the device's safety and effectiveness is being developed. The 1976 Medical Device Amendments also include an investigational device exemption (IDE) provision. IDE allows FDA (technically, the Secretary of Health and Human Services) to exempt a new device from other provisions of the amendments to permit controlled testing of new devices prior to commercial marketing. The IDE process is similar to the IND process that BOD uses.[4]

[4]45 FR 3732.

SAFETY AND EFFICACY CRITERIA

The Food, Drug, and Cosmetic Act mandates FDA to require a drug sponsor to: 1) collect, by all methods reasonably applicable, data that demonstrate a drug's safety, and 2) generate "substantial evidence" from controlled trials to show that the drug is efficacious for use under the conditions set forth in the proposed labeling (144). Although the Act provides no definitions and little guidance for the meanings of safe and effective,[5] it does describe "substantial evidence" of effectiveness as follows (144):

[5]OTA differentiates "efficacy" and "effectiveness" as follows (465):

Efficacy: The probability of benefit to individuals in defined populations from a medical technology applied for a given medical problem under ideal conditions of use. *Effectiveness:* Same as efficacy except that it refers to ". . . average conditions of use."

Although the FDA statutes use the terms "effective" and "effectiveness," the definitions implicit in the statutes appear to mean "efficacy." Thus, "efficacy" is used in the present report.

> The term "substantial evidence" means evidence consisting of adequate and well-controlled investigations, including clinical investigations, by experts qualified by scientific training and experience to evaluate the effectiveness of the drug involved, on the basis of which it could fairly and responsibly be concluded by such experts that the drug will have the effect it purports or is represented to have under the conditions of use prescribed, recommended, or suggested in the labeling or proposed labeling thereof.

Because no drug is absolutely safe or always effective under all conditions of use, FDA weighs a drug's benefits in comparison to its risks. In FDA's evaluation process, an applicant drug's safety and efficacy are compared to the safety and efficacy of approved products or medical procedures used to achieve similar therapeutic objectives. Comparisons are also made with the effects of inert substances (placebos). According to Dr. Marion J. Finkel of FDA (204):

> A drug cannot be considered safe if it is less effective and has more side effects than other drugs labeled for the same indication. It might be safe for certain subpopulations or certain restricted indications, however.

According to Dr. Robert Temple of FDA (594):

> We're not supposed to refuse approval because of lesser effectiveness, but we can on grounds of lesser safety which reduces the benefit/risk ratio. Relative effectiveness can be noted in labeling.

FDA also uses such comparisons when selecting NDAs for its priority review or "fast track" review process.

FDA has published 25 clinical guidelines that define the types of studies it deems appropriate to use to establish safety and efficacy of a drug. The performance criteria used to evaluate an individual drug's safety and efficacy, however, can vary among different pharmacological classes. This variance results from the imprecise nature of pharmacologic intervention in disease. The benefits and risks of a drug can vary from disease to disease, from population to population, and from clinical situation to clinical situation. Drug-induced hair loss, for example, is often viewed as an acceptable risk of taking a drug that is an effective treatment for cancer, especially when no other effective therapy exists. The same side effect, however, would probably not be acceptable for a drug that reduces the severity of a minor, self-limiting condition.

The complete risk-benefit analysis of a drug, therefore, is not based solely on statistical evaluation of data on safety and efficacy generated from clinical trials but also on "the context of the disease for which [the drug] is intended, the availability of other therapeutic modalities, including other forms of pharmacologic therapy, and public health implications of its availability" (144).

The task of evaluating the safety and efficacy of drugs, as well as FDA's ability to perform this task, has been subjected to extensive analysis and debate (119,144,242,613).

CURRENT ECONOMIC CONSIDERATIONS IN FDA'S DRUG AND MEDICAL DEVICE APPROVAL PROCESSES

FDA does not directly use economic criteria in its approval processes for drugs and medical devices. The Agency's primary statutory authority, the Food, Drug, and Cosmetic Act, neither authorizes nor prohibits the use of economic criteria in FDA's evaluation of applicant drugs and devices. The legality of using cost effectiveness to help evaluate new drugs and devices has not been tested.

Although FDA does not formally assess the potential economic impact a drug or medical device might have on the allocation of health care resources, some FDA actions may be based indirectly on—or taken in spite of—economic considerations. Further, some FDA actions certainly have economic impacts. Several examples are cited below.

Classifying the Potential Therapeutic Importance of New Drugs: FDA sets priorities for its review of new drugs according to the potential therapeutic usefulness of each applicant drug. This priority-setting process supposedly begins within 6 months after a sponsor submits an IND to FDA (206). On the basis of preliminary information about a drug's pharmacological effects and on data from clinical trials, a drug is assigned a number and a letter derived from the following classification schemes (221):

Numerical Classification

1 = New molecular entity not previously marketed in the United States.
2 = New salt, ester, or derivative of an active moiety marketed in the United States.
3 = New formulation of a compound marketed in the United States.
4 = New combination of two or more components not previously marketed together in the United States.
5 = Product duplicates a drug marketed by another firm in the United States.
6 = Product already marketed by some firm in the United States; approval being sought for new indication of use.

Letter *Classification*

A = major therapeutic advance over other currently available drugs, etc.
B = modest (or moderate) therapeutic advance over other currently available drugs, etc.
C= little or no appreciable therapeutic advance over other currently available drugs, etc.

The purpose of using a drug classification scheme-of this type is to expedite the review of data for drugs that represent important new therapeutic entities. FDA seeks to review NDAs submitted for drugs it assigned a 1A or IB rating as expeditiously as possible; such reviews receive high priority by BOD (206).

Even though the criteria used to classify a new drug are based on scientific data relating to a drug's clinical safety and efficacy, there are indirect economic consequences of FDA's selection process. The expeditious review and resultant early marketing of **a** new drug that represents a major therapeutic breakthrough in the treatment of a heretofore uncontrollable disease could help reduce the use of existing treatment measures, such as hospitalization and surgery. The recently introduced drug cimetidine, for example, appears to provide safe, effective, and relatively inexpensive (compared to hospitalization or surgery) treatment for duodenal ulcers; using cimetidine, some ulcer patients may avoid hospitalization (203,632). FDA rated cimetidine **as a** 1A drug. If indeed the use of cimetidine reduces ulcer patients' hospitalizations and surgery, then an expeditious FDA review and approval of the drug could help reduce medical expenditures for the treatment of duodenal ulcers. A complete analysis would include calculations of the potential economic impacts of delaying the review of one or more other INDs or NDAs, in order to expedite cimetidine's application review.

According to FDA's Dr. Marion J. Finkel (205):

> NDAs for 1A and IB drugs are full NDAs, containing all of the safety and efficacy data required for any NDA. Expeditious review of these merely means that the NDAs do not wait their turn in the pipeline but are reviewed before NDAs with lesser classifications . . .
>
> Rarely, FDA will accept an NDA for a 1A drug without as much long term human (or animal) safety data required for NDA approval.

In at least some cases, however, the expeditious review of an important new drug might result in an incomplete assessment of the drug's safety, and an unexpectedly significant level of adverse reactions to the drug might occur. To help prevent such an occurrence, FDA can—and sometimes does—ask a sponsor to conduct postmarketing surveillance as a condition of approval for a new drug (131).

Use of Abbreviated New Drug Applications (ANDA) and "Paper" NDAs: Two examples of the indirect use of economic considerations in FDA's drug approval processes are the ANDA and the so-called "paper" NDA. The ANDA process enables a drug manufacturer to obtain from FDA marketing approval for a "generic" drug product that is purported to be an identical version of an already approved product, usually

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after the originator's patent has expired. Because of FDA's earlier interpretation of the Food, Drug, and Cosmetic Act, ANDAs are currently only used for products originally approved by FDA between 1938 and 1962. FDA is preparing a policy, however, that will permit the use of ANDAs for products approved after 1962. A manufacturer can obtain an ANDA essentially by demonstrating that it complies with FDA's current good manufacturing practices and labeling requirements and that it can make a product that is at least chemically equivalent and supposedly bioequivalent to the originators. Although evidence of chemical equivalence is always required, the FDA Commissioner can forgo requiring evidence of bioavailability in the ANDA process for a given drug.

"Paper" NDAs are designed to permit a drug manufacturer to meet the requirements for submitting evidence of the safety and efficacy of its post-1962 product by citing existing data from published clinical trials involving a chemically equivalent, previously approved product. The legality of "paper" NDAs was challenged in court by the Pharmaceutical Manufacturers Association (PMA) and a few major drug manufacturers (473). The court dismissed the suit on the grounds that the plantiffs had not exhausted their administrative remedies within FDA, so PMA has filed a petition that: 1) questions FDA's authority to implement a "paper" NDA policy, and 2) requests that if such a policy is implemented by FDA, it be done through the official notice and comment rulemaking procedure.

The primary purpose of both the ANDA and "paper" NDA modifications in the new drug approval process is to facilitate the marketing approval of drug products identical to those that FDA has previously found to be safe and efficacious on the basis of data supplied in a product's original NDA. These mechanisms are designed to prevent unnecessary work burdens on FDA's resources. The use of ANDAs and "paper" NDAs is supposed to increase the availability of FDA staff to review NDAs submitted for innovative drugs, e.g., those categorized by FDA as 1A. The use of these mechanisms also eliminates the need for sponsors to conduct duplicative clinical trials. Thus, manufacturers' expenses associated with entering some existing drug product markets can be reduced.

Removal of Diethylstilbestrol (DES) From Animal Feed: The estrogenic compound DES was used by poultry and other livestock raisers for many years to increase the muscle and fat content of their animals. FDA first approved such use of DES in 1954.[6] After that, DES was used in animal feed to reduce the total feed intake necessary to achieve maximum animal weight gain. Furthermore, some studies showed that the use of DES in animal feed helped reduce the cost of raising poultry and other livestock. Theoretically, therefore, the use of DES may have helped contain poultry and meat prices for consumers.

Ingestion of DES by humans, however, was eventually correlated with an above-normal risk of developing certain types of cancer, such as adenocarcinoma of the vagina or the cervix.[7] Because of DES's cancer-producing potential, FDA attempted several times to remove this substance from animal feed.

The authority for its efforts was the Delaney clause,[8] a proviso added to the 1958 food additive amendment to the Food, Drug, and Cosmetic Act:

> . . . no additive shall be deemed to be safe if found to induce cancer when ingested by man or animal . . .

This clause was reiterated in the 1968 animal drug amendments to the same Act.' Under the animal drug amendments, approvals for the use of new animal drugs, which are used in the livestock industry for the treatment and prevention of disease and as growth promoters, are to be granted only after a two-part evaluation by FDA, First, FDA must determine that the drug is safe and effective for use in animals. Second, it must assess the safety of potential residues that might occur in food derived from treated animals. 10

"44 FR 54853.

?44 FR 54852.

'Sec. 409(c)(3)(A), Food, Drug, and Cosmetic Act, 21 U. S.C. 348(c)(3)(A),

'Sec. 512, 21 U,S.C. 360b.

los_{ec}, 512, 23 U. S.C. 360 b(d)(l).

After evaluating and finding DES to be a carcinogenic substance, in 1972, FDA attempted to ban its use in animal feed. That effort terminated in a litany of legal proceedings with the livestock industry and animal feed manufacturers. " On January 12, 1976, therefore, FDA once again initiated a legal process to remove DES from animal feed. This time, the Agency also issued an inflation impact statement regarding its proposed ban of the use of DES. [11]* The statement concluded:

1. There are no satisfactory alternatives to the Agency's proposed action which are consistent with the legal constraints imposed by the Federal Food, Drug, and Cosmetic Act as amended.
2. Operating expenses to feedlot producers of cattle for feed and other items are estimated to decrease by $156 million during the first year following a DES ban. These increased costs are expected to fall substantially over the longer term as substitutes to DES become available in greater quantities.
3. Retail prices of beef are estimated to rise by about 2 cents per pound, meaning the per capita cost of beef to consumers at current levels of consumption would increase from $2 to $3 annually. The aggregate consumer impact is estimated at $.503 million.
4. A ban on DES would not cause major inflation impacts, as defined by the HEW / OMB criteria, in the areas of competition, productivity, supplies of materials, or use of energy.
5. The benefits from implementing the proposed action will be the elimination of any risk of any cancer associated with the consumption, via the edible tissues of food-producing animals, of residues attributable to DES.

According to former FDA Chief Counsel, Richard Cooper, FDA's mandate under the Delaney clause and the 1968 animal drug amendments prevented the Agency from considering any economic benefits of DES in its decision to ban the drug from use in animal feed (127a).

On June 29, 1979, then FDA Commissioner Donald Kennedy ordered the withdrawal of approval of the new animal drug application for DES. *3 In taking this action to ban the use of DES, Commissioner Kennedy stated:

> FDA is not authorized, under the Food, Drug and Cosmetic Act, in considering the question of whether a new animal drug has been shown to be safe for use, to weigh the "socio-economic" benefits that that drug provides against a health risk to the ultimate human consumers of treated animals. Even were I to attempt to weigh the benefits of DES against its risks, this record would not provide sufficient information to compute the risk associated with DES or to determine whether, and to what extent, use of DES provides any health benefit or even any economic benefit to society.

This case study illustrates one situation in which FDA interpreted the Delaney clause of the Food, Drug, and Cosmetic Act (its primary statutory authority) to mean that in its decisions concerning the removal of carcinogenic substances from the market, it cannot consider the results of economic analyses.

Evaluation of an X-Ray Equipment Performance Standard Established by the Bureau of Radiological Health (BRH): This case illustrates FDA's use of cost-benefit analysis to evaluate one of its regulations.

In 1972, FDA's BRH promulgated regulations containing a performance standard for medical diagnostic X-ray systems and their major components.[14] These regulations, among other things, required X-ray equipment manufacturers to certify that all specified components manufactured after August 1, 1979, comply with the new standard. Another provision stated that after August 1, 1974, no uncertified components could be assembled or reassembled into an X-ray system.[15] The purpose of these regulations was to help ensure the radiation safety of diagnostic X-ray systems. [16]

In 1974, BRH estimated the costs and benefits associated with these regulations. The Bureau

[11] 44 FR 54854.
[12] FDA docket No. 76N-002, Inflation Impact Statement DES, Office of Planning and Evaluation, January 1976.
[13] 44 FR 54900.
[14] 21 CFR 1020.30, 1020.31, 1020.32.
[15] 21 CFR 100.16.
[16] 44 FR 22755.

estimated, for example, the expected numbers and costs of X-ray systems which would be marketed under the new performance standards. Upon reviewing 3 years of experience with the regulations for diagnostic X-ray systems, BRH realized that their earlier estimates of certain costs and benefits were incorrect. In **1978,** using data not previously available, BRH conducted a second analysis of costs and benefits derived from the 1972 regulations. On the basis of this second analysis, BRH concluded: 17

1. The provisions of section 1000.16 that are effective after August 1, 1979, will affect only a small fraction of the uncertified X-ray systems currently in use.
2. The total impact of this regulation, in terms of increased cost for the X-ray equipment or interruption of health care delivery, may be significant.
3. For those uncertified systems that would be affected, by virtue of their sale and relocation, section 1000.16 is not likely to be a cost-effective approach to improve the radiation safety performance of X-ray systems.

Based on these conclusions, BRH, through the rulemaking process, modified its earlier regulations by, among other things, permitting the installation of uncertified components into existing systems whose components are all uncertified and permitting the continued reassembly of uncertified equipment. It also clarified certain aspects of its performance standard for X-ray systems. 18

[17]1bid.
[18]144 FR 49667.

Incorporating Costs-Savings Studies Into Vaccine Approval Decisions: In its recently published report on viral and rickettsial vaccines, FDA's Panel of Review of Viral and Rickettsial Vaccines identified economics as a major consideration in the evaluation of vaccines .19 The panel noted that because adequate data are often lacking, economic considerations are often ignored in such evaluations.

The panel stated:

> These data would contrast the cost of a vaccine and its administration plus the costs (medical care, rehabilitation, impairment of ability to earn income) of vaccine-related disease with costs of a similar nature that would have accrued from cases of the natural disease.

The panel further stated:

> For an "acceptably safe" and effective vaccine against a serious disease, , . . the ratio should be highly favorable. However, if the preventable disease occurs chiefly in young children and is infrequently associated with permanent sequelae, a different answer might result. The question then might become "how much cost can be justified to prevent one crippling or lethal case of disease?" This clearly requires societal rather than scientific judgment.

This panel has strictly an advisory capacity within FDA, and its reports do not reflect official FDA policy, The panel's discussion of economics in the vaccine evaluation process, however, does illustrate some concern among FDA's advisors for evaluating the potential economic impacts of vaccines.

[19]45 FR 25665.

POTENTIAL APPLICATION OF CEA/CBA

In order to facilitate the discussion presented in the next section of this chapter on the implications of including cost-effectiveness criteria in the market approval process, OTA has developed the hypothetical model of a cost-effectiveness analysis (CEA) outlined below. It is very important to keep in mind that the use of the model for illustrative purposes does not negate or diminish any of the weaknesses or possibilities for misuse involved in formal cost-effectiveness analyses / cost-benefit analyses (CEA / CBAS) that seek a single, quantitative bottom-line, such as a cost-effectiveness ratio. Such a ratio is used in the model because 1) it points out some of the data and other problems related to use of bottom-lines, and 2) it is possible, given the quantitative nature of FDA's regulatory process, that use of a CEA ratio might be seriously considered by the agency if CEA were added to its mandate.

OTA's model is simplistic by intent and does not address the numerous assumptions and variations that are possible. Several caveats and assumptions should accompany an analysis such as the one presented. Potential methodological problems associated with the use of CEA in general are explained in detail in a background paper of this assessment.[20] Additional problems with its use in market approval processes are explained below. One problem, for example, is that data for some important variables would be difficult, if not impossible, to obtain.

In assessing a drug's cost effectiveness, FDA might first assess the agent's efficacy and safety and quantify its effects into measurable units of "net health effect. " Let us assume, for example, that an NDA is submitted for applicant drug "D," which is used to treat high blood pressure (hypertension). Let us also assume that in premarketing clinical trials, drug "D" consistently lowered by 10 percent blood pressure in 50 percent of tested hypertensive patients, and that such a drop in pressure could be correlated with a 5-percent reduction in morbidity and mortality (e.g., heart attacks, strokes, and kidney disease). Suppose drug "D" also produced undesirable side effects that in premarketing clinical trials accounted for a 2-percent increase in days of disability in the tested population. Let us also assume that the tested population accurately represents the general hypertensive population.

To calculate the "net health effect" of drug "D," all such positive effects (e.g., 5-percent reduction in mortality) and negative effects (e.g., 2-percent increase in disability caused by side effects) would have to be converted into a uniform and measurable unit of health .21 The "net health effect" of drug "D" could then be calculated for each indication listed on the proposed drug labeling (e.g., treatment of moderate hypertension in ambulant patients). Specific conditions of use (e. g., in conjunction with other drugs) and peculiar effects in special populations (e. g., the elderly) could be accounted for in the calculation of net health effects.

Once the drug's effects were converted into measurable units of "net health effects, " the "net cost" of achieving a desirable level of health effect (e.g., a 5-percent reduction in mortality and morbidity) would have to be calculated. A "net cost" could include such items as the cost of purchasing the drug, the cost of treating drug-induced side effects, and perhaps the cost of treating other illnesses in the persons whose lives are saved by the use of drug "D. " Subtracted from such costs could be savings resulting from any reduced costs of hypertension treatment (e. g., lower use of previously approved drugs *or* decline in hospitalizations and in physician office visits) resulting from the use of applicant drug "D."

At the conclusion of this phase of the analysis, one could construct a ratio of net cost in dollars to one unit of "net health effect" achieved through the use of drug "D" in the treatment of hypertension, e.g.:

$$\text{Cost-effectiveness ratio} = \frac{\text{Net cost}}{\text{Unit of net health effect}}$$

In the next phase of the analysis, the net cost (in dollars) of achieving a desired net health effect through the use of drug "D" would be compared with the net cost of achieving the same net health effect by using an existing approved treatment modality (e.g., another drug, surgery, or biofeedback) to lower blood pressure. Such a comparison of costs would require that cost-effectiveness ratios, i.e., net cost (in dollars)/unit of net health effect, be derived for each hypertension treatment modality. The following hypothetical ratios for example, might be derived:

Hypertension treatment modality	*Net cost/uniform unit of net health effect*
Applicant drug "D". . . .	**$400**
Approved drug "A" . . .	$250
Surgical procedure "X" .	**$3,000**
Biofeedback	$100

Let us assume that no other treatment of hypertension was available at the time of the analysis.

The final phase of such an analysis would be to establish criteria for judging the cost-effec-

[20] *Background Paper #1: Methodological Issues and Literature Review,* prepared by OTA.

[21] As discussed in *Background Paper #1: Methodological Issues and Literature Review,* some analysts have used the unit termed quality-adjusted life year for this purpose (652).

tiveness ratios for each treatment modality and for using such ratios to help determine if applicant drug "D" should be approved. In the example above, if a criterion for market approval were that the applicant drug had to produce one unit of "net health effect" at a cost lower than the cost of using any other approved antihypertensive drug, then drug "D" might not be approved. If such a criterion included comparison with other approved forms of hypertension treatment, then drug "D" would presumably be approved if compared with surgery, although it might not be approved if compared with biofeedback. When one considers in this hypothetical evaluation process the need for a variety of available treatment modalities to meet individualized patient situations, then the relatively small differences in cost effectiveness between applicant drug D, biofeedback, and approved drug A become much less important.

Cost effectiveness might be used in formal review processes of drugs that have been marketed for a number of years, instead of those for new drugs. FDA conducts at least three such review processes, and to date, the Agency has not used cost effectiveness in any of them. First, in its drug efficacy study implementation project, FDA is conducting a one-time review of the efficacy of drug products approved between 1938 and 1962 (131). The Agency categorizes products according to their documented clinical efficacy, has removed from the market some products for which efficacy documentation is lacking (e.g., selected fixed-dosage antibiotic combination products), and is attempting to remove other such products. Second, in its Over-the-Counter (OTC) Drug Review Program, FDA is conducting a one-time review of the safety and efficacy of several hundred ingredients used in OTC or nonprescription drug products. Third, FDA (technically, the Secretary of Health and Human Services) has authority to remove from the market a drug that represents an "imminent hazard" to the public's health. When this authority was exercised to remove the antidiabetic drug phenformin from the market, FDA extensively reviewed the drug's safety and to some extent its efficacy.

IMPLICATIONS OF CEA/CBA IN MARKET APPROVAL: GENERAL FINDINGS

As described in chapter **2,** CEA is an analytical device that can be used to help decisionmakers allocate resources, usually in the public sector. The primary purpose of FDA's drug and medical device market approval processes is to protect the public from unsafe and ineffective products. FDA's marketing approval processes do, however, indirectly influence the allocation of public resources in at least two ways. First, the types of regulations and procedures established by FDA affect the allocation of its operating budget. Second, many—if not most—of the drugs and medical devices approved by FDA eventually are used in health care services that are paid for through publicly financed programs, such as medicare and medicaid.

The use of cost effectiveness as a criterion in FDA's market approval processes for drugs and medical devices would require a substantial shift in the Federal Government's approach to regulating the medical care marketplace. At present, no Government effort is designed explicitly to reduce medical expenditures by directly slowing down or stopping the market introduction of medical goods and services. The Federal Government does not extensively evaluate the cost effectiveness of drugs or medical devices in any of its efforts to contain health care costs. Conceivably, such evaluation could take place in the National Center for Health Care Technology, which advises the Health Care Financing Administration (HCFA) on reimbursement policies for selected medical technologies. Results of CEAS involving drugs and medical devices could be incorporated into HCFA'S policies for reimbursing the use of selected drugs and medical technologies under medicare, medicaid, and any other federally operated health insurance program.

Potential Positive Effects

There are at least two hypothetical positive effects of incorporating CEA into FDA's market approval process for drugs and devices.

First, manufacturers could formally incorporate cost-effectiveness criteria, based on societal values, into their research, development, and marketing strategies. Manufacturers most likely do use some form of CEA/CBA to allocate their R&D expenditures. The primary criteria used in such allocations, however, may emphasize such items as total sales, market portions, and return-on-investment. If FDA used criteria such as reduced treatment costs, improved levels of health, and improved efficiency in disease prevention or treatment, then more manufacturers might develop new products and seek new markets where existing treatment or prevention measures are ineffective or inefficient. Some industry representatives claim that the leading drug research firms currently include such public health criteria in their research priorities. The existence of orphan drugs (i.e., existing agents for which there is a small, demonstrated clinical need but no manufacturer), however, indicates that at least for certain products, profitability takes precedence over societal need. Conceivably, such use of CEA could help the Government encourage participation by drug and medical device manufacturers in public efforts to develop more cost-effective medical technologies.

Second, if FDA were able to accurately assess the cost effectiveness of an applicant medical device or drug, the Agency might help reduce expenditures for inefficient products by keeping them off the market entirely. By evaluating the cost effectiveness of medical devices and drugs in the market approval process, the Government would be assessing a product very early in its diffusion process. Perhaps this early evaluation process would lead to better direction—and perhaps an expansion—of experimentation with new drugs and devices. At present, substantial non-FDA-approved clinical experimentation with newly approved drugs takes place in uncontrolled situations. Such early evaluation, however, would have no effect on the inefficient use of drugs and medical devices found to be cost effective in clinical trials.

Potential Problems

Among the consumer advocates, FDA employees, and representatives of the pharmaceutical industry surveyed in this assessment, there appeared to be widespread agreement that cost effectiveness is not an appropriate criterion to use in the drug and medical device approval process. Using information and ideas obtained from several individuals, OTA developed the following analysis of potential problems.

First, the market approval process may be too early in the life of a drug or medical device to evaluate its cost effectiveness; information regarding a product's safety and efficacy are usually available for only one or two indications of use at the time a sponsor submits an initial NDA for a new drug *or* an application for approval of a new device. Data regarding a drug's safety and efficacy in medical conditions not listed in the product's official labeling are not often generated until a product has been marketed for at least a few years. If a new drug or medical device were not approved for marketing because its cost effectiveness did not compare favorably with already approved products, then the new product might never be fully evaluated, particularly in the treatment of medical problems other than the one(s) studied initially. The total benefits, risks, and cost effectiveness of drugs that are used in the treatment of more than one medical problem often may not be known for several years after the product's initial development. Examples of such products include propranolol used in the treatment of angina, migraine headaches, and hypertension; selected antibiotics used in the treatment of acne; amantadine used in the treatment of parkinsonism; and phenytoin used in the treatment of certain cardiac arrhythmias. In addition, rare or delayed-onset adverse drug reactions, for example, drug-induced cancers, would not likely be included in premarketing CEA.

James B. Russo, SmithKline Corp., offers an industrial concern about the use of CBA in the market approval process for drugs **(529):**

> Our primary concern is over the fact that cost-benefit analysis in the drug area is a new discipline, and one which simply must not be widely

> applied, particularly in the area of new drug approval, until we have real confidence in its predictive reliability . . . I don't really know how to state that point as strongly as I'd like to. Think back to probenecid. The drug was developed to slow the excretion of penicillin from the kidney, because in those days penicillin production was inadequate. By the time probenecid was shown to be safe and effective for that purpose, penicillin was coming out of the industry's ears. Had that NDA been looked at on the basis of the product's cost against its possible benefits in prolonging blood levels of penicillin, work on the drug would have been stopped in the early '50s. Of course, once it was found that it speeded the excretion of uric acid, an entirely new and a relatively important means of relieving gout became a possibility, It isn't simply a matter of NDA approval. If we knew we would have to pass that kind of test at the NDA stage, I fear that a lot of projects would be cancelled long before we had practical information on the drug's full potential.

In recent years, however, FDA has approved very few NDAs to add to a previously approved product's official labeling a new major indication that required data from new clinical trials. Between January 1, 1974, and September 30, 1979, for example, FDA approved 4 such NDAs out of a total of 484 total NDA approvals for all reasons (221). There are, however, 362 active commercial INDs for products being used for indications not currently listed in the products' official labeling. In addition, for perhaps hundreds of approved products, FDA has permitted manufacturers, through the supplemental NDA process, to make minor modifications (i.e, those not requiring manufacturers to sponsor new clinical trials) in their products' official indications for use. The extent to which marketed drugs are used for unapproved indications is not known. Manufacturers often have no economic incentives to seek FDA approval for popular, unofficial clinical uses of their products.

Second, the calculation of costs needed to assess the cost effectiveness of a new drug would require FDA to either: 1) use existing estimates of costs of treating illnesses using alternative forms of therapy, or 2) generate new data bases. Problems encountered in using existing data bases are discussed in a background paper of this assessment .22 At present, FDA has no intramural capability for generating new data regarding the treatment costs for selected diseases. Conceivably, FDA could ask or require drug and medical device manufacturers' to submit estimates of the costs of treatments provided during the clinical testing of a new drug. Meaningful estimates of treatment costs might be difficult to calculate, however, because the prices charged for medical care are dynamic and can vary substantially among geographical regions.

At the time FDA approves a drug or medical device, it does not know—nor can it influence—how much a manufacturer will charge for its newly approved product. FDA could ask a manufacturer to estimate a product's selling price. Apparently, however, this task would be difficult for manufacturers to accomplish at the time of market approval. Further, the price of a drug or medical device will likely change over time, and cost-effectiveness information calculated at the time of market approval would likely change.

Third, the effect on competition of using cost effectiveness as a criterion for market approval of drugs and medical devices is unknown. Potential implications vary substantially depending on how FDA might use the cost-effectiveness criterion.

If FDA assumed the responsibility for conducting CEAS, theoretically manufacturers would not be burdened with the expense of developing the capability to conduct such analyses. Most research-based companies, however, would likely establish their own capabilities; at least two pharmaceutical firms currently perform CEAS on some of their products. One company, Merck Sharp and Dohme, developed a computerized cost-benefit model to illustrate the costs and benefits of its pneumococcal vaccine (464). Another pharmaceutical manufacturer, SmithKline and French, has extensively studied the costs and benefits of the use of one of its drugs, cimetidine, in different populations (529). If FDA required each sponsor of every

[22] *Background Paper #1: Methodological Issues and Literature Review,* prepared by OTA.

new drug or medical device to conduct CEA as a part of the premarketing approval processes for drugs and devices, then the use of cost effectiveness would add to the financial and regulatory hurdles of new product introduction. Conceivably, that situation could reduce manufacturers' willingness to bring a new product to market and could reduce competition.

If FDA required only the original manufacturer of a new drug or medical device (i.e., one not previously marketed in the United States) to analyze the cost effectiveness of its product, then the burden of conducting such analyses would fall primarily on the leading innovating companies.

If FDA used cost effectiveness as a criterion for the marketing of only new therapeutic entities, and not for either generic or "me too" products,[23] and if manufacturers perceived such a task as too expensive, then manufacturers might attempt to break into existing markets for multiple source products rather than to develop new drugs and create new markets.

If FDA applied cost-effectiveness criteria to "me too" type products, then a manufacturer might lower the introductory price of its new product in order to make it compare favorably to already-approved products in the same therapeutic category. Once a product was marketed, however, its manufacturer could alter its price, and FDA has no authority to control the price of approved drugs and medical devices. Excessive increases in postapproval prices would likely be limited by competition in the marketplace for similar products.

Fourth, the use of CEA to evaluate new drugs and medical devices would require extensive resources, substantial time, and creative application of existing data. FDA would likely have to compare the relative safety, efficacy, and cost of an applicant product with those same characteristics of marketed products. In order to perform such comparisons, FDA would have to do the following:

- quantitatively assess the safety (risks) and efficacy (benefits) of each marketed drug and medical device;
- establish standards for clinical efficacy, safety, and cost of each available form of treatment—and possibly prevention—in numerous disease states (note: such standards could be incorporated into a monograph system such as those used for OTC drug products and antibiotics); and
- calculate cost-effectiveness information for the use of each drug and medical device in specialized populations, such as the elderly and persons with specific medical problems.

Although it is debatable whether FDA has statutory authority to evaluate new products relative to currently marketed products, a provision in the Senate-passed version of the Drug Regulation Reform Act of 1979 would allow FDA to consider formally and explicitly the "benefits and risks of available therapies" when evaluating applicant drugs in the NDA review process.

Fifth, the cost effectiveness of different products would vary substantially depending on the forms of treatment being compared. A comparison between a new drug and a surgical procedure in the treatment of a selected medical problem, for example, could yield large differences in cost-effectiveness ratios. Such a comparison might be useful to FDA in its evaluation of the drug. The information yielded could be helpful in the evaluation of the new product, especially if the product were a new chemical entity that represented a therapeutic breakthrough. Ultimate treatment for uncontrollable malignant hypertension in a young person, for example, has been the removal of one or both kidneys (nephrectomy). Until recently, many cases of this disease have not been controllable through the use of drugs. A potent new antihypertensive drug, minoxidil, has been shown to effectively lower very high blood pressure, and in some studies, its use reduced the need for nephrectomies **(482).** There is no other product on the market that resembles minoxidil either chemically or therapeutically. When compared to sur-

gical removal of the kidneys, minoxidil would likely be quite cost effective for some patients.

Comparisons between two drugs, however, especially two drugs with similar therapeutic effects, would likely yield only small differences in cost-effectiveness ratios. Small differences would be of little value. Thiazide diuretics, for example, represent another form of antihypertensive drug therapy. There are approximately 30 different single-entity thiazide diuretic products on the market, and **each one** produces very similar therapeutic and adverse effects. In cost-effectiveness comparisons among these 30 products, the differences in cost-effectiveness ratios would likely be very small; hence such comparisons would likely be of little distinguishing value in FDA's NDA review process for a new thiazide diuretic.

Sixth, the cost effectiveness of a drug or a medical device might be influenced more by the conditions of its use than by its demonstrated efficiency in premarketing clinical trials. Factors such as dosage regimen, route of administration, and palatability (taste) all could influence a product's acceptance by patients and hence affect its cost effectiveness. Even if FDA were able to accurately assess the cost effectiveness of a drug or medical device at the time of market approval, such an assessment might not accurately predict the efficiency of the product in general use.

Seventh, if FDA used cost effectiveness as a criterion in its drug and medical device market approval processes, the Agency would have to rely on speculative and uncertain data for two important valuations. First, FDA measures the safety and efficacy of drugs and medical devices in terms of changes in physiologic functions, not in terms of changes in a person's overall health status. The efficacy of an antihypertensive drug, for example, is evaluated on the degree to which the drug reduces a person's blood pressure. FDA does not quantify the effect a drop in blood pressure would likely produce in the health status of a hypertensive patient. Second, FDA analysts would face the challenge of assessing the economic value of physiological changes—and if possible of those in health status—among persons using the medical device or drug.

Eighth, by using cost effectiveness as a criterion in the market approval process for drugs and devices, FDA might be extending its role beyond the scope of responsibilities Congress intended the Agency to have. FDA's primary purpose is to protect the public from unsafe and ineffective drugs and medical devices and from unsafe cosmetics, foods, and food additives. Thus far, Congress has not asked FDA to use economic criteria to regulate the choice of safe and effective products during the market approval process. Choices based on economic criteria are left to be made by consumers, health care practitioners, hospital administrators, and private and public health insurance carriers after a product has been marketed.

9 .

R&D Programs

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9. R&D Programs

INTRODUCTION AND BACKGROUND

The Federal Government supports a wide range of health R&D activities. The object of this research, no matter what its form, is the production of knowledge. Some research is designed to yield information on health itself, or on diseases and disabling conditions. Some is designed to produce new tools, technologies, to intervene in disease processes, or to counteract the effects of disease. Some research evaluates those tools; other research investigates the use of technology and other aspects of the health care delivery systems. Much of the existing research serves multiple purposes, and some yields results that are more valuable to other fields than to the field the research was designed to address.

R&D have given the health care system and this country a great deal of beneficial information and many effective technologies, but are activities fraught with uncertainties. These activities can also be expensive ones—billions of dollars are spent on health R&D each year in this country by the Federal Government alone. The expenditure of Federal funds for R&D is an investment in the future—and much of this investment represents a public good. Therefore, it is important that moneys for health R&D be spent as wisely as possible and in accord with a balance between public and scientific priorities.

R&D support by Federal agencies can be viewed from two perspectives: process and substantive. The former involves questions of what methods are used to make administrative decisions regarding selection of priorities, program direction, or projects to be funded within each agency. The issues relate to selection and pursuit of goals and to the methods employed to assist in efforts to allocate resources. The latter perspective, "substantive" performance, involves questions and issues pertaining to the agencies' performance or sponsorship of research that examines the allocation of technological or other resources in the health care system.

Since this OTA assessment focuses on the use of cost-effectiveness analysis/cost-benefit analysis (CEA/CBA), the chapter concentrates on questions dealing with that analytical technique. Four Federal agencies' activities in regard to CEA/CBA from both of the aforementioned perspectives are examined. If an agency does not support or use CEA/CBA, the methods and procedures it does use are discussed briefly. Although the distinction between the process and substantive perspectives of research support is at times unclear, an attempt is made in the discussion below to separate them as clearly as possible and to note the similarities when the situation requires.

Research Classifications.—Health R&D is an umbrella term used to describe a wide range and diversity of activities. A single scientist focusing on an extremely narrow topic within the field of biomedical science is doing health research. The same can be said of the analysts that are examining the performance of **a** patient screening program, developing physician productivity measures, or working on any number of other health care issues.

To help describe the primary focus of health-care-related R&D, the range of activities is often broken into four loosely defined categories: 1) basic research, 2) applied research, 3) application (or transfer) research, and 4) health services research. The demarcations between these four research categories are not clearly defined. Nevertheless, the classifications play an important role in the process of setting health care research priorities, allocating and distributing funds, and evaluating outcomes or products of R&D efforts. At several levels in the health care decisionmaking and policy process, the intended purpose of a given research effort is im-

portant. There is constant tension in the decisionmaking process between those who advocate the allocation of funds for increased support of basic research, those who feel more work is needed in applying more fully the knowledge and technologies that exist (application or transfer research), and those who cite a pressing need to examine what is already in place and how well it is working or how to make it work better before adding more to the system (services research). As a result of these different perceived research needs, the research "label" that is affixed to a given health care program or initiative can be quite important to its ultimate success in the resource allocation process.

For purposes of the discussion below, the Federal health care R&D effort is divided into two broad general categories: 1) biomedical research, and 2) health services research. Biomedical research includes basic, applied, and, to a degree, application or transfer research. Health services research includes work done on technologies or systems that are still considered to be in the development, transfer, **and application stages, as well as on technologies or systems that are in widespread use. Actually, the lines of definition in terms** of where biomedical research activities end and where health services research begins are rather blurred. This is because it is rare for a technology or innovation to proceed in a linear process from basic research to widespread application. It is also unlikely that a single agency or other institution will have an innovation under its purview for the full range of developmental needs that are part of producing a usable end product.

In the discussion that follows, the National Institutes of Health (NIH) is used to represent the biomedical research process. The National Center for Health Services Research (NCHSR) and the Health Care Financing Administration (HCFA) are the examples of Federal agencies that sponsor health services research. And the National Center for Health Care Technology (NCHCT) is the example of an agency whose activities incorporate or relate to both types of R&D.

BIOMEDICAL RESEARCH—NIH

NIH is not the only Federal agency to conduct or fund biomedical research. The Veterans Administration, the Office of Naval Research, the National Science Foundation (NSF), the Department of Energy, and others are involved to varying degrees in a range of biomedical research activities. NIH, however, is by far the largest single provider of biomedical research funds in the United States, NIH covers a wide range of scientific activity and uses peer review, as well as program and project evaluation, processes that are similar to those of other Federal agencies that support health care R&D.

NIH is an agency of the Public Health Service (PHS) in the Department of Health and Human Services (DHHS). Its mandate, stated in broad terms, is to improve human health by increasing understanding of the processes underlying health and acquiring new knowledge to prevent, detect, diagnose, and treat disease and disability.

NIH pursues this mission via an array of intramural programs conducted at NIH and through an extensive network of extramural grants and contracts to private and public institutions in the United States and other countries. Its budget in 1980 will be approximately $3.4 billion, which represents approximately 68 percent of the Federal obligations for health R&D. Forty-one percent of total national health R&D support (Federal and State Government, industry, and private nonprofit organizations), is provided by NIH (1978 estimates) (446).

NIH is organized into 11 institutes, the National Library of Medicine (NLM), and 6 research and support divisions. Two of the institutes (the National Cancer Institute (NCI) and the National Heart, Lung, and Blood Institute (NHLBI)), as well as NLM, have "bureau" status; the other institutes are "division" level organizations. These various semiautonomous orga-

nizations are coordinated through the Office of the Director of NIH.

There are several levels of control and input in the resource allocation process for biomedical research. * Every year, NIH is subjected to numerous examinations of its allocation of research support, its selection and implementation of research priorities, and its requests for funds for the upcoming year(s). This process extends from congressional hearings on NIH budget authorization and appropriation levels to the study section advisory groups that meet three times each year to evaluate the technical and/or scientific merit of research proposals. Furthermore, it is not unusual for an ad hoc presidential or congressional panel, commission, or task force to express its opinions and conclusions regarding the quality, quantity, or usefulness of NIH's efforts. These "advice-giving" groups can carry considerable weight in the priority-setting and allocation process. Another group that can affect the amount of support given a particular research area are the scientists themselves. The thousands of scientists who continue to initiate and support various types of research, submit grant and contract proposals, and remain in an area of research for extended periods of time can have considerable bearing on national research priorities,

Congress plays, and has played, a significant role in the creation, expansion, and contraction of research efforts at NIH, Its budget-setting and oversight authority are powerful levers in the decisionmaking and allocational process. Strickland (586,587), Ward (612), and others (91,367) have noted that medical research is a national policy issue that entails all the political pushing and shoving that is characteristic of other national allocational issues. The allocation of funds for medical research is neither just a budgeting exercise nor a purely scientific decision that is, or will be, made only by the scientific community. In part, this is because medical research, technological advances, new treatments and cures, and the health care research system itself have come under the scrutiny of the general public. Reader's *Digest*, Ann Landers, [2] Marcus Welby, M. D., disease-oriented interest groups, and others have turned millions of Americans into supporters or critics of various aspects of biomedical research, in particular, and the health care system, in general.

Congress is in the position, both constitutionally and politically, to exercise a great deal of control over the priority-setting and allocation process at NIH through its "power of the purse." Maldonado sums up congressional influence via "the appropriating art" (379):

> Health budget review and development fall under the jurisdiction of the subcommittees on Labor-HEW (of the House and Senate committees on appropriations). The subcommittee on Labor-HEW has authority* to (1) recommend the appropriations or funding level in support of program or research activities (or their termination); (2) through the report vehicle, earmark funds for specific programs or projects, set program directions, instruct, warn, and exhort; (3) accept or reject proposed impoundments (rescissions and deferrals); (4) approve or disapprove transfer of unexpended balances; and (5) conduct studies and examinations of agency/department operations and organizations.
>
> The appropriations subcommittees have traditionally played a prominent role in health policy by "earmarking" funds for specific activities, and "requesting" or "expecting" that a certain "emphasis" or direction be taken.** In summary, they set program direction, emphasis, and budget levels for health.

A number of other forces operate both within and outside Government to ensure that a given health care issue receives attention in the deci-

[1] NIH is frequently praised for its contribution to the fields of science and medicine, and its achievements in these areas have been described as extraordinary. Yet in recent years, the charge has been leveled that its accomplishments have not been great enough given the large sums of money that have been channeled its way. It is beyond the intent or capabilities of this study to enter this debate.

[2] An example of the tremendous response that can be generated by "popular" authors, TV shows, publications, etc., is given by Robert Q. Marston regarding the debate between those who did not want a "cancer crusade," and those who did. He cites the fact Ann Landers stimulated more than a million responses to a statement in one of her columns in 1971 asking for support of the cancer crusade (382).

● W. H. Brown, *Ru/es of the House of Representives* pp. 349-351, 393, 396-399, Washington, D. C.: U.S. Government Printing Office, 1975 (71).

* *R. F. Fenno, *The Power of the Purse: Appropriations Politics in Congress*, Little, Brown, Boston, 1966. (199).

sionmaking process. The executive branch can marshal] a considerable collection of expertise—from NIH, the Food and Drug Administration (FDA), HCFA, special task forces of experts, and numerous advisory councils that are part of the R&D process—to provide advice to the policy process. The scientific community, the many disease-oriented organizations (e.g., the American Cancer Society, the Cystic Fibrosis Foundation) and professional organizations (e.g., the American Medical Association, the American Hospital Association) provide a mix of voices that add to the diversity of views on various health care issues. At any time, on any given health care issue, there are likely to be coalitions within both the legislative and executive branches of Government that have the support or opposition of the many nongovernmental interest groups—all urging special consideration for their programs on the research agenda. For a detailed description of this process, see Rettig's (510) and Strickland's (587) accounts of the "war on cancer" declared in 1971 by President Nixon.

Rarely are formal decision-assisting techniques, especially CEA/CBA, explicitly used to make decisions at this broad political or societal level of the policy process. Although the preceding discussion has been based on NIH, much of what was said about decisions at this policy level pertains to the health services research system, as well.

Peer Review Allocation and Evaluation Mechanisms

NIH and each institute within NIH must decide how to divide available resources among: 1) extramural grants, 2) contract research, and 3) intramural projects initiated by scientists within NIH. In the case of extramural grants, further consideration must be given to the allocation of resources for investigator-initiated research grants and large, complex multidisciplinary research team efforts such as center and program project grants. To some extent, NIH priority-setting and research selection is based on the relative merits of basic, applied, or transfer research in each institute's area and the budget the institute will receive.

The mechanism evolved at NIH to manage these many considerations is a peer review system. Most, if not all, of the Federal agencies that support health care research rely on some form of peer review to solicit expert opinion regarding the potential success of a proposed project. The peer review system of NIH consists of **2,200** primarily non-Federal scientists and lay advisors from across the Nation. These individuals are grouped into **130** peer review groups, advisory committees, councils, and panels (449), whose function is to provide NIH with expert opinions both on the scientific and technical merit of grant applications and contract proposals and also on program initiatives and policy issues.

Extramural Grants

The peer review system for grant applications used by NIH is based on two sequential levels of review, referred to as the "dual review system." The first level involves panels of experts established according to scientific disciplines or current research areas for the primary purpose of evaluating the scientific and technical merit of grant applications. In the Division of Research Grants (DRG), discussed below, the panels are called study sections. Generally, however, the panels are referred to as initial review groups (IRGs).

The second level of review is by a national advisory council or board, referred to here as a "council." Council recommendations are based not only on considerations of scientific merit as judged by IRGs, but also on the relevance of a grant application to an institute's programs and priorities.

Receipt and Assignment of Applications.—Grant applications submitted to NIH are received centrally in DRG. This Division, one of the research and support divisions at NIH, is not connected to the research institutes or responsible for funding or managing grant programs.

DRG screens incoming grant applications, determines the relevance of each application to the overall mission of NIH, and assigns acceptable applications to an appropriate IRG and to an

appropriate institute.[3] Assignment to an IRG is based on the complementarily of a proposed research project to the review responsibilities and scientific expertise of IRG's members; assignment to an institute is based on the institute's legislatively mandated program responsibility. If the subject matter of an application is pertinent to the program responsibilities of two institutes, a dual assignment may be made, Should the primary institute decide not to provide funding, the other institute may consider the application for funding.

Initial Review by Peers.—Depending on the type of research proposed, the first level of scientific and technical merit review is by an IRG located either within DRG or within an institute. IRGs in the institutes are usually multidisciplinary and are thus constituted to review more complex program project and center grant applications. An NIH health scientist administrator serves as executive secretary of each review group.

IRG members, who serve up to 4 years per appointment, meet three times a year to review applications. When assessing the scientific and technical merit of an application assigned to their IRG, the members consider, among other criteria: the importance of the proposed research problems; the originality of the approach; the training, experience, and research competence or promise of the investigators; the adequacy of the experimental design; the suitability of the facilities; and the appropriateness of the requested budget to the work proposed (164).

For each application, IRG makes a recommendation of approval, disapproval, or deferral for additional information by majority vote. In addition, for each application recommended for approval, each member of IRG individually and privately records a numerical rating that reflects a personal evaluation of the scientific merit of the proposed research or training. The numerical rating is from 1.0 (the most meritorious) to **s.0** (the least meritorious), with 0.1 increments. After the meeting, the executive secretary averages the individual reviewers' ratings for each approved application and multiplies this by 100 to provide a three-digit number known as the priority score. Priority scores assist the staff of the institutes in determining which applications are to be funded.

If information is needed that is not in the application and cannot be obtained by telephone or mail, a project site visit may be made either prior to an IRG meeting or after an IRG deferral recommendation. In addition, site visits are often routinely made when an application involves complex coordination. In either case, the executive secretary assembles a team of site visitors. For a research project grant application, the site visit team generally includes two or more members of IRG, the executive secretary, a member of the institute staff, and usually one or more ad hoc consultants who are experts in critical aspects of the proposed work. For more complex grant applications (e. g., those for program project or center grants), the site visit team may include as many as 10 to 15 individuals, including members of IRG and ad hoc consultants.

After IRG meets, the executive secretary prepares a summary statement for each application and forwards it to the appropriate institute for review by its council. The summary statement contains a description and critique of the proposed activity, an explanation of the recommendation of IRG, a recommended budget, and notations about any special points (e.g., a split vote or a potentially hazardous experimental procedure).

Review by National Advisory Councils or Boards.—Each NIH funding unit has a council that must review and recommend action on the applications received from IRGs. These councils are mandated by law, and some have minimum levels placed both on the number of times they must meet each year and on the number of members they must have.[4] Members include au-

[3] The Referral Branch also *receives* and assigns applications to other agencies within PHS, such as the Alcohol, Drug Abuse, and Mental Health Administration, the Center for Disease Control, the Health Services Administration, the Health Resources Administration, and the Food and Drug Administration.

[4] For example, the National Cancer Advisory Board was established in August of 1937 by Public Law 244, 75th Cong., and has since been renamed and restructured by subsequent legislation that required that the board shall meet "not less than four times a year" (449).

thorities in scientific and health fields directly related to the program interests of the institute, as well as lay people noted for their interest or activity in national health problems. With the exception of individual fellowship applications and some grant applications recommended at yearly levels not exceeding **$35,000** (in NCI and NHLBI), grants cannot be awarded without approval by a council.

The councils review research proposals in a broader context than IRGs, because the councils' recommendations are based not only on IRG scientific and technical merit evaluations, but also on the needs of NIH and the missions of the individual institutes, the need for initiation of research in new areas, the degree of relevance of the proposed research to the missions of the institutes, and other policy issues (164). In addition, a major focus of the councils is the SATT[5] criteria that have been developed to guide and categorize the support for biomedical research.

The councils forward their recommendations for each application to the institute director for a decision on funding. Usually, the approved projects are chosen according to rank until the budget is obligated. An approved grant application is not assured of funding, because there are almost always more eligible applications than available funds. An unapproved application, however, cannot be funded (164).

R&D Contracts

Contracts are used to procure a specified research product or service from a nonprofit or a commercial organization. The initiative for these contracts generally comes from NIH program staff and advisors, who identify a specific research or service need. Workshops and conferences are a source of invaluable ideas and guidance.

Each institute has developed slightly different methods and procedures for using contracts to satisfy its research needs. The basic mechanisms the various institutes use to develop contract proposals, to review contract applications, and to evaluate the progress and outcomes of contract products, though, are similar enough to be summarized in a general description.

The scientific staff members within a given institute, with assistance from standing committees or ad hoc advisory groups, develop a research project description and plan. In compliance with the law that mandates peer review for NIH contract projects, the concept of the project is evaluated by a scientific review group composed largely of non-Federal advisors. The proposed project is then released as an RFP (request for proposal), which specifies the terms, conditions, and provisions for the requested contract. RFPs are announced in the *Commerce Business Daily* as required by law, in the NIH *Guide for Grants and Contracts* **as** required by policy, and in other appropriate publications as determined by program requirements.

In response to an RFP, applicants submit contract proposals, which are reviewed by the institute's contracting officer and then by a scientific review group consisting mainly of non-Government scientists chosen for their expertise in the relevant area. Their recommendations are sent to a contract review committee composed of senior program staff from the funding institute. Applicants determined to be in the "competitive range" have an opportunity to further defend or clarify their proposals via written or oral discussion with the contracting officer and senior program staff. Once the applicants have made their "best and final" offer, the remaining applications are reevaluated via further negotiations in order to determine the one to be funded.

In addition to contracts solicited by NIH, unsolicited contract proposals are also occasionally received by DRG, and then sent to the appropriate institutes. If the unsolicited proposal is relevant to the institute's program needs, it is reviewed by the contracting officer and scientific review group in a process similar to that for solicited proposals.

5, stands for Science Base activities. This category is, for all practical purposes, basic research support. A is clinical *Application,* research focused on intervention. It is at this level that clinical trials take place. *T* represents the *Transfer* research that NIH undertakes to move products or interventions that have survived testing to the consumers and providers of health care. Demonstrations, practical trials, and consensus development conferences are part of the transfer process. The final T stands for the *Training* function that NIH supports. This effort is geared to supporting and attracting people into the field of research.

The progress and products of contract research are under the supervision and review of a project officer at the funding unit. Informal, as well as formal, procedures are used to monitor the performance of the contract project. A major difference between contract research and grant and intramural research, at least theoretically, is that contractors are required *to* provide an end product based on specifications established by the funding unit before the research begins. The other forms of research support are usually not as tightly bound by requirements to produce a given outcome at the end of their research.

Intramural Research

Intramural research projects are developed, supported, and evaluated by a mixture of institute staff and outside advisors. Each institute has an in-house scientific board that meets on a regular basis to set institute policy, review institute programs, and discuss research needs. For each institute, the scientific board also identifies future goals, needs, and capabilities for intramural research.

Intramural research ideas or project proposals can be initiated by the scientific board, by individual researchers, laboratory chiefs, or by scientific directors. Research proposals are discussed by in-house scientific staff, the institute's scientific board, and outside experts if needed. Depending on the available space, personnel, and budget, and the feedback from this informal peer review process conducted in-house, a specific project is started, rejected, or deferred.

Additional aspects of the system are publication of research in journals and presentation of work to in-house staff and the scientific community. Directors from each institute meet each month to review the work of selected nontenured researchers who are candidates for tenured status at NIH. Finally, each institute has a Board of Scientific Counselors, composed of non-Government scientists, that meets twice each year to review intramural projects and programs for scientific performance and progress.

Discussion of Peer Review Mechanisms

The peer review mechanisms at NIH, by and large, appear to have worked quite well over the last several decades. This is not to say that the peer review system has been beyond criticism or change. There have been a score of studies, hearings, reports, and reviews on the peer *review* process at NIH over the last 25 years, and these many assessments have led to reevaluations and modifications of the procedures used to conduct the research support processes. [b] Despite these changes, however, the fundamental concept and framework of the peer review system at NIH remain intact. Furthermore, the peer review system remains the mechanism of choice for efficiently and effectively allocating research resources.

The research and resource allocation process, both in biomedical and health services research, is an uncertain endeavor that entails probability and risk. The peer review process is an attempt at predicting the probability of success for a given scientific effort. Through this process, NIH and the other research-supporting agencies attempt to estimate or predict a subjective level of quality performance of a researcher, given the very real confines of a budget, a limited time frame, the existing knowledge and technological base, the availability of trained researchers willing and able to work on a given problem, research space and resources, and the presence of often conflicting and changing health care goals and policies. In **a** very real sense, the peer review process is an attempt, either explicit or implied, to select and support "cost-effective" research.

[b] Since 1947, there have been approximately 18 official reviews, studies, inquiries, hearings, and reports examining the adequacy of the peer review system at NIH (447). The most recent study to assess the mettle of the peer review system was supervised by Dr. Ruth L. Kirchstein, Director of the National Institute of General Medical Sciences. The study team received its go ahead in April of 1975 and submitted phase I of the report to the Director of NIH in December of 1976, and the final report, phase II, in December of 1978 (445,447,448). For additional views on the peer review system, see *Report of the President's Biomedical Research Panel* (491); *Opinions on the NIH Grants Peer Review System, Phase II of the Report to the Director* (447); *Investigation of the National Institutes of Health* (115); and "Support of New Principal Investigators by NIH: 1966-1972" (153).

Performance Evaluations

Even though NIH very rarely uses or funds CEA/CBAs in either the allocation or evaluation process (see survey of CEA/CBA use at NIH in app. B), the various institutes do employ a number of analytical techniques to assess the performance of the research process. Most of the techniques are employed after a "product" has been turned in. It is important to note, however, that allocation and evaluation efforts at NIH often merge into the same function. In the minds of most NIH administrators and program and project leaders, evaluation activities are ongoing efforts that cannot be clearly or neatly separated into the categories of planning, allocation, and oversight. A number of the techniques that NIH, other research-supporting agencies, and analysts in general use to evaluate the performance of research efforts are described in the following paragraphs.

General Assessments of Biomedical Research

Citation Analysis.—A form of performance evaluation that has gained considerable attention over the last decade is the use of citation analysis. This analytical technique examines the quantity and, to a degree, the quality of scientific papers, reports, articles, and so on, that result from research projects. NIH, NSF, and others have used this technique to evaluate the performance of their research efforts. NIH and NSF have supported a number of studies over the lasts years that tested: 1) the correlation between their support efforts and biomedical publication output (225,425); 2) the extramural role of NIH as a research support agency (426); 3) the relationship between the peer review system, citations, and biomedical research policy (86); and 4) other aspects of research support (565). Comroe and Dripps (120) and the Department of Defense (565) have employed a variation of citation analysis to evaluate the development of specific scientific and technological innovations.

Morbidity/Mortality Studies.—Another type of performance evaluation is the attempt to measure the reductions in mortality and/or morbidity that have occurred as a result of biomedical research and health care in general. Morbidity/mortality studies attempt to examine the achievements of biomedical research and the health care system that are responsible for prolonging life, improving quality of life and averting health care costs, as well as a host of other averted costs and added benefits that have resulted from the investment in biomedical R&D (9/421,422,512).

In essence, both techniques above examine the usefulness of a field of science over the course of a number of years. For the immediate needs of institute, program, or project managers, more specific decision-assisting techniques are required.

Evaluations of Specific Technologies and Programs/Projects

To assess the performance of specific medical technologies and programs and projects that it administers, NIH rarely uses explicit cost-effectiveness or cost-benefit studies, but it does use a variety of effectiveness studies. For this report, the effectiveness studies will be grouped into two categories: 1) clinical trials and consensus development conferences, which are used to evaluate the safety and efficacy of specific technologies; and 2) more general performance evaluations, which are used to examine a diversity of NIH programs and projects. Whereas clinical trials deal primarily with medical technologies that are in the development/application stage of research, performance evaluations can cover a myriad of types of NIH activity ranging from biomedical research to health services research. These evaluations are used for obtaining information necessary for program and administrative needs.

Clinical Trials and Consensus Development Conferences.—Clinical trials provide the basis for the testing, evaluation, and application of basic and applied research knowledge before it is introduced into the health care system. They also provide the information needed to examine the safety and efficacy of newly emerging technologies. NIH is the major source of support for these trials in the Federal Government (465). Clinical trials are technical in nature and are not usually designed to examine in depth any factors other than the safety and efficacy of a medical technology.

Augmenting the clinical trial process are the recently implemented consensus development conferences that NIH sponsors. These conferences are designed to go a step beyond the limited focus of clinical trials to assess a broader set of issues. A sample of the issues discussed at recent meetings are methods of diagnosing and treating allergies, treatments of ocular melanoma, and estrogen use in postmenopausal women. These meetings may examine the scientific merit, along with the attending issues of risks, benefits, costs, and ethical implications, of implementing a new medical technology. The meetings are primarily a technically oriented approach to discussing whether the innovation is safe, efficacious, and cost effective.[7]

Project Assessments.—The second type of evaluation is the much broader area of project assessment that NIH uses to examine completed and ongoing projects. All 11 institutes, NLM, and the Division of Research Resources support evaluation efforts within their areas of responsibility.

Evaluation activities are divided into three general categories (450): 1) the NIH evaluation classifications, such as program effectiveness, methodology and resource development; 2) funding guidelines, i.e., science base, application, (technology) transfer, and training (SATT); and 3) the Assistant Secretary for Program Evaluation (ASPE) program evaluation categories.

Four of the ASPE evaluation categories will be mentioned (450):

- exploratory *evaluations,* which identify the objectives and expectations of relevant policymakers and program managers, identify the program objectives and performance indicators on which the program will be held accountable, and identify evaluation/management options for changing program activities, objectives, or uses of information in ways that are likely to improve program performance;
- *short-term evaluations,* which summarize available and readily obtainable information on program performance in terms of the objectives and performance indicators identified in the exploratory evaluation and provide designs for full-scale evaluations;
- *full-scale evaluations* of program performance in terms of the agreed-upon measurable objectives and performance indicators identified in the exploratory evaluations; and
- *program performance summaries,* which summarize evidence on how programs are performing in terms of the set of objectives and performance indicators on which the program is being held accountable.

The projects that fall into these four categories, in that they attempt to examine explicit outputs and/or the inputs of a given project, are the closest NIH comes to conducting explicit cost-effectiveness studies. The boundaries established in the definitions above are not much different from the general outlines used in many cases for CEAS. The studies' main divergence from CEAS is in their relatively weaker emphasis on "costs" and stronger focus on the performance, or effectiveness, aspects of the program or project. Cost comparisons remain implicit; the quality of project performance, however, receives considerable attention.

Potential Use of CEA/CBA

The formal technique of CEA/CBA seems ill suited to the NIH biomedical research decisionmaking process (560). Perhaps this methodology could be helpful in certain program or project evaluation situations, or possibly in the context of assisting the awarding of contracts or supporting the research centers, but *generally* the complex, dynamic, and uncertain nature of the research process, the frailty of the methodology, and the backgrounds of the decisionmakers militate against the beneficial use of these techniques in the biomedical research system.

The uncertainties involved in the biomedical R&D process are many, and CEA/CBA has little ability to adequately summarize, include, and compare items involved in bringing research to fruition, The development of a medical technology does not follow a linear or steady

[7]For a more detailed discussion of clinical trials and consensus development panels, see the OTA report *Assessing the Efficacy and Safety of* Medical Technologies (465).

path. Biomedical science may contribute only a portion of the knowledge and research that is needed to develop an idea or technology fully. Even after the technology is in use, the obstacles to defining and measuring the costs, effectiveness, and outcomes are many. Distributional and equity issues present themselves at many points along the R&D process. These methodological problems have yet to be solved. The list could be continued, but the heart of the issue is that formal CEA/CBA is not readily useful or applicable to the process of planning, allocating, or evaluating biomedical research.

The case against CEA and CBA grows even stronger when one examines the mechanisms already in place to assist the biomedical R&D decisionmaking process. Those mechanisms, the peer review system and evaluation processes, seem to have performed adequately over the years to allocate research resources efficiently and intelligently. As the rough edges are removed from those systems, they become even more valuable to the decisionmaking process. At the level of biomedical R&D, a cost-effectiveness attitude probably serves the system better than would formal CEA/CBA.

NATIONAL CENTER FOR HEALTH CARE TECHNOLOGY

In 1978, Congress added a new level of evaluation and coordination to the health care research, development, and application process by establishing NCHCT as part of HEW.[8] NCHCT is responsible to the Assistant Secretary of Health, DHHS. Its mandate is to "undertake and support assessments of health care technologies."

Before the creation of NCHCT, there was no identifiable organization that could act as a coordinator for information concerning emerging technologies. There was no single office that had the responsibility to act as the information manager for the application and dissemination of new medical technologies.[9]

NCHCT has a potentially important role in the decisionmaking process. Its enabling legislation establishes a number of broad-ranging functions for NCHCT. Generally, NCHCT is to set priorities for technology assessment and to encourage, conduct, and support assessments, research, demonstrations, and evaluations concerning health care technology. Specifically, the Center will **(437):**

- undertake and support comprehensive assessments of health care technology, including analyses of safety and efficacy, and ethical issues;
- undertake and support studies of the cost effectiveness and cost/benefit of current and developing technologies;
- undertake and support syntheses of existing research (e.g., state-of-the-art papers);
- provide the best scientific/medical and economic assessments to HCFA on medicare coverage for specific medical procedures and technologies, including evaluation of the costs and benefits of old procedures and assessment of new technologies for which HCFA might require medicare coverage decisions in the future;
- undertake and support dissemination of information derived from its assessment activities to the practicing and scientific communities, Federal agencies with health interests, third-party payers, the public, and others as appropriate;
- undertake and support manpower training programs to provide for an expanded and continuing supply of individuals qualified to perform the research, demonstration, and evaluation activities related to health care technology; and
- undertake and support, to the extent practicable, by September 1, 1981, the planning, establishment, and operation of three extramural centers for assessments, re-

[8]The statute establishing NCHCT is Public Law 95-623, sec. 309 of the Health Services Research, Health Statistics, and Health Care Technology Act, Nov. 9, 1978.

[9]With respect to new medical technologies, the responsibility and involvement of NIH tends not to extend much past the applied research and early transfer stages. The health services research sector usually does not focus on new technologies until they are in place or at least well along the development cycle.

search, demonstrations, and evaluations of issues in health,

By law, NCHCT must have a national council to advise the NCHCT staff. A major function the council, which has been established, will serve is the identification and selection of medical technologies that should receive priority attention.[10] The council is also asked to (142):

- advise the Secretary on the safety, efficacy, effectiveness, cost effectiveness, and social, ethical, and economic implications of particular health care technologies;
- develop, publish, and disseminate standards, norms, and criteria concerning the use of particular technologies, when appropriate and practicable; and
- review applications for grants and contracts exceeding **$35, 000** in direct costs.

NCHCT is less than **2** years old. Much of its activity since its creation has been directed towards organizing and developing procedures to perform the functions it was created to serve. For that reason, it is difficult to examine NCHCT in the same light as NIH, NCHSR, and HCFA—agencies that, together, have several decades of experience behind them.

Research Support Structure

The research support structure of NCHCT is similar to that of NIH, NCHSR, and HCFA. NCHCT will support research via extramural grants, extramural contracts, intramural research, and will support manpower training programs to draw qualified individuals into this area of research. The national council is responsible for reviewing any grant exceeding $35,000 in direct costs, and outside experts can be used to review and provide comment on any applications for research funds or any results of research that are received by NCHCT. The peer review system and the basic organizational procedures for selecting and supporting research at NCHC"T are much the same as elsewhere.

Technology Evaluation Activities

NCHCT is in the process of pursuing a range of research and dissemination activities and is in the planning stages for several additional projects to be initiated this year (1980). At the first national council meeting in October of 1979, Ruth Hanft, Acting Deputy Assistant Secretary for Health Research, Statistics, and Technology, reviewed a number of the activities that are ongoing or in the planning stages at the Center. These activities are (142):

1. *Comprehensive* assessment.—Saf ety, efficacy, cost effectiveness, and economic, social, and ethical impact of a selected technology will comprise a comprehensive assessment. Two such assessments will be initiated in fiscal year 1980.[11]
2. *Coverage* issues.—HCFA asks the Center for advice regarding the appropriateness of paying for the use of certain technologies with medicare funds. Currently, the Center is responding to 53 requests for coverage recommendations.
3. *Consensus development processes of N]H. —The* Center will be more involved in the consensus development conferences sponsored by NIH and will cosponsor some of these. NCHCT provided an economic analysis at the conference on intraocular lenses conducted in September 1979.
4. *Intramural activities, —The* Center is conducting cost-effectiveness studies on a number of technologies (e. g., intraocular lenses, estrogen use by postmenopausal women, and antenatal diagnosis).
5. *Overviews. —The* Center is writing state-of-the-art papers on technologies which are candidates for comprehensive assess-

[10]The NCHCT National Council identified the following technologies as candidates for priority consideration (457):

Ultrasonic diagnostic procedures; coronary by-pass surgery; fetal monitoring; end-stage renal disease—dialysis (home v. hosp.), transplantation, etc.; non-invasive radioactive imaging; barium enema; EEG; nuclear magnetic resonance; auto-analyzers; all skull films; cerebral angiography; dental X-rays; CAT scanner; continuous flow analysis.

[11]In the fall of 1979, NCHCT was handed, or chose, its first technology assessment topic—the alpha fetoprotein test kits. FDA is delaying approval of this new technology that it has found to be safe and effective until the Center completes an assessment of the kit's impact on the health care system and society. NCHCT is coordinating the information, input, and efforts of FDA, the Center for Disease Control, NIH, the Health Resources Administration, HCFA, and others in order to evaluate the full range of issues involved.

ments (e.g., end-stage renal disease, electroencephalograph, and coronary bypass surgery).

6. *Dissemination.* —This activity is just beginning, with the assistance of NIH and FDA among others. In fiscal year 1980, the Center will begin its own dissemination activities.
7. *Early warning system.* —HCFA, the Center for Disease Control, FDA, NIH, and the Alcohol, Drug Abuse, and Mental Health Administration are developing methodologies to identify emerging technologies. Non-Federal organizations, such as the American Hospital Association, will also help identify emerging technologies.
8. *Grant and contract program.* —This program began in fiscal year 1980 in the area of literature syntheses, cost-effectiveness studies, and economic, social, and ethical anal yses.
9. Centers *program.* —Public Law 95-623 requires that three extramural research centers be established by September 1, 1981.

As this list of activities indicates, NCHCT is involved in a wide range of technology evaluation efforts. At one end of the technology evaluation process, the Center is involved in consensus development activities at NIH which are focusing on relatively new medical technologies that have areas of uncertainty to be resolved. In addition, a very important part of the Center's efforts is focused on the reimbursement system (see ch. **5).** NCHCT is specifically charged with coordinating information and making recommendations to HCFA regarding new or existing medical technologies. All indications are that this will be a priority activity of the Center. Finally, the Center will provide comprehensive examinations of medical technologies that have been in use for some time. In many of its functions, NCHCT has the authority to conduct or fund CEAS.

Potential Use of CEA/CBA

NCHCT is in a position to act as an information broker to a number of agencies at a variety of decision points in the policymaking process. Its mandate is so broad, however, that it may become overwhelmed by the number and diversity of functions it is asked to perform. Funding levels, and consequently staffing levels, are significantly lower than those called for in NCHCT'S authorizing legislation.[12] This factor may significantly affect the number and range of duties the Center can be expected to perform. So far, HCFA has requested NCHCT to examine **53 coverage** issues. With this area of responsibility and the other functions listed above, NCHCT will likely find it difficult, at current funding and staff levels, to totally fulfill the expectations placed on it.

NCHCT has developed a priority-setting process that may help it handle the influx of requests for information, recommendations, assessments, and general assistance. To a degree, priority-setting is vested in the national council, but the council focuses primarily on the selection of medical technologies that warrant full-scale assessments. The remainder of the priority-setting process is a mixture of identifying in-house needs, perceptions of what areas require more immediate action, the assimilation of priority areas and views of the agencies NCHCT must work with and respond to, and the general consensus of the scientific and medical community regarding issues that require attention. In addition, NCHCT priorities will be set to a degree by the deadlines of the agencies with which NCHCT works.

The result of these many factors and considerations is an unclear picture of how formal CEA / CBA fits into NCHCT'S activities. The agency is new; it remains to be seen to what extent NCHCT will conduct or support formal CEA / CBA. If, where, and when these techniques are employed, it will be interesting to note their level of sophistication, their use in decisionmaking efforts, and their level of input into and impact upon the policy process.

[12] The enabling legislation (Public Law 95-623, sec. 309) authorized $15 million, $25 million, and $35 million for fiscal years 1979 through 1981, respectively, for NCHCT to carry out its mandate. The fiscal year 1979 budget for the Center was $344,000, and the fiscal year 1980 budget is $3.25 million.

conclusions regarding the performance of services research. One side of the debate cites numerous examples of major health policy decisions where services research was of little consequence (31,359,383,423). The other side cites evidence to support the notion that health services research has been successful in producing information useful to the policy process, in some instances has been instrumental in stimulating policy debate and change, and in general, compares favorably to other forms of social services research (139,186,428,566).[15]

It is doubtful whether the debate surrounding the performance and usefulness of health services research will be settled conclusively one way or the other. The range and types of issues that services research must address, the dynamic nature of the policy issues and health care system, and the complexity of the decisionmaking process combine to almost preclude a conclusion regarding the relative worth of the information produced by health services research efforts. Nevertheless, there does seem to be a general feeling that health services research has not attained the high level of expectations set for it a decade ago and that improvements can be made. The ongoing examination and evaluation of services research may help improve its focus, usefulness, and quality.

The primary focus of this section is on the current or potential use of CEA/CBA in the health services research decisionmaking process, i.e., the procedures used to establish health services research priorities, fund research projects, and evaluate work that is ongoing or that has been completed. The section also discusses the extent to which certain health services research agencies support CEA/CBAs as part of their research missions.

NCHSR and HCFA represent the Federal Government's most substantial commitment to the area of health services research. NCHSR and

[15]It is beyond the intent of this assessment to examine this debate. For detailed discussions of the history of services research, its contributions, its weaknesses, and its development, see the following references listed at the end of this report: 24, 36, 210, 428, and 643.

HCFA provide the highest level of funding of health services research in the United States. The funding levels for health services research have declined in actual and real terms since the peak years of the early 1970's. NCHSR'S 1978 budget represented less than 40 percent of its purchasing power for research and training programs compared to the levels of the early 1970's (428). Together, NCHSR and HCFA contribute roughly 40 percent of the total amount of Federal funds allocated to health services research (428). In fiscal year 1980, they will control approximately $50 million in moneys **ear**marked for services research. These agencies occupy a very influential position in the health services research community and are in a position to exercise considerable influence on the content, direction, and level of health services research in this country.

It is unclear where applied biomedical research ends and where health services research begins. Several agencies that focus primarily on conducting and supporting basic and appied research routinely conduct or fund health services research as part of their programmatic missions (428).

Health services research is unlike most other areas of scientific inquiry in that it is not organized around a single discipline with unique perspectives, closely drawn areas of expertise, common methodologies or techniques, and standard nomenclatures. Health services research is a mixture of concepts, methodologies, attitudes, and professions that could easily span a large university's graduate school catalog. The field of health services research must accommodate data, methodological frameworks, disciplines, and perspectives from the diverse fields of medicine, other health-care-related disciplines (epidemiology, nursing, public health, etc.), biostatisticians, engineers, lawyers, demographers, geographers, operation researchers, economists, social workers, hospital and business administrators, and so forth. Individual health services researchers tend to approach the issues from the confines and perspective of their particular discipline.

David Mechanic describes the purpose of health services research as follows **(396):**

> The health services research field focuses on the production, organization, distribution, and impact of services on health status, illness, and disability . . . it concentrates attention on improving the distribution, quality, effectiveness, and efficiency of medical care.

A study by the Institute of Medicine on health services research attempted to provide guidelines for the description or classification of the types of health services research that exist. According to that report, a study is classified as health services research if it satisfies two criteria (428):

1. It deals with some features of the structure, processes, or effects of personal health services.
2. At least one of the features is related to a conceptual framework other than that of contemporary applied biomedical science.

National Center for Health Services Research

NCHSR was created in **1968** without explicit congressional authorization. It was not until 1974 that NCHSR received legislative authority via the Health Services Research, Health Statistics, and Medical Libraries Act (Public Law 93-353). Since then, several laws have added to or modified NCHSR'S research domain. [1b]

NCHSR has two principal responsibilities. One is to develop information that might be used by various decisionmakers in the public and private sectors. The other is to ensure that the information that results from the research, evaluation, and demonstration activities of NCHSR is disseminated rapidly and in a form that is usable.

NCHSR is a major supporter of broadly focused health services research. Its fiscal year 1980 operating budget will be almost **$30** million. NCHSR is almost unique in the Federal Government in that it can sponsor health services research apart from direct administrative or

[1b] A few of those laws are the National Health Planning and Resources Development Act of 1974 (Public Law 93-641), the Emergency Medical Services Systems Act of 1973 (Public Law 93-154, sec, 1205), and the Health Maintenance Organizations Act of 1973. These and others have influenced the direction of research priorities and level of funding from NCHSR.

programmatic needs. It is not responsible for the administration of any health care delivery or reimbursement activities; it exists solely to conduct and sponsor health services research and to disseminate the results of that research to relevant Government agencies, the research community, and other interested parties.

The NCHSR statute's language is so broad that the Agency retains considerable leeway in its selection of specific research issues to pursue. To identify the areas of research it needs to conduct and support, NCHSR employs an informal consensus development process.

Priority Setting

The priority-setting process involves several steps. First, NCHSR canvasses policy makers, consumers, Government and non-Government experts, health care providers, professional associations, program administrators, and others, in an attempt to identify current and emerging issues that present the most immediate problems to the health care system. In the first cut at the list of suggested issues, a number of criteria are used, two of which are the relative importance of the issue and the perception that there is a good chance that the research will provide information that will contribute substantively to the policy process.

NCHSR staff, -as well as outside professionals involved in various research areas, are involved in the culling process. The Director of NCHSR selects the top priority concerns from the major issues identified by this process. Before the list is adopted, it is submitted to an assorted group of NCHSR members and non-NCHSR experts for review. The priority issues that emerge from this process become the areas of health services research that NCHSR focuses on. The issue selection process is not a one-time occurrence, but rather an ongoing interaction between NCHSR and the health care community.

The list of priority areas does not lock out all other health care issues. NCHSR can, and does, *consider* "meritorious and potentially important" proposals for a range of nonpriority issues. In-house, or intramural, research can also pursue areas of interest that fall outside the priority areas.

Currently, NCHSR supports five general priority areas and a special studies category that is concerned with examining issues of emerging importance or of research methodology. The current priority areas are **(438):** 1) health care costs and cost containment, 2) health insurance, 3) planning and regulation, 4) technology and computer science applications, and 5) health manpower

Evaluation of Research Proposals

NCHSR'S health services research efforts are allocated among intramural research, extramural research grants and contracts, and center grants. Intramural research is subject to an informal review process within NCHSR. When the need arises, staff proposals for in-house research projects may also be reviewed by non-Federal experts. Projects are formulated according to special research needs or personnel capabilities at NCHSR, The in-house project proposal moves through the administrative hierarchy, receives comments and suggestions from the staff, and may be circulated to outside reviewers before final action is taken by NCHSR. Health services research centers receive funding apart from the extramural and intramural research budget. NCHSR'S authorizing legislation called for the funding of at least six "existing and new centers for multidisciplinary health services research, evaluation, and demonstrations." There are eight such centers currently in operation (650).

NCHSR uses a dual review type of evaluation process to screen and select grant and contract applications it receives (385). This process is quite similiar to the peer review system described earlier for NIH. Grant and contract proposals that exceed $35,000 in direct costs are required to be reviewed for scientific and technical merit by study sections composed of non-Federal Government experts. Proposals that do not exceed $35,000 **are** evaluated by NCHSR staff, and where needed outside reviewers.

Explicit CEA/CBAs are not used in the priority-setting, project selection, or research evaluation processes. A few of the project criteria used in the selection process, however, make it likely that there will be significant emphasis on the

relationship between the application's budget (cost) and its potential outcomes (benefits). NCHSR receives far more applications than it has the funds to support, so in the project selection process, it does consider cost and effectiveness. The budget and outcomes criteria used to evaluate applications are considered with reference to the Agency's budget and goals.

CEA/CBA as Part of NCHSR'S Research Mission

NCHSR supports a number of cost-effectiveness and cost-benefit studies that cover a broad range of health services issues (439) and is the major supporter of CEAS and CBAS in the Federal health care research system at this time. Two of NCHSR's five research priority areas—health care costs and cost containment, and planning and regulation—specifically call for research using CEAs to examine the issues within these priority areas (438).

The CEAs and CBAs that NCHSR supports range from rigorous analyses of specific health care topics to broad studies of more complex health care issues. The results of these studies, like most of NCHSR's research products, are circulated via the Agency's formal publications dissemination process, conferences, seminars, journals, announcements, etc. NCHSR is in a position to monitor the eventual use of the research results by other agencies and decisionmakers. In most cases, though, the nature of the policymaking process makes it quite difficult to determine to what extent a given piece of information is used to reach a final policy decision. NCHSR is powerless to assure that the research results will actually be used in the decisionmaking process. The Agency's legislative mandate is to support health services research projects that answer, or at least address, the issues that the research community and policymakers feel are important. Decisions about whether and, if so, how to use the information that is generated are left up to agencies and decisionmakers outside NCHSR.

Health Care Financing Administration

HCFA, established in 1977 as a result of a major reorganization at the Department of Health, Education, and Welfare (now DHHS), is a recent addition to the Federal Government's health services research community. This Agency is the organizational center that administers medicare/medicaid programs, the Professional Standards Review Organization (PSRO) program, and the research and statistics programs for these areas.

The legislation authorizing these programs allows HCFA to consider and pursue a range of health services research topics. '7 HCFA is particularly responsible for sponsoring research that relates to its primary mission: the administration and evaluation of the medicare/medicaid area and the PSRO function. The mandate of the Office of Policy Planning and Research at HCFA, however, is broad enough to include a wider range of research topics. 18

HCFA currently has five major priority areas of grant support. Specifically, Agency support is focused on projects that meet one of the following criteria (284):

1. develops or demonstrates new financing mechanisms for health care services;
2. utilizes financing mechanisms to influence the effectiveness or delivery of health care services;
3. develops or demonstrates management or administrative procedures that will benefit HCFA programs;
4. develops knowledge or undertakes analyses of the basic nature and structure of health care costs and factors that affect their rate of increase; or
5. examines the economic and behavioral relationships between the financing of health care services and the total activities of the health care sector of the economy.

[17]The legislation that establishes HCFA's scope of responsibility is the Social Security Amendments of 1972 (Public Law 92-603). Sections 222 and 245 of this law provide for health services research and demonstration projects for a variety of reimbursement issues, performance incentives, etc. The National Health Planning and Resources Development Act of 1974 (Public Law 93-641) added provisions for work in ratesetting and development of quality data for providers.

[18]The Office of Policy Planning and Research of HCFA grew out of the Office of Research and Statistics (ORS), formerly of the Social Security Administration. ORS became a major source of support for health services research in the late 1930's.

Priority Setting

HCFA'S priority research areas are chosen in an informal process much like the one used by NCHSR. Opinions, suggestions, and recommendations are solicited from a variety of people with a broad background of experience and training. Their recommendations are culled to form a list of research priorities. Although priority areas receive special attention in the selection process, new ideas and innovations are not automatically excluded from consideration if they fall outside these areas, Relevant examples of the issues on which HCFA is focusing are hospital costs, physician reimbursement, and the quality and effectiveness of various health care areas.

Evaluation of Research Proposals

The peer review mechanism and the project evaluation process at HCFA are very similiar to the systems at NCHSR. The major difference between the two approaches is the formal involvement of HCFA staff in the initial technical review panels. Instead of being composed of all non-Federal experts like NCHSR's and NIH's panels, HCFA's review panels are composed of an equal mixture of HCFA staff, non-Federal experts, and non-HCFA Government experts (284). The criteria used to evaluate the merit of the research application are geared as much to HCFAsS program needs as to technical and scientific merit. Final review and funding decisions are made by HCFA staff and administrators.

Use of CEA/CBA

HCFA is like the other agencies discussed above, in that it does not use explicit CEA/CBA to select research goals or grant proposals, or to evaluate project outcomes. Awareness of projects' "cost effectiveness" to the Agency's goals, however, is present.

HCFA focuses much of its substantive research on priority issues that try to determine the relative efficiency of various methods of delivering care. Many of its research solicitation areas are for work to arrive at CEA/CBA-like evaluations of specific issues. Or, data are pursued that might assist HCFA in determining cost and effectiveness measures. Several of the projects recently completed, as well as a number of ongoing efforts, are directly focused on cost-effectiveness issues (283).

The CEA/CBAs that HCFA is supporting are similar to those at NCHSR, in that they range from fairly rigorous attempts at examining the costs and effectiveness of a medical technology to being closer to effectiveness studies that include costs as an analytical afterthought, if at all. The information produced as a result of these analyses is combined with the other information and considerations that are part of HCFA's program responsibilities. At HCFA, as at the agencies previously described, the support, evaluation, and use of analyses or information in general is not usually a linear process. It is extremely difficult to point to a given piece of information, a cost-effectiveness study for instance, and determine where, how, or even if, the knowledge gained from a given research project was directly incorporated into a given policy decision. Nevertheless, since HCFA can tailor its use of CEA/CBA to suit its special program needs and might then be able to implement that information within its organizational boundaries, it could possibly serve as an instructive example of the support and use of CEA/CBA in the decisionmaking process. HCFA is in a position to evaluate the impact of its actions after programs or program changes have been in place for a period of time. NCHSR and NCHCT do not share this type of start-to-finish authority. NCHSR and NCHCT have more limited access and input to the decisionmaking process than do HCFA, NIH, and other health care agencies that maintain program responsibilities.

Although HCFA is a collection of fairly mature programs that have been reorganized into a new agency, the organization has not fully settled into its new niche. It will be interesting to note how, or if, HCFA uses any CEA/CBAs that are produced by HCFA's own research support system, by NCHSR, or via the new efforts of NCHCT.

USE OF CEA/CBA IN R&D PROGRAMS: GENERAL FINDINGS

Formal CEA/CBAs are not used or supported to a great extent in the health care R&D system. At the process (or administrative) level, Federal agencies rarely use explicit CEA/CBA as a decision-assisting tool. In only a few cases has CEA/CBA been instrumental in facilitating a given allocational or policy decision. (See "Survey of Agency Use of CEA/CBA," app. B.) The agencies do seem to make efforts to employ a "cost-effective" approach and attitude towards the decisions that are made regarding the setting of priorities, selection of research projects, and in the evaluation of research products. These attitudes and approaches are transferred into action primarily via external and internal peer review mechanisms. At the substantive level, several formal CEAs and CBAs have been or will be supported by NIH, NCHCT, NCHSR, and HCFA. The research done at this level may feed back to a degree into the administrative decisionmaking and evaluation processes at these agencies. **A** significant amount of the CEA/CBA research will be used to add to the body of knowledge in the health care system.

Efficiency-based techniques such as CEA/CBA have not gained a substantial foothold in the R&D decisionmaking process. The same can also be said with respect to the other formal methodologies that have received some use and recognition in the field of policy analysis (i.e., decision analysis, operations research, technology assessment, risk-benefit analysis, etc). The relatively infrequent reliance on these types of analyses stems from a number of factors related to the techniques and the nature of the health care system in general and the R&D process in specific. The complexity and uncertainty of the R&D endeavor and its eventual products does not lend itself well to the constraints of efficiency-based methodologies. This may be the most important reason for the lack of use of CEA/CBA in R&D. The health care R&D process is extremely complex, essentially political, and quite often is passive. The Federal R&D process is authorized, funded, and supervised by representatives of the public and is under the scrutiny of the Nation as a whole. The R&D system must be responsible and responsive to the changing needs and goals of the country; hence, it is a highly political process. Finally, the system tends to be passive. Many problems are often presented to the decisionmaker with the objectives preordained and the viable options available to attain the goals few in number. The R&D process is fueled by the imagination and initiative of those researchers outside the decisionmaking process. A research goal or national health policy objective can be established, but the system does not move without the initiative of those who must create and submit research ideas and plans to the funding agencies. To a significant degree, R&D-supporting agencies must wait for, and react to, the ideas, suggestions, efforts, and research findings of the thousands of health care researchers both within and outside Federal Government. It is extremely difficult for CEA/CBA to predict, evaluate, or include the importance of the dynamic aspects of R&D. As a result, CEA/CBA's credibility, usefulness, and input to the R&D decisionmaking process are limited.

CEA/CBA is most supported and used at the health services research end of the R&D spectrum and least supported and used at the basic and applied end. This situation follows logically from the inability of formal analytical techniques to adequately deal with the high level of uncertainty that is part of the technology R&D process. Predictions, opinions, and "guesstimates" are the tools of the trade in this area. One can include considerations of uncertain factors in CEA/CBA; sensitivity analysis can help to an extent. But the level of uncertainty remains high.

The methodological shortcomings of CEA/CBA techniques are compounded by the attitudes of many of the potential users of CEA and CBA in the R&D system. The decisionmakers' perceptions regarding the usefulness and validity of CEA/CBA are such that many doubt that these techniques are either necessary or helpful in much of the R&D context. This view is particularly strong at the basic and applied research level. This situation should not be surprising. Until recently, cost containment and cost-effec-

tiveness criteria were not heavily stressed in health care research. NIH has traditionally focused on the quality of the research supported and the safety and efficacy of the technologies developed as results of research efforts. Finding cures for health care problems, not saving money, has been the primary goal. At the health services end of the R&D spectrum, distribution, cost, and quality of care have received needed attention. Cost and effectiveness criteria have been part of the R&D efforts; but only parts of a much larger focus. Recently, decisionmakers have been asked to make them a larger part of their decisionmaking criteria. As a result, increasing numbers of decisionmakers are becoming aware of the uses (and possible abuses) of CEA and CBA techniques.

Another impediment to the use and support of CEA/CBA is the presence of other decision-assisting techniques that have been fairly successful in guiding and informing the decisionmaking process. Peer review panels, publication of results in reputable journals, advisory councils, conferences, and other mechanisms have all worked fairly well as "quality" controls and, to an extent, as cost-effectiveness filters. These processes are firmly in place, have performed reasonably well over the years, and have by and large produced commendable results. The pressure to maintain these existing support systems might tend to overwhelm any serious effort to incorporate CEA/CBA in the mainstream of the decisionmaking process.

The organizational and statutory frameworks are currently in place to allow the use of CEA or CBA in the decisionmaking efforts of the R&D-supporting agencies. The limiting factors are the perceived need for and usefulness of the information that might result from CEA/CBA research.

If CEAS could be adapted to the need of biomedical R&D, the use of CEA-type studies at NIH might logically occur at the late transfer stages of a technology's movement from the lab to the clinic and at the consensus development meetings held by NIH and NCHCT. NIH may also be able to incorporate some form of efficiency-based analysis in its center, contract, and intramural research efforts. NIH has more control over the formulation, direction, and evaluation of these types of research efforts than it does over extramural project grant research efforts. It is at these points in the decisionmaking and information-gathering processes that the CEA/CBA technique might help serve the needs of the NIH decisionmakers. NIH has in a very few instances used CEA techniques to evaluate the ongoing or completed programs and projects that are performed as adjuncts to the basic and applied research missions (e. g., health education programs, disease prevention advertising programs, information dissemination projects, screening programs, etc.). It might consider the usefulness of increasing its efforts along these lines. Once again: Doing CEA/CBA for any reason requires that the limits and usefulness of the analyses be kept in mind.

NCHCT could provide input into a range of decision points in the R&D process. Its legislative mandate authorizes the use of CEA/CBA to examine newly emerging and existing technologies. NCHCT cosponsors consensus development conferences at NIH and is charged with providing information to HCFA on reimbursement issues concerning medical technologies. It remains to be seen how NCHCT will use CEA/CBA in the decisionmaking process.

NCHSR and HCFA hold promise as supporters of CEA/CBA research. HCFA might also be a user of CEA/CBA information in its PSRO and medicare programs. These two agencies focus on technologies in later R&D stages and on the services end of the health care system. CEA/CBA has recently received increased attention in this area of research and one sees increasing use of this technique to examine a host of services research issues. 19

"Such use is discussed at greater length in *Background Paper #1: Methodological Issues atrd Literature* Review, prepared by OTA in conjunction with this assessment.

10m Health Maintenance Organizations

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Health Maintenance Organization;

INTRODUCTION AND BACKGROUND

Health maintenance organizations (HMOS) have been described by their advocates as a "cost-effective' way to provide health care (489). It appears that HMOS do provide care to their enrollees at varied but substantial cost savings: Empirical evidence shows that HMO enrollees pay in the range of 10 to 40 percent less in total costs (premium plus out-of-pocket costs) than conventionally insured comparison groups **(373).**

Explanations of HMO performance, whether measured by costs, access, quality, or physician or enrollee satisfaction, however, are a hybrid of rhetoric, theory, and evidence. There is very little information available on the details of how HMOS actually function, especially on the analytical tools they use to make internal decisions on how to allocate their resources **(295).** This chapter presents some preliminary evidence regarding the current and potential use and usefulness of cost-effective analysis/cost-benefit analysis (CEA/CBA) in resource allocation decisionmaking in HMOs. It does not, however, examine directly the question of the cost effectiveness of HMOs themselves.

HMOs, representing the prepaid segment of the health care market, are both insurers and providers of health care (300). Their revenue comes from cavitation payments, and they are responsible for delivering care to an enrolled population (371). Theoretically, the HMO has a direct economic incentive to provide "cost-effective" care. Because of this, some people have assumed that the HMO might be more receptive than conventional health care delivery settings to the use of CEA/CBA (615).

It is important to note, however, that HMOs exist in a predominantly fee-for-service environment. Since potential enrollees have a choice of providers, and physicians a choice of practice settings, the HMO must compete for both. Because its financial viability depends on its ability to attract and retain enrollees (as well as physicians), the HMO will be induced to offer benefits and services comparable to those offered by its fee-for-service competitors.

While the distinctive characteristics of the HMO guarantee control of total costs (by virtue of cavitation payments) and promote efficiency (by virtue of the HMO's responsibility to deliver appropriate services to an enrolled population within a constrained budget), there is nothing to require the HMO to be any more or less concerned with the effectiveness and benefits of a service than are providers in other health care delivery settings: Rather, the benefits side of the CEA/CBA problem is largely accepted as being defined by the norms of "good medical practice" that prevail in the marketplace. The expectations of HMO enrollees and physicians tend to diminish the importance of resource allocation questions pertaining to whether or not a benefit or service should be offered.

In general, the HMO plan bears primary responsibility for the financial viability of the HMO (in a management or administrative sense). The plan faces explicit resource allocation decisions upon which economic incentives and financial constraints directly bear. Whether or not physicians are sensitive or responsive to these incentives and constraints in making their implicit resource allocation decisions, however, is the subject of considerable debate (152,371, 589,590). Some claim that physicians are unaware of incentives and constraints, and even point to complete coverage as giving them a free hand **(372).** Yet others assert that physicians in the HMO setting are sensitive to costs (at least on a subliminal level) and that even *a* decision, for example, to treat bronchitis, or to perform a coronary bypass, specifically involves the aspect of costs (619).

'This range reflects the differences observed between types of HMOs. Prepaid group practices consistently show the lowest total (premium plus out-of-pocket costs), though there is much variation among them; individual practice associations show among the highest costs (372).

In fact, the available evidence shows that almost all of the observed cost savings in HMOs are attributable to lower rates (by 30 percent) of hospitalization for HMO enrollees (373). Decisions concerning the need for hospitalization of HMO enrollees are primarily made at the discretion of attending physicians. It is true that the resource allocation decisions made at the administrative level will define the parameters of decisions faced by HMO physicians. Apart from direct and indirect controls imposed by the plan of the HMO, however, there is little consensus on any one explanation of why HMO physicians should exhibit this apparent "cost consciousness." HMO physicians, trained no differently than physicians practicing in the fee-for-service environment, bring with them habits and values acquired in medical school or in some other prior practice setting **(300).** Theoretically, there is no reason to expect that HMO physicians would be any more likely than their fee-for-service counterparts to explicitly consider the aspect of cost, or beyond that, to consider cost concomitantly with the effectiveness of a service.

If anything, the HMO physician can be expected to allocate the available resources to a given medical problem foremostly on the basis of effectiveness and/or efficacy, differing in this respect from the plan, which will be motivated to allocate resources in significant part on the basis of costs, i.e., efficiency. No further attempt will be made in this chapter to investigate the use and/or usefulness of CEA/CBA in the decisionmaking of HMO physicians,

The preliminary evidence and conclusions that are presented in the discussion that follows pertain largely to the resource allocation decisions made at the organizational, i.e., administrative, level of the HMO plan. The evidence discussed in this chapter should be regarded as tentative because of the great diversity in types of HMOs and the small number of HMOs from which the evidence was gleaned. The influences assumed to determine the resource allocation questions relevant to the HMO theoretically derive from "generic" characteristics of HMOs that distinguish them from other health care providers. It is important to note, however, that there is probably no such thing as a "typical" HMO. There is a tendency (in the rhetoric and limited literature available on HMOs) to juxtapose HMOs against "other providers," but this has masked important differences among HMOs (295).

The findings presented here do seem to substantiate the preliminary conclusion that formal CEA/CBA, except in its "net cost analysis" forms, is not used to any significant degree in decisionmaking in HMOs. In large part, the evidence is based on: 1) actual resource allocation decisions recently made in a handful of HMOs around the country, 2) the analytic techniques those HMOs used, and 3) the role that analysis played in those particular decisions.

LEGISLATIVE HISTORY AND FEDERAL SUPPORT

HMOs have occupied a prominent position in Federal health policies during the last decade. They have been promoted as one strategy for controlling health care costs and encouraging a more rational allocation of resources to health care needs **(295).**[2] Much of HMOs' original and sustaining appeal derives from the fact that these organizations have been viewed as a more desirable alternative than Federal regulation for achieving those objectives (95).

The term "health maintenance organization" was the brainchild of the Federal HMO initia-

[2] In 1979, the Office of Health Maintenance Organizations (OHMO) contracted for a CBA to examine the economic costs and benefits of continued Federal assistance to new HMO development. That study determined the estimated rate of return (in cost-savings to the community) derived from the Federal HMO development investments. It found that Federal assistance costs are recovered in the form of community health care cost savings after 8 years of HMO operation, and projected even more substantial future savings (302). Based on these findings, OHMO has devised a 10-year strategy for focusing Federal funds and support of HMO development in those areas that offer the greatest potential return (489).

tive in the early 1970's, [q]but the concept of prepaid group practice was born under private sponsorship and developed without Federal assistance before 1970 (372, 489). In 1973, when the original Health Maintenance Organizations Act was legislated (Public Law **93-222),** there were 39 prepaid group practices in existence, some of which had been providing care since the late 1920's (489). A few large programs, such as Kaiser Foundation Medical Care Plan (then, as now, the Nation's largest HMO), accounted for the bulk of enrollment,

Based on the performance of these "prototype" HMOs already in operation, advocacy of the HMO approach to health care became a major Federal initiative in 1971 (589). Federal efforts culminated in the enabling legislation for what was to be a 5-year demonstration program. This legislation (Public Law 93-222) provided a congressional mandate and Federal resources to demonstrate the ". . . extent to which the HMO concept can be transplanted to new environments with new participants putting it into operation. " The authority has been extended twice, once in 1976 (Public Law 94-460) and again in 1978 (Public Law 95-559) (489).

Federal legislation provides explicit directives on how an HMO should be organized and operated. According to Federal law, HMOs are required to provide both ambulatory and hospital services. The law recognizes three variations in organization that distinguish three major types of HMOs (discussed below). A stringent and precise definition of the federally qualified HMO mandates a very comprehensive benefits package (including such benefits as mental health, dental, alcohol treatment, and family planning services), sets limits on charges related to utilization (in addition to premiums), and specifies enrollment mechanisms and the extent of risk sharing (369,640). Of the 212 HMOs in operation as of April 1979, only 99 (47 percent) qualified under this strict Federal definition (489).

Critics of the legislation have viewed it as unfairly restrictive, citing a lack of comparable restrictions in the health insurance industry (372). Still, increasing levels of funding have been authorized during the past decade for the continued Federal support of new and developing HMOs. Approximately 97 (45 percent) of the 212 HMOs operational in April 1979 had received some sort of Federal financial assistance. Of the 99 federally qualified HMOs, 74 (75 percent) have received Federal assistance. The Federal HMO program is responsible for a great deal of growth in prepaid plans over the past decade (489). In June 1979, there were 215 HMOs in the United States with a total enrollment of 8,226,000 persons. The consistent promotion of the HMO program by Congress reflects the opinion that Federal support of HMOs can yield substantial national benefits.

In September 1979, Congress passed amendments (Public Law 96-620) to the Health Planning Act (Public Law 93-641) that effectively exempt HMOs from the certificate-of-need (CON) regulations administered by local health systems agencies (HSAS). The exemption, based on evidence that CON regulations were being used to delay or prevent HMO development (372), reflects congressional support of the broader rationale that HMOs may generate competition among health care providers. As boldly stated by the Office of Health Maintenance Organizations (OHMO) in the Department of Health and Human Services (DHHS) (489):

> . . . HMOs have proven to be a quality, cost-effective, competitive alternative to traditional fee-for-service forms of medical practice . . . Both Federal support and substantial private initiative have coalesced over the past several years to generate a new momentum for future HMO growth . . . It appears that HMOs are moving into the mainstream of the health delivery system of this country,

[q]Paul Ellwood is credited with coining the term "health maintenance organizations" in 1973 to encompass the prepaid group practices, foundations for medical care (otherwise know as individual practice associations), and other types of prepaid systems of health care delivery (174).

DEFINITIONS

As indicated in the introduction, there is probably no "typical" HMO. That notwithstanding, **however, all** HMOs do share some characteristics that distinguish them from other health care delivery systems.

A "generic" definition identifies a minimum set of common denominators that are distinctive characteristics of HMOs **(372).**[a] One such definition identifies the following as HMOs' common characteristic(s): "the provision of comprehensive services by a defined set of physicians to a voluntarily enrolled population paying a prospective per capita fee" (95). Luft has recently developed and elaborated on a five-point definition of the "generic" HMO (372). The five criteria he identifies are:

1. The HMO receives a fixed annual (or monthly) premium from enrollees that is independent of their actual use of services.
2. The HMO assumes at least part of the financial risk (or gain).
3. The HMO serves a defined population.
4. The HMO assumes a contractual responsibility to provide services to its enrollees.
5. HMO enrollees are voluntarily enrolled.

Since each of these criteria is subject to being met to a greater or lesser degree, Luft's definition allows for substantial variation among HMOs that meet these requirements of the "generic" HMO. The extent to which these criteria are met can be used to distinguish between HMOs.

Since the "generic" definition encompasses the two or three major types of HMOs generally recognized[5]— the prepaid group practice (PGP), including the group and staff models, and the individual practice association (IPA) model— additional criteria are required to distinguish between these types.[b]

Two additional criteria that allow the distinction between PGP and IPA types to be made are: 1) how the physicians are paid by the HMO; and **2)** whether they provide services to enrollees in the context of an organized group (closed panel), or from a number of independent offices (open panel). In the PGP model, physicians are paid on either a salary or cavitation basis. In contrast, IPA physicians are paid by the HMO on a fee-for-service basis (according to a fee schedule) for service received by HMO enrollees. Enrollees in the IPA model HMO have their choice of physicians from those belonging to the IPA. In practice, the PGP type of HMO clearly predominates: In June 1979, about 63 percent of all HMOs were PGPs, but PGPs served about 84 percent (or 6,942,000) of the over 8 million total HMO enrollees (300). The distinction between group and staff model PGPs is based on differences in the nature of the relationship between the physicians group and the plan. In the group model PGP, the physicians are a distinct legal entity that contracts with the plan of the HMO, whereas in the staff model PGP, the physicians are hired "on the staff" of the HMO.

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[a] This discussion draws heavily from a draft of the forthcoming book by Harold Luft entitled *Dimensions of HMO Performance (372). OTA wishes to* especially acknowledge Dr. Luft for this helpful conceptual definition of HMOs, of which there are (in reality) virtually limitless varieties.

[5] A fourth kind of HMO, the network model, is also recognized by some, but is not legally recognized (in the HMO statute) as are the prepaid group and staff models, and the IPA models (640). The Health Insurance Plan of Greater New York is the oldest, largest, and best known of the network models. Though network models are currently fewest in number, the model is gaining popularity, primarily because of its attractiveness to physicians and consumers alike, while maintaining a competitive posture in the community (413).

[b] These characteristics are: 1) the method of paying the key decisionmakers, 2) whether physicians are full or part time, 3) the extent of risk sharing, 4) whether the HMO *is* non- or for-profit, *5)* whether the HMO controls its hospital, *6)* whether there are economies of scale, and *7)* the competitive market environment faced by the HMO (372).

INCENTIVES, CONSTRAINTS, AND IMPERATIVES

There is considerable speculation on the relative influence on resource allocation questions relevant to the HMO of the internal incentives and budget constraints and of the external market environment. These two influences derive from the five distinctive characteristics of the "generic" and major types of HMO. The internal economic incentives and constraints arise from the combined features of *prepayment, financial risk,* and *defined population.* The external competitive imperative stems from the feature of *uoluntary enrollment,* The *contractual responsibility* of the HMO to provide services has a dual effect, acting in concert with both sets of characteristics to reinforce their respective influences on resource allocation questions relevant to the HMO.

Economic Incentives and Financial Constraints

The key feature of prepayment implies two things for the HMO: 1) that it has a fixed or constrained budget (total revenue being determined by the number of enrollees and the fixed premium rate), and 2) that the premium paid by HMO enrollees is independent of their actual use of services. Thus, the usual financial incentives that exist in fee-for-service practice, in which the net income of the provider is directly related to the services rendered, are reversed. Within the constraint of a fixed payment per member per month, an HMO's net income, to some extent, is related to the number of services provided. Consequently, there is a direct financial incentive to provide services more efficiently, or to provide fewer services (presumably reducing unnecessary or inappropriate services), since this will translate into lower premiums to enrollees or higher profits to the HMO, or both (615).

The economic incentives and financial constraints deriving from prepayment are enforced by the second criterion which says that the HMO assumes at least part of the financial risk (or gain) in the provision of services. As far as the "generic" HMO is concerned, this feature only implies that some portion of the burden of risk must be borne by the plan and not shifted to a third party through reinsurance.

Within the HMO, however, there are risk-sharing arrangements between the plan and physicians.[7] The extent of "risk sharing" between these two decisionmaking entities has been tendered as the theoretical explanation of observed differences between the performance of HMOs and that of other forms of health care delivery. But the extent of risk borne by physicians varies considerably among HMOs, and generally but not always, by type of HMO. In the IPA, for example, the plan may act essentially as a third-party insurer: Reimbursed by the plan on a fee-for-service basis (according to a fee schedule) for services rendered to enrollees, the IPA physician (and patient) is often somewhat more insulated than the PGP physician from the financial burden associated with decisions regarding the services actually received.

With respect to hospitalization rates, both IPA and PGP enrollees had considerably lower rates **(25** and **35** percent respectively) than conventionally insured comparison groups (373). These observed differences are due to very different kinds of decisions being made in these HMOs. In the case of the IPA, where physicians are often at lower risk for the implicit resource allocation decisions that they make, the plan translates its own economic incentives and financial constraints to physicians by imposing implicit controls on their decisionmaking. Decisions made at an administrative level to implement such mechanisms as preadmission certification and length-of-stay review translate this

[7]There are three functional parts to an HMO, which may even be legally distinct entities: 1) the plan, 2) physicians (already discussed), and 3) the hospital that provides inpatient services *(372).* Usually, the hospital does not enter into risk-sharing agreements with the HMO.

influence and assure that it will be reflected in decisions made at the provider level.[8]

Several alternative explanations for the lower hospitalization rates of PGP enrollees, and for PGP physician behavior in general, have been tendered and debated (152,590). One longstanding, simple explanation has been the availability of hospital beds, as determined by explicit resource allocation decisions made by the PGP plan (152,590). Such constraints, if rigidly adhered to, could be effective controls. Evidence of impact on physician decisionmaking, however, is minimal **(619).** Another hypothesis, currently supported by the Group Health Association of America (GHAA) and others, is that the combination of peer pressures, organizational factors, and cavitation motivates physicians to act in accord with professional incentives as well as the constraints of the HMO (590). A third hypothesis is that there may be something "different," i.e., in attitudes, values, etc., about physicians who choose to practice in an HMO rather than in a more traditional fee-for-service practice setting. Although this explanation is intuitively appealing, virtually nothing is known about the possible effects of "preelection" on physician behavior in the HMO setting (372).[9]

The influence of economic incentives and financial constraints on resource allocation decisions faced by the plan and physicians in the HMO may depend on the risk-sharing arrangements between them. This characteristic varies (generally) by type of HMO, but essentially it is the plan that is responsible for making administrative decisions affecting the financial viability of the organization, while physicians are still primarily responsible for decisions affecting the well-being of HMO enrollees. The influence of physicians, especially heads of services (e.g., a chief of surgery), however, is often substantial.

In general, theory would have it that the greater the share of risk borne by each of these entities, the greater is the influence of these incentives and constraints on their decisions. With respect to explaining the apparent "cost-conscious" behavior of HMO physicians, this is a tenable, but vigorously contested, hypothesis, With respect to the plan, however, the criterion of risk assures that it is held accountable for meeting the conditions fostered by prepayment: Its impact on administrative resource allocation questions made by the plan is explicit and immediate.

The third criterion of a defined population exerts an influence only insofar as it determines the total revenue available to the HMO, and the nature of the population for whom it must provide services. The defined population of an HMO currently ranges from about **3,000** to 2.5 million enrollees (the latter in five different State regions) **(372).** Predominant age and sex characteristics of the enrolled population may differ markedly between HMOs. The size and character of the HMO population will directly influence the types of decisions to be made, as well as the administrative structure required to manage the organization. But in any case, at any given point in time, the HMO can project its annual revenue, and enrollees, and therefore estimate the demand for various services. Such foresight facilitates the achievement of technical efficiencies, i.e., the cost per unit of service, and also enables the HMO to make tradeoffs in providing benefits and services that will be appropriate to its particular population.

To summarize, the internal economic incentives and financial constraints faced by the HMO derive from the fact that the HMO plan has finite resources to allocate, is legally responsible for meeting the medical needs of its member population, and is also primarily responsible for maintaining its own fiscal viability. This set of conditions, arising from the distinctive characteristics of prepayment and risk, guarantees that the HMO will be "cost conscious. " But the internal economic incentives and financial constraints do not necessarily require that the HMO be any more (or less) concerned with efficacy, or effectiveness, of a ben-

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[8]The fee schedule used to set reimbursement rates for ambulatory services in the IPA is another example of such direct controls imposed on physicians by the plan. Some IPAs have imposed very rigid controls on physicians *(187).*

[9]There is also a self-selection process suspected for enrollees which is believed to affect choice and disenrollment. Clearly, they see the plan as having some advantage, as witnessed by their tendency to display "voice" (rather than exit) in the tace of dissatisfaction with services (372).

efit or service in allocating its resources than any other health care delivery setting is,

The Competitive Imperative

This influence derives chiefly from the characteristic of voluntary enrollment, which implies that prospective enrollees have a choice between alternative health care providers (and insurers), and that they have the option to disenroll, as well as to enroll, in the HMO (372). The HMO, existing in a predominantly fee-for-service environment, must compete for its enrollees: Its financial viability depends on its ability to generate revenue through enrollment, and consequently, on its ability to attract enrollees.

The HMO may compete on the basis of cost, as well as the range and quality of benefits and services it can offer, In this regard, HMOs have a competitive "edge" in the health care market, by virtue of their generally much lower out-of-pocket costs. Competition can reintroduce the concern (by consumers) for cost (300). In the situation where HMOs compete against one another, the limited data available suggest that relatively small differences in premiums can attract enrollees (372). But when premium rates are comparable, the question becomes one of offering the most (and/or best) services for the least money (95).

The ultimate question for the HMO is what impact any change in the services and benefits offered will have on the plan premium. Theoretically, the use of "cost-effective" techniques in the development of a comparably attractive, yet low-cost, product would seem to be essential to attract and retain customers in a competitive market. At the least, the competitive imperative does not negate the potential for viewing resource allocation decisions in the HMO as CEA/CBA problems.

Ultimately, however, the competitive imperative means that the HMO must meet the expectations of enrollees regarding benefits and services, expectations that are dictated largely by the norms of practice established by the predominating fee-for-service mode of health care delivery. The leverage of enrollees to demand these services stems from their option to disenroll.[10]" The competitive imperative implies that simply because competing providers are offering a service, and doing so in a certain manner, the HMO will be induced to provide it also, and in a similar fashion. The fact that HMOs must also compete in the marketplace for physicians reinforces this tendency: HMOs will seek to provide opportunities for medical practice comparable to those offered in the fee-for-service setting in order to attract and retain physicians. This situation will change as the number of HMOs increases and they are forced to compete among each other as well as with fee-for-service care.

The fact that HMOs must compete for both enrollees and physicians means that the HMO will try to provide benefits and services similar to those available from other insurers and providers—but within its financial limits. The HMO's overall strategy in making resource allocation decisions will be to maintain or improve its competitive position. This implies that any possible efficiencies achieved in providing services, reductions made in the number and/or range of services, and/or tradeoffs made in alternative ways of offering services that might be induced by the internal economic incentives and financial constraints will be subject to the condition that the HMO remain an attractive, competitive alternative.

Thus, given both a competitive imperative and inherent economic incentives and financial constraints, the two questions most commonly of immediate concern to the HMO are: 1) how to allocate its limited resources to provide the given benefits and services in the most efficient manner possible, and/or 2) how to reduce inappropriate services and benefits (in volume and frequency) without a perceptible loss in quality **(373),**

Evidence appears to indicate that the cost savings of HMOs are due to changes in the mix and number of services provided. Overall, ambulatory services received by HMO enrollees are no

[10]There is a considerable literature regarding what is essentially a cost-benefit decision made by consumers to join or opt out of an HMO: ". . . *they face the question of whether the benefits they are* receiving are worth the premiums they are paying" (159).

different from those received by persons covered under conventional insurance. [11] (Among other things, this reflects the imperative of the HMO to meet the consumer's demand for ambulatory services.) Rather, as previously indicated, almost all of the cost savings can be directly attributed to the lower (by 30 percent overall) hospital admission rates for HMO enrollees (373).

Similarly, empirical data suggest that persons conventionally insured with full coverage for preventive ambulatory visits receive at least as many, if not more, preventive services than HMO enrollees (374). In contrast to the ideology implied in the name "health maintenance organization, " the apparent greater use of preventive services in HMOs appears to be attributable to better financial coverage, i.e., the result of lower costs of ambulatory visits faced by enrollees (374).

Because of its financial incentives and constraints, the HMO (by and large) tends to deliver a "conservative" brand of medical care. This implies that where the cost of a preventive service exceeds that of treatment, and efficacy is unclear, the HMO tends not to provide that service, or to provide less of it (374). With respect to preventive services that are of questionable efficacy, it will tightly ration the resources required to provide them. For example, the Northern California Kaiser Foundation Health Plan shifted its stance on health examinations in 1976 by advocating a triannual rather than an annual physical checkup for individuals under a certain age.

Thus, in allocating resources in the HMO, the rationing of preventive services appears to be one of the principal questions where the potential benefits (i. e., efficacy) of a service are considered in relation to costs, and to which formal CEA/CBA has most often been applied. That HMOs should show concern for the efficacy and effectiveness of preventive (rather than surgical, for instance) services in relation to costs maybe interpreted as another reflection of their adherence to the status quo. More specifically however, it is the low "market medical benefit" of preventive services that allows and encourages questions regarding their efficacy.

In summary, the limited empirical evidence available regarding HMOs suggests that the pertinent resource allocation questions and decisions are foremostly influenced by the competitive imperative, but are also ultimately subject to the inherent economic incentives and financial constraints in the HMO.

With the exception of preventive (usually secondary) services, the rationing of resources in the HMO at an administrative level generally does not include the explicit consideration of efficacy relative to costs, or of whether or not a benefit or service should be offered on that basis. Instead, the competitive imperative introduces the consideration of a "market medical benefit, " or the qualitative attribute(s) of a benefit or service that will attract and retain physicians and enrollees in the marketplace. While these are not societal benefits, they are assumed to have some health benefit **(619).** For HMOs, these benefits are largely prescribed by competitors in the marketplace: Within HMOs, they are rationed in terms of how and/or how much to provide (rather than whether to provide them).

By and large, the resource allocation questions pertinent to this task are concerned with achieving technical efficiencies and/or volume and frequency processes. These are questions generally addressed by CEA/CBAs, though not by those that take health effects into explicit account. The concurrent appreciation and consideration of benefits and costs in HMOs may be regarded as implicit "net cost" CEA/CBA, and possibly help lead to the "cost-effective" behavior of the organization. The remaining discussion in this chapter supports these conclusions with evidence regarding the use and potential usefulness of CEA/CBA in HMO resource allocation decisionmaking,

[11] There is considerable variation, however, in ambulatory visit rates by type of HMO. IPA model HMOs have a rate almost 20 percent higher than that of conventionally insured persons; PGP model HMOs have slightly varying rates that even out to be only negligibly different from the rate for conventional groups.

SURVEY OF HMOS

Given the limited scope of this investigation, and the tremendous diversity possible in organizations characterized as HMOs, the HMOs included in this small survey do not begin to approximate a representative sample. The HMOs surveyed here were identified with the assistance of several individuals who, on the basis of their personal knowledge and experience with them, suggested various organizations that they thought might be most likely to be able to respond.

The final selection of 11 HMOs was based on whether each HMO had recently made some important resource allocation decision, since this decision was to provide the initial basis of discussion during the interview. Table 5 indicates the HMOs interviewed, specifying for each the following traits: 1) type of HMO, i.e., staff or group model PGP, or IPA; 2) age, i.e., the length of time in operation; and 3) size, i.e., the number of enrollees. These variables were recognized as potentially important influences on findings. In the small survey sample, however, it was not thought worthwhile to try to select HMOs according to these traits.

Only one or two individuals were interviewed at each HMO, except at Kaiser, where several individuals in research offices were interviewed. [12] Consequently, some of the individuals interviewed were analysts, some were administrators, and some were physicians. But some individuals acted in two or even all three roles.[13]

There is reason to be skeptical about the generalizability of findings regarding the usefulness of CEA/CBA in these few HMOs. Because the HMOs and individuals interviewed were selected on the basis of informed (and candid, if not objective) opinions as to their ability to re-

[12] Special assistance in selecting HMOs and the individuals in them was given by Mildred Arrill of OHMO. The suggestions of Frank Seubold, Harold Luft, Martha Kendrick, Jane Willems, and Morris Collen were also helpful in singling out the several organizations and persons surveyed here.

[13] In addition to the individuals interviewed in HMOs, almost as many persons from OHMO, trade organizations, and scholars of HMOs were interviewed. Many of the comments were useful in both anticipating the findings and drawing conclusions based on them. The comments of these individuals are referenced throughout the discussion. Referenced individuals are listed in the bibliography.

Table 5.—HMOs Surveyed: By Type, Age, and Size

Name of HMO and location	Type	Age	Size (# enrollees)
1. Capital Area (Albany) Community Health Plan ..... Latham, N.Y.	Staff PGP	3 years	27,000
2. Arizona Health Plan ..... Phoenix, Ariz.	Group PGP	8 years	66,000
3. Group Health Cooperative of Puget Sound ..... Seattle, Wash.	Staff PGP	33 years	270,000
4. Harvard Community Health Plan ..... Boston, Mass.	Staff PGP	11 years	85,000
5. HMO of Pennsylvania ..... Willow Grove, Pa.	IPA	5 years	42,000
6-8. Kaiser Foundation Medical Care Plan[a]	Group PGP	47 years	
Northern California Region, San Jose, Calif.			1,637,050
Southern California Region, San Diego, Calif.			1,554,500
Oregon Region, Portland, Oreg.			231,743
9. Northern Community Health Plan ..... Glenview, Ill.	Group PGP	5 years	23,000
10. Rhode Island Group Health Association ..... Providence, R.I.	Group PGP	8 years	30,000
11. Rutgers Community Health Plan ..... New Brunswick, N.J.	Staff PGP	4 years	38,000

[a] In addition to individuals at these three HMOs, individuals at the Kaiser Permanente Medical Care Program research facility and the Kaiser Foundation Health Services Research Center were also interviewed.

SOURCE: Office of Technology Assessment.

spend to the inquiry, however, these findings may represent an estimate of the maximum potential for the use and usefulness of CEA/CBA in HMOs. Thus, although these findings should be regarded as very preliminary, they are not without significance.

GENERAL FINDINGS

As previously mentioned, the discussions with individuals in the HMOs centered on some specific recent resource allocation decision, and whether or not CEA/CBA was used, or would have been a useful analytical technique in the decisionmaking process. The resource allocation questions, analytical techniques used, and their role in the decisionmaking process were ascertained. To ascertain the type of analysis used, a brief technical description was obtained. Inquiry regarding the usefulness of analysis was concerned with determining the decisiveness of its impact on the final decision; its effect of a political, ethical, economic, or other nature; and its contribution to the decision made as viewed by the various parties affected. Each of these three topics—resource allocation questions, analysis used, and the usefulness of the analysis—are discussed below with respect to both the current and potential appropriateness of CEA/CBA in HMO decisionmaking. In addition, the extent to which HMOs have institutionalized the resource allocation process is discussed. Finally, the current and potential applicability of CEA/CBA to decisionmaking in OHMO, and to the HMO statutes that it administers, is discussed.

Current Applicability of CEAICBA in HMOs

Resource Allocation Questions

A wide range of resource allocation questions were reported in the surveyed HMOs. Most frequently, HMOs were engaged in a simple "make or buy, " "lease or purchase" type of decision. The question was not so much whether to do something at all, but whether something was less costly to do "in" or "out" of the HMO. The most commonly cited example of such a decision was made with respect to the addition of physician staff, usually a specialist (i. e., whether to contract with a physician in the community, or to hire another physician on staff of the HMO). Other examples of "lease **or buy"** types of decisions were made with respect to obtaining: 1) improved transcription (of medical records) services; 2) diagnostic ultrasound equipment; and 3) new physical facilities. The "make or buy" decisions arise because of the capital expenditure issue associated with them, but more importantly, they arise from the imperative exemplified in these statements:

> Every time a new technology comes into medical practice, we groan because we have to find some way of getting it . . .(527);
>
> If the "DOCS" come to us and say they want to do open heart surgery, the question we face is not whether, but how we can do it (369); and
>
> We are conservative, practicing "spartan" medicine, (and) perceived of as "tightwads," but we are also constrained to go along with what is practiced in the medical community . . . If someone introduces something new, then we have no choice but to get it or do it somehow (619).

A common resource allocation question in the HMO, therefore, is "How can we provide what is demanded?"

Other resource allocation decisions reported in the HMOs, however, tended to be more concerned with whether or not a particular service should or could be provided. Some of these decisions were whether to: 1) implement the COSTAR (medical information) system rather than some alternative systems, 2) install a centralized v. a decentralized automated reception system, 3) cease doing intestinal bypass surgery on obese patients, 4) implement a health education program (2 cases), and s) offer biofeedback as a palliative care measure.

Even in the case of the two-option "make or buy" type of decision, where the benefits or ef-

fects are generally accepted as being similar, the concern is not solely for efficiency. "Relative to just saving money, there is (at least) the assumption that what's being done is of benefit . . .," either to the organization (in the case of administrative decisions), or to enrollees (in the case of medical services); the benefit is assumed **(619).** Often, questions about benefits were identified on the basis of patient or physician dissatisfaction with current practices and the desire for improved quality rather than a less expensive way of providing the service.

In the case of questions involving the aspect of whether to provide a service, however, the relative benefits and effects of various alternatives were explicitly compared, as in the case of COSTAR and the automated reception system decisions. In the case of one of the health education programs, the decision involved evaluating the efficacy associated with these services. In particular, planners were specifically asked to measure the health benefits, i.e., how is this service going to improve health? In order to answer the question of "should we spend those dollars?" the health benefits (and to whom they pertained) had to be proven. In the case of biofeedback, a clinical trial of that technique was being undertaken to ascertain whether it should be made available.

In all questions, the associated parameters were limited by the rigorous constraints of time and money. Consequently, much of the resource allocation process in HMOs could be characterized as "problem-solving," in which problems were often identified by those closest to them. For example, at an administrative level, there is a great deal of monitoring—of hospital admissions and length of stay, referral patterns among physicians, and so on. In the case of intestinal bypass surgery, the problem was flagged by the observation that "too many" complications were developing in postsurgical obese patients. In the case of the health education program, it was noted that the HMO was losing its "healthier" enrollees, because "sicker" enrollees impeded their access to HMO services. In that case, it was thought that a health education program might increase enrollees' satisfaction and thus help retain the "healthier" ones. Another "problem solving" example is found in the implementation of a health appraisal system. In that case, the problem was recognized, analyzed, and resolved by the physician in charge within the medical department. The physician in charge faced a huge backlog of initial physical examinations due to recent rapid expansion of the HMO and limited physician manpower. On the basis of previous research and principles of multiphasic health testing (106), he devised a "linear system" for "batching" intake examinations and freed physician time by substituting other health personnel insofar as possible.

Thus, much of the resource allocation occurring in HMOs may be characterized as incremental decisionmaking where the parameters are subject to the constraints of cost and timeliness of a solution given the existing situation and possibilities: The number of available options is usually very small. Even at an administrative level, there are few "strategic" resource allocation questions. There appears to be very little "zero-based" decisionmaking in HMOs. The HMO rarely faces the question of whether to decentralize or centralize (e. g., to have two or six physical facilities). Instead, it is faced with the question of where to build the next facility.

At an administrative level, HMOs are simply trying to be more efficient, an objective which is not so different from that of other organizational entities delivering health care (369). "Cost effestiveness, " however, is viewed as being an integral consideration in the decisionmaking process at all times. An interviewee's perception of whether the resource allocation questions addressed by HMOs were questions of cost effectiveness depended greatly on whether the individual was familiar with the formal technique of CEA/CBA or instead had a more "lay" sense of the meaning of "cost effectiveness" (such as that exemplified in the statement "we must be cost effective"). In the former case, the resource allocation questions were generally not viewed as being amenable to sophisticated, health-effects-oriented CEA/CBA, but in the latter case, they were viewed as problems of cost effectiveness.

Analyses Used

Generally speaking, formal CEA/CBA beyond "net cost" analysis was not found to be used by **HMOs. This does not imply, however, that benefits were not integrated into their analyses. It was recognized that it is possible to be efficient without being effective. A common perception was that** in considering every major decision, there would be an evaluation as to whether the service would be "worthwhile." The criteria of both cost and quality were included in making this determination: The first question was **often, "how will this affect the quality of service?" and the second, "what is the impact on the cost per member per month?" Analyses, therefore, involved the evaluation of both costs and benefits: Analyses were characterized by** the **objective of ascertaining the financial impact of a** given (or multiple) option(s), but the idea of quality loomed over all considerations.

Two examples illustrate this generalization regarding analyses used in HMO decisionmaking. The example of whether to lease or purchase ultrasound equipment illustrates that benefits were also taken into consideration in the costing out of options, and that the question of medical efficacy of diagnostic ultrasound also arose in considering those benefits. Questions surrounding this "cost" analysis included not only what the costs of the lease option would be, but also whether the equipment could be put to other uses (besides obstetrics) in the HMO. This question involved ascertaining whether ultrasound diagnostics could replace other diagnostic tests, for example, CT scans in some cases, and the costing out of such "replacement" effects. Radiologists and internists in the HMO were questioned on the potential benefits of these other uses of ultrasound. These physicians concluded that the use of ultrasound in these capacities would still have to be backed up by other diagnostic tests. Consequently, the decision was made that it was not "worthwhile enough" to purchase the equipment at that time, and the lease option was chosen.

The second example of the level of detail attained in evaluating the costs and benefits associated with a particular decision is the case of the COSTAR medical information system. Although, it was clear from the outset that the COSTAR system would probably be a more expensive option, it was also recognized that it could provide a completely different level of service in the HMO. An extremely detailed analysis of per dollar-impact over the course of several years was conducted. Part of this analysis involved estimating how far into the future the various options would work and included such factors as the rate of growth (of the HMO) and the future addition of a (third and fourth) medical center. Qualitative aspects of "after hours" accessibility and the continuity of care were also crucial factors. Even potential problems of future energy shortages and how they could affect an automated recordkeeping system were considered. Though in the long run, a projected yearly cost difference of $20,000 to $40,000 more for COSTAR v. other systems was estimated, COSTAR was chosen on the basis of its clear-cut superiority, i.e., its "different" level of service.

A wide range of analytical expertise and resources was found to be applied to the resource allocation questions in different HMOs. Practicing physicians, economists, operations researchers, and accountants in various administrative and/or research capacities were found to be "analysts:" Even in small HMOs, where the organization is run very "lean" and there are few spare resources to devote to analyses used in decisionmaking, analyses were being conducted and were generally more sensitive to the costs and benefits of various decisions than might have been expected. Where resources allowed, as in the case of a health education program, methodological expertise was sought to deal with the question of determining the associated health benefits. Only the very largest HMO was found to have conducted a formal CEA/CBA "in-house" (and this with some Federal support). By and large, the "in-house" efforts and resources applied in pursuit of these questions were a function of the size, age, and financial situation of the particular HMO. Though HMOs were generally thought of as being "too small" to do CEA/CBAs, it is clear that none of the HMOs in this limited survey were too small to conduct analyses that were respon-

sive to the perceived information needs of the problem at hand.

The analyses themselves ranged from "back-of-the-envelope" to rigorous fiscal analyses. The consensus opinion was that most resource allocation questions relevant to the HMO did not require formal, sophisticated CEA/CBA to ascertain a satisfactory answer because the parameters of the decisions were relatively narrow. In the case of developing and implementing a health appraisal system, the physician in charge did a "back-of-the-envelope cost analysis, " in the belief that if cost savings (in terms of substation of physician for other medical manpower) were not evident at that crude level, they would probably not show themselves in a more detailed version of analysis.

It was commonly appreciated that there was never enough time (or money) to do all the analysis one might wish, and that decisions had to be, and ultimately are, made on the basis of the information at hand at the time the decision is made. Concomitantly, however, there was an overall confidence in HMO staff's comprehension of the scope of most resource allocation questions and in the extent and depth of information required to get an answer that was satisfactory, if not absolutely optimal.

The OTA survey examined whether the HMOs borrowed CEA/CBA information from external sources if the HMO's own resources were not sufficient to produce such information internally. The HMOs reportedly consulted the medical CEA/CBA literature, but relatively infrequently used it as a basis for decisions. The decisions in which it was used as a basis (all made by a single HMO) included the elimination of routine chest X-rays, modification of adult physicial examinations and pediatric prevention schedules, and indications for CT scans, In other cases, including electronic fetal monitoring (EFM) and coronary bypass surgery for angina, the literature was also considered, but was regarded as too equivocal to be used as the basis of deciding to withhold these services,

Thus, while being "cost effective" was viewed as essential, the rigorous forms of CEA/CBA were seen as being largely irrelevant in these HMOs. Rigorous CEA/CBA studies, among the HMOs surveyed, were found to be very rarely required by resource allocation questions, even more rarely conducted, and only infrequently borrowed and applied.

Role in Decisionmaking

A general consensus on the role of analysis in the resource allocation decisions found in this survey is summarized by the following statement: "The analyses are critical, . . . (we) couldn't make the decisions without them, but the analyses do not make the decisions . . ." **(330).** Thus, analyses were widely appreciated as an integral part of the decisionmaking process in HMOs, even though that process is still perceived as an essentially political one. Important functions of these analyses were the following: 1) to define the parameters of the decision at hand, 2) to clarify the impacts of various options, and 3) to serve as a basis for the discussion of issues associated with a particular decision.

These generalizations are exemplified in the case of COSTAR. The main impact of that analysis was said to be that it was ". . . the basis for bringing all the issues forward in a political power struggle . . ." (568).

The aforementioned observations suggest several conclusions regarding the usefulness of analyses in the decisionmaking process. First, they indicate that when introduced into the "political" decisionmaking environment, any "objective" analysis will come under harsh scrutiny by those representing the opposing viewpoint. They also suggest the constraints of time and resources within which analysts and HMO managers and other decisionmakers must operate, i.e., how few options present themselves, and how many fewer still may be analyzed and considered. They also suggest that among these alternatives, the option about which analysts and decisionmakers may have the most information, or with which they are most familiar, may have a greater likelihood of being chosen: The decision made will often be the one where the most detailed information is available. While this may not be the "best" or optimal choice, it is a rational one.

Many of the cost analyses might technically be termed "net cost" analyses, in one sense an extreme of CEA/CBA. Yet when these analyses were incorporated into the decisionmaking process, decisionmakers became extremely sensitive to the aspects of quality and benefits: Though it was not considered to be the most "cost-effective" option, i.e., in the sense of least cost, the COSTAR option was chosen anyway on the basis of its clearly superior capabilities (as well as power politics).

The COSTAR example illustrates that perhaps the first reason to chose a particular option is so-called "program effectiveness." At a subliminal level, it is physician and enrollee satisfaction which is the benefit of foremost concern. There is great hesitance to do anything that does not have readily discernible benefit, and anything without an immediately perceived value (however defined) will probably not be done at all.

> We have a long history of taking care of the medical needs of people. It is very difficult to justify withholding something on the basis of analyses in general, and on the criterion of costs in particular . . . What people want is what is important . . . (527)

Institutionalization of Decisionmaking Processes

The degree to which resource allocation decisionmaking and the analyses were formalized, or institutionalized, is (to some extent) a function of the size and financial situation of the HMO, and consequently, of the administrative structure required to manage it. The majority of examples of resource allocation decisions discussed so far may be characterized as "problem solving," where those individuals closest to the problem have identified and possibly even analyzed and resolved the problem. In general, these persons were not "handed" solutions (i.e., resource allocation decisions) by managers from higher echelons of the administrative hierarchy. Such "problem solving" was critical to the efficient allocation of resources within particular HMOs, and is a standard, ongoing feature, even if it is not a formalized procedure.

Institutionalized, formal analysis for resource allocation decisionmaking was the rare exception which proved this rule. The decisionmaking process meeting these criteria that was most often cited was the "budgetary process." It was often perceived that CEA/CBA takes its most explicit form in the HMO in the annual budget-setting process. In HMOs, both large and small, the budgetary process involved projecting the annual revenue (based on forecasts of membership), and estimating the budget (based on estimates of demand for services by that population).

In two of the largest and oldest HMOs, Kaiser Foundation Medical Care Program (KFMCP) and Group Health Cooperative of Puget Sound (GHC), examples of very rigorous, institutionalized procedures for allocating resources were found. Without delving into the complex organizational and administrative structure of Kaiser, it is sufficient to say that: 1) there is an overall vertical hierarchy within both the overall organization and in each of the five regions, and 2) that the program has established considerable (relative to any other HMO) research capabilities and "in-house" analytical expertise. In both the KFMCP Central Office and in the Southern California Regional Office, there are what are called "benefit-cost offices." These offices essentially perform financial impact analyses, i.e., they examine the impact of a proposed change in resource allocation on the cost per member per month. According to the director of one of them: " . . . We do not do cost-benefit analysis here—at least like they are done in academic or government institutions . . . where the bottom line is life-years saved, or some other society-based measure . . ." (527). Analysis of both costs and benefits is based on the internal economics of KFMCP. Thus, Kaiser does appear to conduct some "program-perspective" CEA/CBAs.

At least in the Northern California Office, requests for analyses are approached in a standardized fashion, with preprinted worksheets being used to evaluate various proposed changes. Discounting (usually at the rate of 10 percent) and sensitivity analysis are a standard part of the evaluation. The analyses typically examine

outcome measures such as total net benefit and internal rate of return on investment, as well as a cost-benefit ratio. They are key planning tools in daily use at Kaiser, and results are regarded as proprietary information. Both the performance and use of these analytical tools have been incorporated in the organizational structure and administrative processes of KFMCP.

Two other examples of the institutionalization of research capabilities and analytical expertise in Kaiser are found in: 1) the Division of Technology Assessment in Oakland, Calif.; [14] and 2) the Health Services Research Center of the Bess Kaiser Foundation in Portland, Oreg. At the former, the assessment of the efficacy of biofeedback as a palliative care measure for various conditions is an example of the research conducted in the relatively new analytical entity. This research will be one input, along with "... organizational impact, long-term cost considerations, and patient satisfaction, as well as other alternative measures . . ." in making the decision of whether or not to include biofeedback as a benefit (300).

The Health Services Research Center has published several studies on the "cost effectiveness" of the substitution of nurse practitioners and/or physicians assistants for physician manpower in the HMO setting (496,497). Research on "do-not-admit" surgery was also conducted at this center (263). [*5]

The results of both have been used throughout KFMCP (as well as other HMOs) resulting in efficiencies and thus, cost savings. Interestingly, although

> there was never a conscious decision made ~by" management) to pursue a "do-not-admit" strategy, there were soon 20 percent of all surgeries being done on a "do-not-admit" basis. As the idea "caught on, " and administrative support developed, 40 percent of surgery was soon being done in this way . . . . The cost implications of this change (in physician behavior) were enormous . . . (263)

This and the nurse practitioner/physician assistant example illustrate the use of formal methodologies to evaluate various configurations of resources, but the analyses were only loosely, and sometimes not at all, related to "across the board" policy decisions made by management.

At GHC in Seattle, the Pharmaceutical and Therapeutics Committee is a specific example of the institutionalization of cost-effectiveness and cost-benefit considerations in organizational decisionmaking. Prescription drugs, included in the benefits package of GHC enrollees, are subject to review and approval by this committee: The objective is to regulate the availability of prescription drugs (within the plan). No GHC physician may introduce a pharmaceutical into the "stockpile" of those already available unless it has been passed by this committee. The process is similar to those of formularies in hospitals. For example, with the appearance of a "new" antibiotic on the market, an "across-the-board" decision will be made to use either the new or the old drug, but not both, in that HMO.

In considering the addition of another drug, some of the questions asked by the committee were (given in this order): 1) Is it effective? 2) Is it costly? 3) Are there other, and perhaps too many other, options already in stock? and 4) What is its effectiveness and cost relative to these options? Data for these decisions were taken from the current available literature, clinical experience, and so on. It was claimed that this control resulted in very substantial **(50** percent) reductions in the cost per (comparable) prescription for GHC enrollees. The committee is an example of a formal review mechanism in one HMO that has institutionalized the consideration of "cost effectiveness" as a decision criteria—and specifically the medical (or health) benefit—if not the actual conduct of formal analyses.

Potential Applicability of CEA/CBA in HMOs

Resource Allocation Questions

The question of whether something is cost effective or cost beneficial—in the most formal

[14] This new Division was created in the summer of 1979, and is currently headed by Dr. Morris F. Cohen.

[*5] "Do-not-admit" surgery refers to cases in which relatively simple surgical procedures are performed in the operating room, but the patient is not admitted unless there are complications (263).

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analytic sense of the term—is by and large an academic one in HMOs. As perceived by the Director of the Kaiser Health Services Research Center (262):

> . . . posing the question inherent in these analyses is like asking ". . . if the money supply is tightened in our economy, then . . ." These analyses are too broad and inclusive, while the problems of management are much more constrained: CEA/CBA do not fit the parameters of management decisions.

Another criticism of CEA/CBA voiced by both analysts and decisionmakers was that, besides insufficient time and money, there was inadequate freedom to ask such questions (619):

> We must serve the same psychological and social, as well as medical, needs that traditional providers do . . . we must be able to react to the same emotional and social pressures . . . (even though) we think in per capita terms . . .

There are two reasons why ". . . it would be very difficult to justify withholding a service on the basis of a CEA/CBA . . ." (527). First, as in the fee-for-service practice setting ". . . an ethical/medical question intervenes in trying to use **CEA/CBA as a basis for making resource allocation decisions . . ." (588). Secondly, the HMO faces competitive pressure to offer as similar** (in appearance) services as possible to those of fee-for-service providers in order to meet the expectations of both physicians and enrollees regarding comprehensiveness and quality of service.

Decisions involving ethical and moral questions, as well as those that are otherwise value laden, are perceived as "risky" areas for the rigorous application of analysis such as CEA/CBA. For example (619):

> . . . although the availability of hospital beds may be held at a very finely tuned ratio of 1.5 or 1.6 beds per 1,000 enrollees, when there is a "crunch," then (we) physicians simply seek other available hospial beds in the area . . . we don't not admit patients who in our judgment require hospitalization . . ,

Similarly, the implementation of a midwife program initiated by enrollees would not be a decision likely to be subjected to, or influenced by, a formal analysis.

Decisionmakers will not be inclined to rely on analysis in addressing resource allocation questions where analysis could too easily (because of uncertainties) lead to the "wrong" answers.

Generally speaking, the question of whether something is cost effective or cost beneficial in the absolute sense is seen appropriatel, as having little overall practical utility in the HMO. This view was expressed by two high-ranking officials of GHAA:

> . . . There is a misplaced emphasis on the "absolute" cost-effectiveness of capital intensive investment like lab analyzers, or CAT scanners, or open heart surgery facilities when the more important question is how (at what level or intensity) these are utilized . . . (588);

and

> . . . Much of the cost-effectiveness literature is around decisions on individual technologies, but it is the decisions regarding volume and frequency processes (such as laboratory and other diagnostic testing) where there is the greatest potential for cost savings . . . (299).

HMOs seem to be more straightforwardly concerned with returns to scale, returns on investments, etc. Thus, in HMOs, "cost effectiveness" is correctly interpreted as a relative, rather than an absolute, attribute: There may be many "levels" of "cost effectiveness" associated with a particular option rather than an answer to the binary question of whether or not an option is "cost effective. "

Analyses and Role in Decisionmaking

HMOs' "in-house" research and analytic capabilities were usually found to be too limited (in terms of both money and expertise) to allow the conduct of rigorous and broad-based CEA/CBAS or clinical trials of efficacy. Other potential ways of obtaining CEA/CBA information are through technical assistance and the existing CEA/CBA literature.

Technical assistance by which CEA/CBA might be obtained could potentially come from three sources: 1) private consulting firms;

2) GHAA, the trade association for prepaid group practice type HMOs; and 3) OHMO in DHHS. The financial resources required to obtain this from private consulting firms would make this source an infeasible one for many (probably most) HMOs, even if they were interested in pursuing a CEA/CBA problem. OHMO concedes that there is no reason why CEA/CBA could not be done under the existing statutory provision for general technical assistance to HMOS (159,313). Although it is conceivable that a CEA/CBA problem could be undertaken within this technical assistance provision, the possibility seems remote in light of competing, and more pressing, problems. Finally, GHAA also brokers and provides technical assistance to its member HMOs. GHAA's Associate Director confidently stated, however **(588):**

> **. . .** I would expect that the chances of an HMO's approaching us to do a CEA/CBA are next to nothing . . . because these questions just aren't there . . .

Although several instances were found where the available CEA/CBA literature was consulted regarding the specific technologies, this resource was more often regarded as equivocal—both because of the technical difficulties and uncertainties of analyses, and the lack of available efficacy information **(619):**

> **. . .** (we) have looked to the literature for a consensus on EFM, . . . if there were decisive evidence, then we could use that to make decisions regarding its use . . . but there's not . . . (we) wish there were, but there isn't . . .

The issues of transferability of existing CEA/CBAS to individual HMOs in particular, and to HMOs in general, adds yet an additional source of uncertainty **(295):**

> **. . .** We just don't have the health delivery knowledge (in HMOs) yet to be able to predict "what happens if 'X' happens?" . . . even in one HMO, let alone to generalize . . .

All things considered, a representative consensus would seem to be that " . . . various uncertainties make formal analyses available in the literature an inadequate basis for decisionmaking . . ." (615).

Thus it seems that even if CEA/CBA were directly relevant to the resource allocation processes in HMOs, most HMOs would be hard-pressed either to produce or even to find and borrow CEA/CBA information that would be useful to their individual needs.

The receptivity of decisionmakers to analyses is summarized in the following comments:

> . . . when a CEA/CBA will tell you to throw out a certain option, and *you* intuitively feel that it's the right one . . , usually you disregard the analysis . . . but in any case . . . you scrutinize and challenge the analysis . . . (568);
>
> . . . Decisionmakers know or will find the limits of analytic tools, even when analysts don't, because if the analysis is to be used as a basis for decisionmaking . . . and if the analysis turns out to be wrong, it is the manager who must endure the consequences . . . (295).

Given the motivation—political, ethical, economic, or other—to do so, someone can cite enough technical problems to make any analysis equivocal. Recommendations for changes in resource allocation based on analysis, therefore, have been and are likely to continue to be tentative, with pilot and demonstration projects and considerable nonanalytic input preferred as the basis for decisionmaking **(295).**

There also seem to be misgivings about the "overall *post facto"* nature of research generally, and about analysis that tends to follow rather than precede decisions. As noted by one observer **(588):**

> **. . .** CEA/CBA are in "vogue" again . . . When I worked for CDC (Center for Disease Control) years ago, we used CBA to evaluate tuberculosis vaccination programs, and I can tell you that even then, the answer came before the analysis was ever done, and it always justified the decision . . .

Institutionalization of Decisionmaking Processes

Although it was found that the conduct of analyses and its incorporation in HMO decisionmaking was typical of the HMOs surveyed, it was also found that there was considerable

variation among HMOs with respect to whether and how these processes were formalized. An additional finding was that even though sophisticated forms of CEA/CBA were not perceived as relevant, the issues perceived to underlie CEA/CBA were considered an essential part of the decisionmaking process at all times.

More specifically, however, if the most potentially pertinent question in the HMO is "at what level of provision is 'X' cost-effective?" then the institutionalization of particular organizational procedures such as peer review of patterns of use of various resources, what constitutes inappropriate use, and who is qualified to designate their use in the HMO, as well as the monitoring of referral patterns, hospital admissions, and the like, are potentially very useful institutionalized mechanisms for assuring cost effectiveness in HMOs. Many HMOs already have instituted some of these formalities, and as a result have achieved dramatic cost savings (344).

Because of the great diversity of HMOs and variation in the competitive positions they hold in their respective communities, however, it is important that these procedures remain "individualized" (rather than standardized), i.e., each according to the HMO's need for analyses and/or review (295). Recently, GHAA has recommended to the council of the National Center for Health Care Technology that a study be undertaken with the purpose of developing criteria for the acquisition of technological resources in HMOs. The proposed study would indicate the probable impact of various acquisitions on the costs and utilization of these technologies and their relationship to per capita and aggregate costs, as well as the applicability of findings to other clinical organizational settings (300). The challenge of this proposal is formidable indeed. Still, the results might be useful if posed as a *relative* guide or as one model to follow.

Statutory Requirements for CEA/CBA in HMOs

Until January 1, 1980, the regulations promulgated under the Health Planning and Resources Development Act of 1974 (Public Law 93-641) required that HMOs demonstrate the cost effectiveness of their requests to State health planning agencies for CON approvals of capital expenditures. During 1979, an extended process of developing a detailed set of criteria to be used to ascertain the "cost effectiveness" of a given proposal raged on. The 1979 amendments (Public Law 96-79) to the health planning law, however, exempted HMOs from CON review by local HSAS and approval by State planning agencies. Neither Federal nor non-Federal HMOs are now covered under CON regulations. Because of the new health planning law, the controversial endeavor regarding cost-effectiveness criteria for HMO proposals was suspended. In January 1980, the relationship and responsibilities of HMOs to local HSAS and State health planning agencies were described as being in a state of limbo (159).

OHMO has a formalized system to assist potential or young HMOs in making cost-effective resource allocation decisions. HMOs are perceived as businesses by OHMO, and the main talent and skills required are cited as those of management. Therefore, under the authority of the technical assistance provisions of the Health Maintenance Organizations Act, OHMO has developed and published six manuals or review guides addressing the topics of: finance, quality assurance and health services delivery, management information systems, marketing, structural and contractual relationships, and management arrangement assessment (308).

To aid applicant HMOs in making cost-effective decisions, the review criteria provide cost ranges and reasonable upper cost limits at various levels of enrollment with which quoted bids may be compared. Similarly, in the quality assurance and health services delivery review guide, a table indicates the number of physicians required, as well as staffing pattern suggestions, at various levels of enrollment. (These standardized criteria for cost-effective performance are intended to serve as benchmarks only, and are not meant to indicate mandatory standards.) In summary, it seems that the analyses, decisionmaking practices, and criteria and standards formally institutionalized in the administrative procedures developed by OHMO are very similar in spirit to those less formalized in, but typical of, individual HMOs.

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Appendix A.— Method of the Study

Most of the studies undertaken at OTA rely on the advice and assistance of an advisory panel of experts. The advisory panel for a particular assessment suggests source materials, subject areas, and perspectives to consider; assists in interpreting information and points of view that are assembled by OTA staff; and suggests possible conclusions based on the accumulation of information produced by the study. The panel members review staff and contract materials for accuracy and validity, discuss policy options of the study, and present arguments for and against the options and conclusions. They do not determine the report's final form, however, and are not responsible for its content, direction, or conclusions.

The advisory panel for the current assessment consisted of 12 experts with backgrounds in business, ethics, health policy, law, economics, statistics, and medicine. The panel was chaired by Dr. John R. Hogness, former President of the University of Washington and current President of the Association of Academic Health Centers, Two members of the OTA Health Program Advisory Committee, Dr. Stuart H. Altman and Dr. Frederick Mosteller, also served on the advisory panel.

The first panel meeting was held in Washington, D.C. (the site of all four panel meetings), on December 13, 1978. Panel members reviewed the study plan of the assessment, examined the need for specific case studies, and reviewed a number of suggestions for potential case study examinations. The case study approach, as part of the general study framework, is intended to illustrate the uses of CEA or CBA in health care, especially to evaluate medical technology, along with the impacts, and strengths, and weaknesses of these uses. The advisory panel was instrumental in helping the staff set goals for the assessment, establish boundaries for its focus, and define the basis for and role of CEA or CBA as a decision-assisting tool in the health care system. The panel was also helpful in identifying public and private sector uses and users of CEA/CBA techniques or information in decisionmaking.

To help select medical technologies for the final list of case studies, the following criteria were developed:

- examples of types of technology by function (preventive, diagnostic, therapeutic, and rehabilitative);
- examples of different stages of development and diffusion (not yet diffused, experimental or pilot, established in medical care);
- examples from different areas of medicine (such as general medical practice, pediatrics, obstetrics, radiology, and surgery);
- examples addressing medical problems that are important because of their high frequency or significant impacts;
- examples of technologies with associated high costs;
- examples of technologies in widespread use; and
- examples with sufficient evaluable literature.

On the basis of these criteria and panel recommendations, OTA staff selected the case study topics. In addition, the Senate Finance Committee had specifically requested four case studies: psychotherapy, respiratory therapy, diagnostic X-ray, and length-of-stay in hospitals. Nineteen case studies (identified in app. F) were added to the study plan (Background Papers #z, #3, and #5).

The second advisory panel meeting was held on February 14, 1979. The panel reviewed the literature gathered by OTA staff, suggested additional references and sources of information, and evaluated the case study plan. In addition, it examined the staff's preliminary work on CEA and CBA methodology to identify strengths, weaknesses, and omissions. The panel was also helpful in commenting on the decision area papers (e. g., use of CEA in reimbursement coverage decisions) that were being prepared by the staff. Finally, it was asked to review a separate study being conducted as part of the overall assessment: *The Marzagement* of Health Care Technology in Ten Countries (Background Paper #4).

Several subprojects were pursued: a survey of relevant State, Federal, local, and private and nonprofit agencies and organizations to determine the extent of use or support of CEA/CBA activities (app. B); and an extensive review of the health care Literature to develop a comprehensive bibliography of health care CEAS, CBAS, and related publications.

The psychotherapy case study (Background Paper #3) used a separate, additional advisory panel that performed functions similar to the ones the overall advisory panel performed for the full assessment. The psychotherapy panel met on August 7, 1979, in Washington, D. C., to review the study plan, suggest improvements, and evaluate the background work performed. This panel met again on November 15, 1979, to review and critique the initial draft of the case study.

The third meeting of the advisory panel for the full assessment took place on October 16, 1979. In gener-

al, the panel focused on reviewing the status of the various parts of the study. It was also asked to discuss the early drafts of the methodology report (Background Paper #1) and of the use of CEA in medicare reimbursement coverage.

During 1979, two additional subprojects were initiated: a survey of analysts who had performed CEAS and CBAS of health care technologies (app. C), and a paper examining the ethical implications of CEA and CBA techniques (app. D). The survey of **analysts** focused on the resource "costs" used to perform actual CEAS or CBAS. The analysts were also asked to estimate the expected or potential costs of an "ideal" study team doing CEA/CBA analyses of health care technology on a routine and continuing basis. The second subproject was a paper by the Hastings Institute on the ethical considerations of conducting and using efficiency-based analyses such as CEA and CBA in the health care system.

On January 18, 1980, the authors of the case studies assembled in Washington, D. C., to review the applicability of CEA/CBA to health care decisionmaking and to discuss the methodological or data problems they faced in trying to apply CEA/CBA to their case study areas. Case study authors also discussed the policy issues involved in the use of CEA/CBA in health care decisionmaking.

The final meeting of the advisory panel was held on March 28, 1980. At this meeting, the panel reviewed drafts of the summary report, including the policy options for congressional consideration. Using the comments generated at this meeting, OTA staff revised the assessment report drafts.

The results of this assessment are being issued in six volumes (described in app. F). A two- or three-tiered review process was used for each of the volumes and for each individual case study. The initial drafts were reviewed first by OTA staff and advisory panel members. In certain instances, outside reviewers were also asked for comments. After the authors completed their revisions based on the reviewers' suggestions and comments, the drafts were sent out for a second round of review by a much broader range of experts in a diversity of settings: Federal agencies, State or local offices, private and nonprofit organizations, academic institutions, practicing health professionals, consumer groups, and other selected individuals. Altogether, more than 400 individuals or organizations were asked to comment on drafts of case studies and other volumes of this assessment in the second round of review. The final report, the volume containing congressional options, was reviewed by more than 100. After appropriate revisions based on the comments received had been made, drafts were prepared for a final review by the assessment's advisory panel, by the Health Program Advisory Committee, and by other individual reviewers.

Appendix B.—Survey of Agency Use of CEA/CBA

Introduction

This appendix reports on the results of an OTA survey of the use of cost-effectiveness and cost-benefit studies by the major Federal health agencies, State and local governments, and nongovernmental organizations. For the most part, OTA's survey showed that CEA/CBA analyses are not frequently conducted or applied to health care decisionmaking. Further, when such analyses are used, they tend to be cost-benefit rather than cost-effectiveness analyses.

Office of Assistant Secretary for Planning and Evaluation, DHHS

In May 1966, John Gardner, Secretary of HEW (now DHHS), established five program analysis groups to conduct CBAS of several disease control programs. The objective of this effort was to provide a basis for comparing alternate programs and setting priorities for additional funding. Thus, an HEW official responsible for overseeing the analyses observed (266):

> HEW supports, or could support a number of categorical disease control programs whose objectives are, or would be, to save lives or to prevent disability by controlling specific diseases. The studies were therefore an attempt to answer the question: If additional money were to be allocated to disease control programs, which programs would show the highest payoff in terms of lives saved and disability prevented per dollar spent?

The effort was originally undertaken in response to a request by the Bureau of the Budget for thorough analysis of the costs, benefits, and objectives of existing and projected programs. That request reflected an interest in attempting to rationalize Government allocation decisions by building on the planning-programing-budgeting system adopted by the Defense Department under Secretary McNamara (637).

Ultimately, the five HEW program analysis groups produced several cost-benefit studies:

1. Disease Control Programs: Arthritis, 1966.
2. Disease Control Programs: Cancer, 1966.
3. Disease *Control Programs: Delivery* of Health Services for the Poor, 1967.
4. Disease Control Programs: Kidney Disease: Program Analysis, 1967.
5. Program Analysis: Maternal and Child Health Care programs, 1966.
6. Application of Benefit-Cost Analysis to Motor Vehicle Accidents, 1966.
7. Human Investment Programs: Vocational Rehabilitation, 1967.[1]

In general, the impact of these studies appears to have been very limited by existing political and bureaucratic considerations, methodological shortcomings in the analyses, and unrealistic expectations of the impact such evaluation studies could have on decisionmaking (637).

One of the analyses, however, did have a major impact. The analysis of maternal and child health programs that examined the cost savings produced by federally sponsored periodic screening for low-income children played a major role in congressional passage of the Early and Periodic Screening, Diagnosis, and Treatment (EPSDT) Program (H. R. s7100, 1967) (637).[2] The Johnson administration's interest in enacting a child health program created a receptive audience for the ideas presented in the analysis, Difficulties in the implementation of EPSDT have arisen over the years. One of the major obstacles has proven to be the high costs that States incur when they participate in the program (213). This problem was not focused on in the original examination of societal costs and benefits.

National Institutes of Health

NIH is the Government's principal biomedical research agency. Its 18 major organizational components support research on the causes, diagnoses, and treatment of diseases. Although a few institutes have engaged in economic analyses and cost-benefit studies, such efforts have been relatively atypical. The institutes have traditionally focused almost exclusively on gathering new knowledge as it relates to the disease process, having added an emphasis on assessing the safety and efficacy of new and existing technologies only recently. In addition, NIH has a very limited capacity to conduct economic analyses.

These factors, among others, have combined to discourage NIH from conducting CEA or CBA studies, as noted in an NIH staff memorandum (481):

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[1] For a description of this effort, see R. N. Grosse and W. Smith, "Cost-Effectiveness and Cost-Benefit Analysis for Public Health Programs," *Public Health Reports, 1968* (268).

[2] For description of this analysis and the role played in the passage of EPSDT, see J. Wholey and G. Silver, *Maternal and Child Health Care Program, 1966* (638); A. Levin, "Cost Effectiveness in Maternal and Child Health: Implications for Program Planning and Evaluation," *N. Eng. J. Med., 1968* (356); A. M. Foltz, "The Development of Ambiguous Federal Policy: Early and Periodic Screening, Diagnosis and Treatment (EPSDT)" (213), *Milbank Memorial Fund Quarterly*, 1975; and R. N. Grosse, "Cost Benefit Analysis of Health Services," 1972 (266).

> The Institutes of the NIH lack the staff, funds, and in-depth expertise to undertake large-scale economic analyses. Furthermore, safety and efficacy are still the primary concerns in the areas of disease research in which NIH is involved, the state-of-the-art in many of these areas is not yet sufficiently advanced to make cost-benefit analyses feasible. However, the NIH shares with agencies responsible for health care delivery and regulation the mandate to evaluate new medical procedures and devices, and where appropriate the Institutes do conduct small-scale cost-benefit /cost-effestiveness analyses as components of more comprehensive technology assessment studies. Extensive efforts at cost-benefit analyses would be more appropriately undertaken by other federal agencies within DHEW . . . which relate more closely with the health care [delivery] sector.

Economic and cost-benefit studies that have been conducted by NIH have generally resulted from the interest of individual staff people (480). By far the most common types of economic analyses produced by NIH have been various cost-of-illness studies. These have been used in both planning and budget justifications. NIH cost-of-illness studies include:

1. National Institutes of Health, Office of the Director, "Trends of Economic Costs of Illness, " 1979.
2. National Cancer Institute, "Measurement of the Cost of Cancer Care," 1978.
3. P. A. Hoffstein, K. K. Krueger, and R. J. Wineman, "Dialysis Costs: Results of a Diverse Sample Study," Kidney International, 9:286, 1976. [This is a summary of a study conducted for the National Institute of Arthritis, Metabolism, and Digestive Diseases by the Nephrology Cost Group.]
4. National Institute of Arthritis, Metabolism, and Digestive Diseases, "The Cost and Prevalence of Complications of Dialysis in Five Dialysis Centers, " 1978.
5. E. J. Sondik, "Heart Disease, Magnitude of the Problem, " report prepared for the National Heart, Lung, and Blood Institute Task Force on Heart Disease in Childhood, July 3, 1979.
6. National Institute of Neurological and Communicative Disorders and Stroke, "Survey of the Incidence, Prevalence, and Cost of Intercranial Neoplasm, " 1979.

The last study is part of a current effort by the National Institute of Neurological and Communicative Disorders and Stroke to link research priority setting more closely to identification of areas of greatest societal need and projects with the greatest potential for investment return. Thus, it is perceived as contributing to an effort to produce more "cost-effective" research (480). Similarly, the cost-of-dialysis studies of the National Institute of Arthritis, Metabolism, and Digestive Diseases are perceived as having a cost-effectiveness element because they underline the different costs and complications of various modes of dialysis.

Each institute of NIH seeks to reduce the morbidity and mortality associated with the disease under its mandate and considers research directions that are intended to reduce the human and economic toll of the diseases. Cost considerations have occasionally been considered in NIH consensus development conferences. Although their primary focus is on "clinically relevant research and the readiness of certain important findings to be put to use in health or regulation, " these conferences may also consider social, ethical, legal, and cost implications. In general, however, they consider these implications only as necessary extensions of their scientific and technical evaluations of specific technologies. The principal responsibility for examining these "nontechnical" issues rests with the National Center for Health Care Technology (NCHCT), which is discussed in a separate section of this appendix. The consensus development conferences sponsored by NIH serve only as the forum for the issues' presentation. Consensus development conferences to date have addressed a wide range of subjects, including mammography, dental implants, and electronic fetal monitoring.

In addition, three NIH institutes have produced more explicit cost-benefit work. One, the National Heart, Lung, and Blood Institute (NHLBI) has conducted several cost-benefit studies. NHLBI's first experience with CBA was in a 1973 assessment of heart transplant surgery and artificial heart development and use. As part of its long-term program of examining mechanical circulatory technology, NHLBI issued a report on the medical, ethical, legal, financial, and social implications of artificial heart implantation (442). This assessment did not include a formal CBA, but did incorporate the concept by providing a detailed listing of the costs, as well as the medical and social benefits, of the artificial heart. The report's examination of the cost of the device probably proved less important than its discussion of the formidable engineering obstacles to development, however, since it was these obstacles that played a major role in discouraging greater investment of resources by the institute in the totally implantable artificial heart program (514).

In 1972, NHLBI's mandate was broadened to include prevention, education, and control responsibilities. Subsequently, the National High Blood Pressure Education Branch and the National High Blood Pressure Demonstration Program were created to work with Government and private agencies in an effort to increase an awareness of the dangers of hyper-

tension and to encourage the development of effective treatment. Both of these programs have utilized information produced by the cost-benefit studies to help convince State and local health agencies and private organizations of the value of instituting hypertension control programs (611,614).

In 1975, NHLBI's National High Blood Pressure Education Program conducted a CBA of a model hypertension control program in order to produce a model for analyzing the costs and benefits of a national hypertension treatment program. This study was recently updated (441) through the incorporation of new data and the development of a computer program which tests several of the major assumptions made in the 1975 report (577). NHLBI has used information from this and the earlier analysis to encourage effective treatment programs.

Further, the National Institute of Dental Research (NIDR) has produced two cost-benefit studies. NIDR's National Caries Program was established by Congress in 1971 to sponsor R&D activities directed toward ultimately reducing the incidence of dental disease in the American public. In carrying out this mission, program officials have used CBAS to assist them in their long-range planning of R&D investment decisions (83,522). In 1974, NIDR contracted for the development of a computer-based model for predicting the long-term "net social value" of the use of preventive procedures (462). The results of the study were expressed in terms of the dollar savings produced by dental care treatment on both a nationwide and regional basis. In 1978, an internal NIDR staff report assessed the costs and benefits of a specific caries prevention treatment, pit and fissure sealants (72).

Both of these NIDR cost-benefit studies contributed to a decision to halt investment in occlusal sealant clinical trials until a sealant and a system of delivery could be developed that would significantly reduce the clinical time required in sealant application (523). Examination of the "net social value" model also contributed to program decisions to increase funding of efforts aimed at reducing the high carcinogenicity of American diets and projects directed toward development of fluoride-based regimens for use in nonfluoride areas (523).

Another study employing the cost-benefit concept has been funded by the National Cancer Institute (NCI). NCI originally contracted with the Blue Cross/Blue Shield Associations to develop a model prepaid health service benefit package for a cancer-screening program (435,436). Although originally the contents of the benefit package were to be determined through the consensus of a panel of experts, the screening program that eventually was designed was largely determined by the use of cost-effectiveness methods (168).

Cost-benefit considerations are often present in the various NIH institutes' clinical trial deliberations. Although the evaluation of benefits is largely qualitative, judgments about whether to undertake a clinical trial in order to test a given therapy often broadly consider costs and benefits. Perhaps the most systematic and formal example of this process is provided by NCI'S method of selecting new drugs for clinical trials. Within NCI, a large staff committee called the "Decision Network Group" engages in a formal evaluation of costs and potential benefits. A staff member describing the process observed (542):

> 1n making the selection of specific drugs to develop toward clinical trial, we consider the relative biological activity of the drug in comparison with the cost of development, including large-scale production. Thus, if the drug represents a new type of chemical class which is highly active and relatively inexpensive, we, of course, have no difficulty in reaching a decision. If, however, the new drug is of relatively marginal benefit in the experimental systems we utilize, and, in addition, it is estimated to cost a great deal of money to produce and develop, we very seriously consider the advisability y of proceeding.
>
> Another example of this type of cost consideration is a new drug related to one previously developed. If the original drug was very expensive and the new analog is considerably cheaper and of equivalent activity, we would very likely decide to switch to the new drug.
>
> Cost has a major role in the development and design of the approaches we utilize for screening new potential antitumor drugs in experimental systems. Thus, in developing the panel of experimental systems currently utilized, we realized that it was impossible financially to carry out the testing in what might be considered the ideal scientific manner, namely, to test all materials in the complete battery of experimental systems. Since that approach was not financially feasible, we developed a pre-screening approach, so that the large number of compounds being tested would be evaluated in a relatively sensitive system first, and then further evaluated in the panel of experimental tumors based primarily on activity in that pre-screen.
>
> Another example of cost analysis involves the Iarge animal preclinical toxicology studies carried out on a drug being developed for clinical trial. Our standard protocol for studies requires about 9-10 months and costs on the order of $120,000. We experimented with one very high priority drug to determine whether these experiments could be carried out in a shorter period of time. We found, indeed, that this could be done, but unfortunately, it required approximately twice as much money to carry out those experiments, since more tests had to be done simultaneously with some wasted effort in order to save time. Thus, it was decided that we would not routinely evaluate drugs in that manner since it was so expensive, but reserve

such alternate procedures for those very rare compounds that are considered of such high priority that they must be moved as rapidly as possible regardless of the cost . . .

In summary, although we cannot carry out what one might like to see as completely quantitative cost benefit analyses, cost considerations have always been and remain a vital part of our everyday life in making scientific decisions and in attempting to develop new and better modes of therapy in the shortest amount of time with the funds available to use.

Although other NIH institutes utilize a less formal selection process than NCI, cost-benefit considerations often enter into their decisions concerning the need for clinical trials. A staff member of NHLBI, for example, cited three instances in which cost considerations played a part in such decisions[3] (577).

A number of the Institute's clinical trials were undertaken for reasons very much concerned with the potential benefits to be accrued from a careful testing of a given therapy or preventive regimen. In particular, the coronary artery surgery study (CASS) is to determine the efficacy of surgical vs. medical intervention for coronary artery disease . . . Coronary artery surgery (by-pass surgery) is an extremely expensive operation (between $10,000 and $20,000 per case) that may be able to be treated as efficaciously and at considerably less cost through non-surgical means. In arriving at the decision to undertake CASS, the Institute weighed the potential benefits of the information to be derived against the considerable cost of the study. In similar fashion, the Institute considered a trial related to mild hypertension treatment, but decided that the Institute's current portfolio of clinical trials related to hypertension would provide much the same information as a new trial. It was calculated that such **a** trial was not currently warranted, The Extracorporeal Membrane Oxygenator (ECMO) and Intermittent Positive Pressure Breathing (lPPB) trials are two additional NHLBI supported trials concerned with determining the effectiveness of costly, invalidated therapies.

Food and Drug Administration

FDA is responsible for monitoring the safety of foods and cosmetics and evaluating the safety and efficacy of drugs and medical devices, food, feed, and color additives. With the exception of several studies performed by the Bureau of Radiological Health (BRH), FDA's experience with CEA is limited to eight analyses conducted in response to regulatory initiatives.[4]

On March 23, 1978, Executive Order 12044 mandated that Government agencies examine the costs and benefits of major proposed regulations (i. e., regulations having an annual impact on the economy of "$100 million or more or [causing] a major increase in costs or prices for individual industries, levels of government or geographic regions"). This mandate was built upon an earlier executive order issued by President Ford which required an "inflation impact" analysis of major proposed regulations in order to assure that the private sector not be burdened by unjustified costs.[5]

Under these mandates, FDA has conducted economic analyses of eight proposed initiatives (218):

1. Food and Drug Administration, Office of Planning and Evaluation, Office of the Commissioner, "Inflation Impact Statement of Proposed Rulemaking: Certain Fluorocarbons and Chlorofluorocarbons in Food, Food Additives, Drug, Animal Food, Animal Drug, Cosmetic and Medical Device Products as Propellants in Self-Pressurized Containers, Prohibition on Use," May 1977.
2. Food and Drug Administration, Office of Planning and Evaluation, Office of the Commissioner, "Inflation Impact Statement of Proposed Rulemaking: Nitrofuran (S-Nitro) Compounds, " May 1976.
3. Food and Drug Administration, Office of Planning and Evaluation, Office of the Commissioner, "Inflation Impact Statement of Proposed Rulemaking: Diethylstilbestrol, " January 1976.
4. F. H. Dworkin, Economics Staff, Office of Planning and Evaluation, Food and Drug Administration, "An Analysis of Economic Impact of the Drug Regulation Reform Act of 1978, " November 1978.
5. F. H. Dworkin, Economics Staff, Office of Planning and Evaluation, Food and Drug Administration, "Chemical Compound in Food-Producing Animals: Criteria and Procedures for Evaluating Assays for Carcinogenic Residues, " March 1979.
6. Food and Drug Administration, Office of Planning and Evaluation, Office of the Commissioner, "Inflation Impact Statement of Proposed Rulemaking: Saccharin and Its Salts in Foods, Drugs, Animal Feeds, Animal Drugs, Cosmetics, and as a Food Additive, Prohibition of Uses, " April 1977.
7. P. F. Lewis, Environmental and Economic Impact Staff, Bureau of Foods, Food and Drug

'For a discussion of NHLB1 clinical trial planning and decisionmaking, see R. 1, Levy and E. J. Sondik, "Decision-Making in Planning Large-Scale Comparative Studies, " and R. 1. Levy and E. J. Sond]k, "Initiating Large-Scale Clinical Trials, " 1978.

'For other regulatory agencies' experience with these requirements, see T. B. Clark, "Its Still No Bureaucratic Revolution, But Regulatory Reform Has Foothold, " NatIona/ ***Jourtlal,*** Sept. ***29, 1979 (97).***

'This earlier executive order was Executive Order 11821, superseded by Executive Order 11949, 1977.

Administration, "Regulatory Analysis for Final Regulation for Reduction of Temporary Tolerances for Polychlorinated Biphenyls in Food, " June 27, 1977.

8. F. H. Dworkin, et al., Economics Staff, Office of Planning and Evaluation, Food and Drug Administration, "Draft Regulatory Analysis: Patient Labeling Requirements for Prescription Drug Products, " June 1979.

In most of these economic analyses, however, FDA was only able to list benefits. In general, it was not able to quantify them owing to a lack of adequate data, methodological problems, and difficulty in projecting behavior precipitated by proposed manufacturing controls (43,646). Further, when an analysis involves a carcinogenic food additive, as it did in three instances,[b] FDA is legally forbidden to weigh costs and benefits in making a decision. According to the "Delaney clause" of the Food, Drug, and Cosmetic Act of 1938,[7] if a food additive is found to be carcinogenic, FDA must ban it.[8] Nevertheless, FDA seems to have found economic analyses generally useful, and the consideration of specific benefits probably has contributed to the modification of some provisions of several proposed regulations (43).[9,10]

One of FDA's component bureaus, BRH, has used economic analyses and CBAS more extensively than other component bureaus. BRH is responsible for protecting the public from unnecessary exposure to radiation and ensuring that radiation is used safely and efficaciously. In 1977, BRH conducted an "Economic Impact Assessment of the Proposed Performance Standard for Sunlamp Products, " January 1977. Because of the limited projected impact of this regulation, BRH assessed only costs.

In April of 1977, however, BRH used the cost-benefit concept more specifically, to justify budget requests for expanding the Bureau's effort to reduce the unnecessary use of X-rays. Thus, the fiscal year 1979 Preliminary Budget Justification stated (217):

> The proposed national X-ray system will cost an additional $7.7 million contract dollars and 181 positions. This gives a benefit/cost ratio ranging from 32 to 1 to 70 to 1 for the 20-50 percent possible reductions in the genetically significant dose alone.

This cost-benefit argument was the "driving force" (341) behind the development of a cost containment plan: "A Proposed FDA Program To Reduce Unnecessary Patient Exposure From Diagnostic X-Rays: Cost Containment Considerations" August 1978. In this plan, CBA was used to demonstrate the cost savings produced by reducing unnecessary X-rays.

Another CBA performed by BRH was "The Diagnostic X-Ray Equipment Performance Standard and the Policy on Assembly and Reassembly, " November 1978. This study represented a refinement and elaboration of an earlier attempt (216) to examine the costs and benefits of the X-ray equipment standard in an environmental assessment report in 1974. The results of this updated study contributed to FDA's decision to amend its policy on the assembly and reassembly of diagnostic X-ray equipment and revoke two regulatory provisions which were shown not to be cost beneficial (215,340).

Center for Disease Control

CDC is responsible for monitoring, controlling, and reducing the incidence of preventable diseases and conditions. Over the years, CDC has conducted a number of assessments of the costs of illnesses and treatment methods, as well as cost-benefit studies. In 1966, CDC involvement in the HEW program analyses of the costs and benefits of tuberculosis and syphilis control promoted staff interest in examining the economic impact of disease.[11] Since then, CDC has often used economic analysis to supplement the Center's traditional public health perspective by allowing the costs of a disease to be considered along with morbidity and mortality statistics. Since much of CDC'S work involves providing information to State and local public health officials who must be concerned with budgets, economic analysis has proved particularly helpful in supporting the value of suggested disease prevention control programs (32, 601). A CDC official noted:

> [These analyses] enable the cost of disease control efforts and investigations to be placed in perspective. For vector-borne diseases (dengue, equine encephalitis), such analyses make it easier to justify costly spraying and clean-up efforts.

The economic impact studies conducted by CDC staff include:

1. D. J. Sencer and N. W. Axnick, "Utilization of Cost Benefit Analysis in Planning Prevention Programs, " Acta Medica Scandinavia, Supplement 576, 1975. [This study reports on the

[b]The inflation impact statements for nitrofuran, diethylstilbestrol, and saccharin.

[7]See Office of Technology Assessment, U.S. Congress, *Cancer Testing Technology and Saccharin*, October 1977 (466).

[8]This legislation takes precedence over Executive Order 12044.

[9]The cost of releasing safety and efficacy data on new drugs, revealed by the analysis of the 1978 Drug Regulatory Reform Act, for example, contributed to the dropping of the draft requirement that such information be released.

[10]Another case in which economic analysis contributed to a modification of policy is FDA's action on antibiotics and feed: "Combined Economic Impact Assessment of Series of Proposals Regarding Penicillin/Tetracycline-Containing Premixes," undated.

[11]See the section in this appendix on the Office of the Assistant Secretary for Planning and Evaluation, DHHS. Also, for a discussion of CDC's analyses of syphilis and tuberculosis control, see R. N. Grosse, "Cost-Benefit Analysis of Health Services," *Ann. Am. Ac.*, 1972 (266).

costs of hepatitis, hospital infections, and the 1964-65 rubella epidemic.]

2, D. D. Tolsma and J. A. Bryan, "The Economic Impact of Viral Hepatitis in the United States, " Public Health Reports 91:349, 1979.
3. S. D. Von Allmen, R. H. Lopez-Correa, J. P. Woodall, D. M. Morens, J. Chiniboga, and A. Casta-Valez, "Epidemic Dengue Fever in Puerto Rico, 1977: A Cost Analysis, " American Journal of Tropical Medicine, November 1979.
4. E. L. Baker, Jr., W. Peterson, S. D. Von Allmen, and J. Fleming, "Economic Impact of a Community-Wide Waterborne Outbreak of Gastrointestinal Illness. "
5. M. L. Cohen, R. E. Fontaine, R. A. Hollard, S. D. Von Allmen, T. M. Vernon, and E. J. Gangarosa, "An Assessment of Patient-Related Costs in an Outbreak of Salmonellosis. "
6. D. D. Tolsma and J. D. Millar, "Costs and Accomplishments of the United States National Influenza Immunization Program, 1976, " Eighth International Scientific Meeting of the International Epidemiologic Association Symposium on Epidemiologic Aspects of Cost-Effectiveness Studies, San Juan, Puerto Rico, Sept. 19, 1977.
7. R. C. Rendtorff, J. C. Curran, R, W. Chandler, W. L. Wiser, and H. Robinson, "Economic Consequences of Gonorrhea in Women: Experiences From an Urban Hospital, "]ournal of the American Venereal Disease Association, I(I), September 1974.
8. S. D. Von Allmen, W. Cates, Jr., K. F. Schulz, D. A. Grimes, and C. W. Tyler, Jr., "Cost of Treating Abortion-Related Complications, " Family Planning Perspectives, 9(6), November/December 1977.
9. J. L. Bradley, "The Cost of Diphtheria Epidemic in San Antonio, Texas, 1970-71, " paper presented at the American Public Health Association Meeting, San Francisco, Nov. 7, 1973.
10. P. M. Schwab, "Economic Cost of St. Louis Encephalitis Epidemic in Dallas, Texas, 1966, " Public Health Reports, 83(10):860, October 1968.
11. B. S. Levy, "The Economic Impact of a Food-Borne Salmonellosis Study, "]ournal of American Medical Association, 230:1281, Dec. 2, 1974.
12. N. W. Axnick, "Cost of 1968-69 Influenza Epidemic," paper presented to the Surgeon General's Advisory Committee on Immunization Practice, April 1970.
13. S. D. Von Allmen, R. H. Lopez-Correa, J. P. Woodall, D. M. Morens, J. Chiniboge, and A. Casta-Valez, "Epidemic Dengue Fever in Puerto Rico, 1977: A Cost Analysis, " American Journal of Tropical Medicine, November 1979.
14. E. L. Baker, Jr., W. Peterson, S. D. Von Allmen, and J. Fleming, "Economic Impact of **a** Community-Wide Waterborne Outbreak of Gastrointestinal Illness."
15. M. L. Cohen, R. E. Fontaine, R. A. Pullard, S. D. Von Allmen, T. M. Vernon, and E. J. Gangarosa, "An Assessment of Patient-Related Costs in an Outbreak of Salmonellosis. "
16. N. W. Axnick and J. M. Lane, "Costs Associated With the Protection of the United States Against Smallpox," World Health Organization Report, March 1973.
17. P. M. Layde, S. D. Von Allmen, and G. P. Oakley, "Antenatal Screening for Downs Syndrome: An Economic Viewpoint, " September 1979.
18. J. J. Witte and N. W. Axnick, "The Benefits From 10 Years of Measles Immunization in the United States, " Public Health Reports, 90:3, May-June 1975.

In recent years, CDC also has conducted a number of cost-benefit studies. Several CDC officials noted that CBA contributes an additional dimension to more traditional evaluations of morbidity and mortality, and where adequate data exist, it has become logical to consider this information an evaluation. In some cases, CBA seems to provide a particularly meaningful perspective, because humanitarian concerns can be weighed against dollar costs and the value of prevention can be made more tangible by describing benefits in terms of averted costs. As a consequence, CBA has been used by CDC staff to plan and evaluate prevention and control programs and justify investment in such efforts (32,325).

Cost-benefit assessments conducted for the CDC staff include:

1. P. M. Layde, S. D. Von Allmen, and G. P. Oakley, "Maternal Serum Alpha-Fetoprotein Screening: A Cost Benefit Analysis, " *American Journal* of Public Health, 69(6):566, June 1979.
2. P. M. Layde, S. D. Von Allmen, and G. P. Oakley, "Congenital Hypothyroidism Control Programs: A Cost-Benefit Analysis, " Journal of the American Medical Association, 24(21): 2290, May 1979.
3. P. V. Strange and A. T. Sumner, "Predicting Treatment Costs and Life Expectancy for End-Stage Renal Disease, " N. Eng. 1. Med., 298: 372, Feb. 16, 1978.
4. D. J. Sencer and N. W. Axnick, "Cost Benefit Analyses, " Proceedings of the 145th Syrnpo-

sium on Vaccination Against Communicable Diseases, Monaco, March 1973.
5. J. P. Koplan, S. Schoenbaum, M. Weinstein, and D. Fraser, "Pertussis Vaccine: An Analysis of Benefits, Risks, and Costs," N. Eng. J. Med., Oct. 25, 1979.
6. J. K. Paperfuss and B. C. Ejeldsled, "Cost-Benefit Study of Selected Interventions in Control and Prevention of Tuberculosis in the State of Michigan," study prepared by the Michigan Division of Health for CDC, December 1969.

Alcohol, Drug Abuse, and Mental Health Administration

ADAMHA has major responsibility for the prevention and treatment of mental illness, alcohol abuse, and drug abuse. One of ADAMHA's three component bureaus, the National Institute on Drug Abuse (NIDA), has produced several cost-of-illness and cost-benefit studies. They include:

1. Arthur D. Little, "Social Cost of Drug Abuse," 1976.
2. Macro Systems, Inc., "Unit and Episodic Costs of Drug Abuse Treatment: A Feasibility Study," May 1978.
3. Center for Social Research on Drug Abuse, "The Assessment of Social Cost Savings," March 1978.
4. Research Triangle Institute, "Management Ef - festiveness Measures for NIDA Drug Abuse Treatment Programs: Costs to Society," vol. 11, 1977.

These studies have been used in both planning and budget justification. The cost information yielded by the analyses has provided basic data for CEA / CBA of drug abuse treatment.

In 1972, the U.S. Bureau of Narcotics and Dangerous Drugs conducted a study of the costs and benefits of alternative approaches to addiction control (386). A study funded by NIDA in 1975 extended this idea by developing four primary measures of the effectiveness of drug treatment and comparing the cost-effectiveness of five treatment modalities: methadone maintenance, therapeutic community, inpatient detoxification, outpatient detoxification, and drug-free treatment (535a).

More recent studies have focused on the development of a more sophisticated cost-effectiveness and cost-benefit ratios for drug abuse treatment programs (504) and examination of the applicability of CEA to primary drug abuse prevention programs (298,505). These recent NIDA analyses are acknowledged to have serious methodological limitations because of inadequate data about such factors as the total number of drug abusers, the relapse rate of treated abusers, the relationship between drug abuse and unemployment and crime, and the relationship between patient outcome and treatment modality (535).

Nevertheless, the cost-benefit / cost-effectiveness studies are credited with providing valuable information for program evaluation and management and NIDA budget justification (360,520,534,535a). The studies have also confirmed the worth of allocating more funds to the less traditional and expensive treatment approaches (i. e., of a funding formula which favors outpatient methadone maintenance, outpatient drug detoxification, and outpatient drug-free programs rather than inpatient detoxification and residential community approaches). In addition, they have been useful in confirming the benefits produced by the Federal investment in drug abuse treatment. As an official of NIDA observed (535a):

> . . . Based on an empirical analysis of data, we have established average gains per patients year on performance criteria and can translate these gains into cost savings benefits to society. In turn, we can compare them to what society has invested in treatment. The conclusion is that it pays to invest in treatment.

Unfortunately, such analyses are still severely limited by their inability to specify which treatment modality-tends to work-best for a_given type of client. In order to better address this question, NIDA is currently attempting to collect more accurate data on drug abuse and rehabilitation patterns through the Drug Abuse Reporting Program (DARP) and the Treatment Outcome Prospective Study (TOPS).

At the National Institute of Mental Health (NIMH), there are two potential applications of CEA / CBA studies: 1) in measurement of service program outcomes, such as the community mental health centers (CMHCS) that receive Federal sponsorship, and 2) the measurement or evaluation of psychosocial interventions. It is important to keep these two quite different research levels separated. Depending on the research focus—program outcomes or psychosocial treatment outcomes—there will be differences in what is measured, the means of measurement, the purpose of measurement, the uses of the information, and the types of people involved in the research.

The Psychotherapy and Behavioral Intervention Section of NIMH's Division of Extramural Research Programs is responsible for funding research on the effectiveness, efficacy, safety, etc., of psychosocial treatment mechanisms for specific mental disorders. Although one or two of the NIMH-sponsored psychotherapy outcome studies have examined variables related to cost benefit or cost effectiveness (e. g., duration of treatment required), most such studies are not intended or designed to address cost variables.

Traditionally, psychotherapy research seeks only to elucidate the mechanisms of action and the efficacy of specific treatments for specific diagnostic categories of mental disorders. The Psychotherapy and Behavioral Intervention Section is currently attempting to encourage the use of a range of standard procedures for the measurement of change in psychotherapy in the hope of achieving greater comparability in the assessment of outcomes across various studies. Cost-effectiveness variables, though, are even more difficult to apprehend and often are not useful at this level of research.

The Division of Biometry and Epidemiology at NIMH has conducted several studies of the services programs and episodes-of-illness costs of patients treated in federally sponsored CMHCS. Costs and cost-effectiveness criteria appear to be potentially relevant for use in evaluating program costs and examining the outcomes of delivering mental health services at the community level. NIMH has funded studies under contract which seek to advance the methodological framework of CBAS as applied to CMHCS' programs (443). NIMH (or the Division) does not conduct such studies itself because of the limited development of the methods, differences in CMHCS' clientele, and the tendency for such assessments to be subject to misrepresentation and to be used to criticize the management of individual CMHCs.[12]

NIMH has supported training courses in CBA through its staff ..college, and through the contract mechanism, is supporting several State governments in the development of management systems that will provide unit cost data for mental health service systems. A few States (Colorado, Oregon, 13 and Washington) have officials who are working with NIMH to develop a more sophisticated methodology for examining CMHC program costs and outcomes.

National Center for Health Care Technology

(See ch. 9 for a discussion of NCHCT.)

National Center for Health Services Research

NCHSR funds a number of CEA and CBA studies as part of its services research function (439). It is currently the major supporter of CEAS and CBAS in the Federal health care research system. Two of NCHSR'S research priority areas—health care costs and cost containment, and planning and regulation—specifically call for research using CEA studies to examine the issues in these priority areas (438).

The types of economic analyses NCHSR supports range from what could be considered traditional CEAS or CBAS to analyses that focus on either costs or outcomes of a given technology or program. It is difficult to characterize the range of economic studies NCHSR supports. The studies range from rigorous examinations of specific health care topics to broader studies of more complex health care issues. CEA or CBA may be the focus of a grant or may be incorporated into larger, more global **assessments.** Nevertheless, NCHSR is a significant source of support for these types of techniques. Some of the more recent CEA and CBAS that have received support from NCHSR are listed below (439):

1. "Impact of Ophthalmic Technicians on Outpatient Care," HS 03647.
2. "Improving Clinical Decision Making in Arthritis," HS 03115.
3. "Prediction of Cost Effective Stroke Rehabilitation, " HS 03693.
4. "Controlled Trial of a Quality Assurance Mechanism," HS 02485.
5. "Evaluation of Breast Cancer Detection Strategies," HS 03256.
6. "Cost Effective Strategies in Ambulatory Care, " HS 02063.
7. "A Guide to Investment Criteria for Critical Care Units, " HS 03569.

National Center for Health Statistics

NCHS is an agency within the Office of the Assistant Secretary for Health of DHHS. It is one of the principal health services research agencies of the Federal Government. Health statistics activities, which eventually were formalized and combined by the creation of NCHS, were authorized by Congress in 1946 (vital statistics) and in 1956 (National Health Survey). NCHS was formed in 1960 and 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 dis-

seminating health data including information on the costs of illness, health care, and health financing.

The importance of data collected by NCHS cannot be overemphasized, Information such as that concerning the incidence and prevalence rates of diseases, natural history of disease, medical care utilization, workloss, surgical rates, and premature mortality is crucial to CEA/CBA. Although NCHS does not conduct full cost-effectiveness studies, it has conducted cost-of-illness studies, and these have been used in other agencies' CEA/CBAs. Currently, NCHS staff are coordinating an interagency Public Health Service (PHS) Cost of Illness Committee which is examining the state-of-the-art of estimating costs of illness and disability. As noted by an NCHS official, it has often proved impossible to compare the results of cost-of-illness studies conducted by NCHS and other DHHS agencies (297):

> Frequently . . . the assumptions, methods and data employed vary to such an extent that estimates from two different studies differ markedly, and the costs of several illnesses cannot be compared.

In an attempt to address this problem, the PHS Cost of Illness Committee will recommend a set of guidelines for future PHS studies.

Health Resources Administration

HRA is responsible for improving the national capacity to develop and effectively use health resources. Its Office of Program Planning and Evaluation has sponsored conferences which produced two studies focusing on cost-effectiveness methodology:

1. S. O. Schweitzer, "The Economics of the Early Diagnosis of Disease." [This article presents a methodological framework for the evaluation of the cost-effectiveness of early diagnostic tests.]
2. H. A. Foley, D. B. Fisherman, and F. Kalibat, "The Cost-Effectiveness of a Mental Health Center: An Experiment, " in Evaluation in Health Services Delivery, edited by R. Yaffe and D. Zalkind, proceedings of an Engineering Foundation Conference, South Berwick, Maine, Aug. 19-24, 1973.

The Bureau of Health Manpower (BHM), one of HRA's component agencies, has also produced one cost-effectiveness study:

1. N. Doherty, "Study to Determine the Cost and Effectiveness of Different Practice Modes of Dental Care for Children, " February 1977.

Another HRA agency, the Bureau of Health Planning (BHP), has sponsored two cost-benefit studies:

1. Arthur D. Little, "Introduction to Cost-Benefit Analysis Applied to New Health Technologies, " December 1977.
2. Stanford Research Institute, "Decision Making Methodology for Health Care Services, " study prepared for the Region IX (San Francisco) Office of DHEW, December 1979.

Title V of the National Health Planning and Resources Development Act of 1974 (Public Law 93-641) suggests that local health planning agencies (health systems agencies (HSAS)) should consider the costs and benefits of projects (sec. 1514(3)):

> In establishing the Annual Implementation Plan, the agency shall give priority to those objectives which will maximally improve the health of the residents of the area, as determined on the basis of the relation of the cost of attainingsuch objectives to their benefits, and which are fitted to the special needs of the area.

But most HSAS do not appear to use CBA. '4 The two guidebooks to cost-benefit methodology which BHP has funded constitute only a small segment of the technical assistance literature which is being distributed to HSAS.

Health Services Administration

The Health Services Administration is responsible for Federal programs that provide health care services to specific populations. It has sponsored a number of studies that deal with cost-benefit considerations:

1. Abt Associates, Inc., "Analysis of Benefits and Costs of Ambulatory Care Services Offered by the Bureau of Community Health Services, " March 1975.
2. Geomet, Inc., "Comparison of Cost-Per-Day in PHS Hospitals: An Approach to Service and Cost Comparisons, " September 1975.
3. Health Services Administration/OPEL and Geomet, Inc. (A. Dobson and H. L. Heaton), "An Economic Analysis of HSA Program Potential, 1976-1981," report No. OPEL 75-4, May 1975.
4. Health Services Administration/OPEL and Geomet, Inc. (I. Enger and N. Pindus), "Cost and Utilization Evaluation Migrant Hospital Program, " report No. OPEL 77-I, January 1977.
5. Health Services Administration/OPEL and Geomet, Inc., "Improved Pregnancy Outcome Program Evaluation Manual, " report No. OPEL 77-2, March 1977.
6. Health Services Administration/OPEL, "PSRO: An Evaluation of the Professional Standards Review Organization Program," vol. 1-11, report No. OPEL 77-12, October 1977.

[14] For further discussion of this subject, see ch. 6 of this report

7. Transaction Systems, Inc., "CredentialIing of Health Care Professionals: Its Costs and Some Alternatives for the Health Services Administration, " March 1975.
8. General Research Corporation, "PSRO Program Cost Study, " April 1974.
9. Geomet, Inc., "Cost and Utilization Evaluation of Migrant Ambulatory Care Entitlement Programs, " March 1979.
10. Geomet, Inc., "Comparative Cost and Financial Analysis of Ambulatory Care Providers, " June 1976.
11. Health Services Administration, "Migrant Ambulatory Care Entitlement Programs ~ Cost and Utilization Evaluation of Rural Health Initiative," October 1978.

Health Care Financing Administration

HCFA is responsible for administering the medicare, medicaid, Professional Standards Review Organization (PSRO), and End-Stage Renal Disease Programs. It has conducted several studies of the economic toll of illness and the cost of health care financing. These include:

1. B: S. Cooper and D. P. Rice, "The Economic Costs of Illness Revisited," Social Security Bulletin, 39(2), February 1976.
2. D. P. Rice, "Estimating the Cost of Illness, " Health Economics Series No. 6, Public Health Service, 1966.
3. R. M. Gibson and C. R. Fisher, "National Health Expenditures, Fiscal Year 1977, " Social *Security Bulletin,* 41(7):3, 1978.

In addition, HCFA is currently sponsoring an effort aimed at encouraging the integration of cost containment information into medical school curricula:

1. American Association of Medical Schools, "A Primer on Quality Assurance and Cost Containment for Faculty and Students. "

Also, HCFA has sponsored a study which indirectly employed the cost-effectiveness concept:

1. Massachusetts Department of Public Welfare, "Cost Effectiveness Under Medicaid," 1978.

This study was designed to investigate whether medical criteria could be utilized within a medicaid program to reduce inappropriate and ineffective medical care. Problems in implementation, however, led to HCFA'S termination of funding for the study.

In addition, HCFA has conducted an assessment of the costs and the benefits of PSROS. Under Title XI of the Social Security Act, PSROS are responsible for reviewing the medical necessity, quality, and appropriateness of federally financed health programs. In order to perform this function, PSROS must develop norms, criteria, and standards for the appropriate utilization and acceptable quality of health care services. A 1979 report by HCFA, elaborating on a previous evaluation (288), concluded that the savings produced by PSROS through reduced use of hospital services by medicare patients exceed the cost of PSRO review (285). Subsequently, this finding was used in PSRO program justification before Congress.

Two other Government evaluations of PSROS, however, have challenged HCFA'S findings. One, a study by the Congressional Budget Office found that PSROS only slightly reduce medicare patients' use of hospital services and concluded that the "PSRO program probably yields a net loss" (121). Similarly, a 1979 General Accounting Office study stated that the data HEW reviewed in its 1977 and 1978 evaluations "are not based on appropriate hospital statistics" and that several estimates of cost savings attributable to PSROS were overstated (245).

Office of Human Development Services

OHDS of DHHS administers a wide range of programs which are designed to aid children, youth, families, the aged, the handicapped, and native Americans. Over the last few years, since the first application of cost-benefit framework to vocational rehabilitation by Ronald Conley in 1965 (124), OHDS has conducted a number of CBAS of rehabilitation services. OHDS, as well as many State vocational rehabilitation agencies, has used this type of analysis to justify increased Government spending on training programs for the disabled.

The application of CBA to vocational training appears to have been facilitated by the existence of both a traditional set of program goals (e. g., gainful employment of the disabled) and a long-term information system about such factors as increased earnings and reduced costs of special medical or custodial care (479). Among the many federally and State-sponsored studies of this type are the following:

Federally Sponsored Studies

1. Rehab Group, Inc., "Feasibility Study for the Evaluation of Methodologies for Cost Benefit Analysis of Restoration Services in Rehabilitation, " prepared for OHDS, October 1978.
2. R. O. Washington, D. Y. Rowland, C. Hairston, "Quantification of Human Services Outcomes: A Manual for Applying Program Budgeting, Systems Analysis and Cost-Benefit Analyses to Human Service Programs, " prepared for Social and Rehabilitation Services, August 1974.

3. L. Mars, "An Exploratory Cost-Benefit Analysis of Vocational Rehabilitation, " prepared for the Vocational Rehabilitation Administration, 1967.
4. D. E, Carter and T. J. George, "A Manual for Reporting and Analyzing the Costs and Benefits of Social Services, " prepared for Social and Rehabilitation Services, November 1973.
5. H. Emlet, et al., "Estimated Health Benefits and Cost of Post-Onset Care for Stroke, " prepared by Analytic Services, Inc., in cooperation with Johns Hopkins University, for Social and Rehabilitation Services, September 1973. [This is an assessment of the cost and benefits of poststroke care in the population of three States.]
6. D. R. Matlack, "Cost Effectiveness of Spinal Cord Injury Center Treatment, " prepared by the National Paraplegia Foundation for OHDS, 1974. (This report provides a CEA of two alternatives spinal cord injury treatment methods.]
7. Stanford Research Institute, "Feasibility and Cost Effectiveness of Alternate Long-Term Care Settings, " prepared for Social and Rehabilitation Services, May 1978. [This is a pilot study which is designed to identify methods that could be used to determine the relative cost-effectiveness of various alternate types of long-term care settings.]

State-Sponsored Studies

1. Michigan Department of Education, Division of Vocational Rehabilitation, "A Benefit-Cost Analysis of Vocational Rehabilitation Programs in the State of Michigan, " 1970.
2. R. D. Struthers, "A Benefit/Cost Model Developed in a State Vocational Rehabilitation Agency: Michigan Vocational Rehabilitation, " Journal of Rehabilitation Administration, July 1977.
3. E. Wright, "A Benefit-Cost Analysis of Vocational Rehabilitation Programs in the State of Michigan, " prepared for the Michigan Rehabilitation Service, Lansing, Mich., 1970.
4. C. M. Grigg, A, G. Holtman, and P. F. Martin, "Vocational Rehabilitation for Disabled Public Assistance CIients: An Evaluation of Fourteen Research and Demonstration Projects, " prepared for the Institute for Social Research, Florida State University, Tallahassee, Fla., 1969,
5. J. E. Muthard, et al., "The Vocational Rehabilitation of Public Assistance Recipients: A National Survey, " prepared for the Rehabilitation Research Institute, University of Florida, Gainesville, Fla., 1976.
6. K. W. Reagles and G. N. Wright, "A Benefit-Cost Analysis of the Wood County Project: An Illustrated Lecture, " prepared for the University of Wisconsin Regional Rehabilitation Institute, 1971.
7. M. Berkowitz, "Benefit Cost Analyses: An Application to a Vocational Rehabilitation Project: An Interim Report, " prepared for the Bureau of Research, Rutgers University, New Brunswick, N. J., May 1976.
8. C. Cole and R. Dodson, "An Introduction to Cost Benefit Analysis of the Vocational Rehabilitation Program: A Model for Use by State Agencies, " prepared for the Institute of Urban and Regional Development, University of California, Berkeley, Calif., October 1972.
9. F. C. Collignon, "A Working Outline for Cost-Benefit Analyses of Vocational Rehabilitation Programs, " prepared for the Institute of Urban and Regional Development, University of California, Berkeley, Calif,, November 1971.
10. D. W. Dunlop, "Benefit/Cost Analysis: An Analytical Framework for Vocational Rehabilitation, " prepared for the Michigan Vocational Rehabilitation Service, Lansing, Mich., 1969.

It is generally conceded that these studies possess a number of serious limitations. Several of the analyses, particularly those conducted by State vocational rehabilitation agencies, suffer from poor methodological design and inadequate data (52). In addition, cost estimates are often imprecise because of the difficulty of measuring rehabilitation costs, and benefit assessments tend to represent general estimates because a number of benefits are psychic or intangible and do not lend themselves to quantification (123).

The 1973 amendments (Public Law 93-112) to the 1965 vocational rehabilitation legislation (Public Law 89-333) mandated that Federal officials broaden their efforts and work towards achieving the goal of independent living for the severely disabled. Measurement of the costs and benefits of services to the severely handicapped is very difficult, though, because of the problem of expressing in economic terms the worth of independent living, Further, many policymakers emphasize the importance of considering together with the costs of rehabilitation programs their humanitarian goals, and they argue that a traditional cost-benefit framework is inappropriate for this purpose (123,460,599).

Currently, OHDS is trying to deal with some of these problems and make CBAS more useful for its decisionmaking by sponsoring the development of a more sophisticated cost-benefit model. At the same time, however, several officials emphasized the danger of using CBA without understanding its limitations (479). Many seem to agree with John Noble's

observation made in 1977 that "the state-of-the-art needs substantial upgrading before CBAS can be taken seriously as a guide to priority-setting in the field of rehabilitation" (461).

Veterans Administration

VA operates the largest centrally directed hospital and clinic system in the United States. VA also is extensively involved in medical and health services research. Apart from one current study of the cost-effectiveness of hospice care, however, VA has not been involved in conducting cost-effectiveness or cost-benefit studies. Health systems research officials intend to study the results of the hospice study, "An Evaluation of the Wadsworth Palliation Treatment Programs," which is to be completed in 1983, in order to assess the feasibility of using CEA (184).

Officials within VA are considering intensifying VA's efforts in the evaluation of health care technology and are therefore interested in exploring the cost-benefit methodology. VA's new health services R&D director, Dr. Richard J. Green, has expressed a special interest in the use of evaluation techniques such as CEA in the examination of health care issues. A few of the areas in which VA hopes to employ CEA techniques in the future are rehabilitation medicine, alternative models of care, extended care programs, and contracted services. An area that VA hopes to focus more attention on in the future is preventive care and preventive care packages for veterans. It is uncertain at this point whether CEA will p!ay a role in the evaluation and planning in this area, but it seems clear that there is great interest in its use.

State and Local Governments and Nongovernmental Organizations

Although a few State and local governments and nongovernmental organizations have had experience with cost-effectiveness and cost-benefit studies, they appear to use such analyses only rarely. This is not surprising for at least two reasons, First, these groups traditionally devote far less funds and staff to evaluation than the Federal Government does. And second, State, local, or regional CEA/CBAs tend to be expensive because the necessary data are generally difficult to obtain. Where State and local CEA/CBAs have been conducted, their performance has usually reflected individual staff interest in CEA/CBA techniques. Perhaps, the one major exception to this generalization lies in the area of rehabilitation, where many State and local governments have followed the Federal Government's lead in using cost-benefit studies to justify investment in vocational training. [1]

As the following lists of State **and local studies show, apart from vocational rehabilitation studies, most State and local CBAS have been conducted** in Massachusetts and New York (19).

State Government Studies

1. Massachusetts Department of Public Health, "Cost-Benefit Analysis of New Born Screening for Metabolic Disorders, N. Eng.], Med., 291:1414, 1974.
2. Massachusetts Department of Public Health (M. E. Farber and S. N. Finkelstein), "A Cost Benefit Analysis of A Mandatory Premarital Rubella-Antibody Screening Program, " N. *Eng.].* Med., Apr. 12, 1979.
3. D. S. Shepard, and M. Thompson, "The Economics of Prevention: The Method of Cost-Effectiveness Analysis, " prepared for the Office of State Health Planning, Boston, Mass., July 1977, revised and published as "First Principles of Cost-Effectiveness Analysis in Health," Public Health *Reports,* November/December 1979.
4. A. Rogers and P. M, Bloomburgh, "Cost-Effectiveness Under Medicaid, " Dec. 31, 1979, prepared by staff of the Massachusetts Department of Public Welfare for the Health Care Financing Administration. [HCFA terminated this project before its scheduled completion.]
5. E. L. Hannany and J. K. Graham, "A Cost-Benefit Study of a Hypertension and Screening and Treatment Program at the Work Setting, " Znquiry, 4(4):345, December 1978, prepared for the New York State Department of Health, Bureau of Disease Control.
6. J. K. PaPerfuss and B. C. Ejeldshed, "Cost-Benefit Study if Selected Interventions in Control and Prevention of Tuberculosis in the State of Michigan, " prepared by the Michigan Division of Health for the Center for Disease Control, December 1969.

Local Government Studies

1. A. Leslie, "A Benefit/Cost Analysis of New York City Heroin Addiction Problems and Programs, 1971," in *Analysis of Urban Health Problems,* edited by I. Levinson and J. Weiss, New York: Spectrum Publications, Inc., 1976.
2. M. L. Ingbar, "Data System To Evaluate Cost-Effectiveness of Ambulatory Health Services to the

[1] "See the section of this appendix on the Office of Human Development Services (p. 154) for a discussion of these State and local studies,

Poor in Cambridge, Massachusettsr " 1971, prepared by staff of the Department of Health, Hospitals, and Welfare, Cambridge, Mass.

Nongovernmental Studies

Among nongovernmental agencies, cost-effectiveness and cost-benefit studies also have been infrequent. Further, as the following list shows, those studies that have been conducted have often been funded by the Federal Government.

1. D. R. Matlack, "Cost-Effectiveness of Spinal Cord Injury Center Treatment," prepared by the National Paraplegia Foundation for the Office of Human Development Services, HEW, 1974.
2. A. Zuvekas, "Cost-Effectiveness of Community Health Centers, " prepared by the National Association of Community Health Centers for the National Center for Health Services Research, 1979. [This study provides a cost-effectiveness methodology for evaluation of CHCS.]
3. M. M. Kristein and S. Jonas, "A Cost-Effectiveness Manual for HSA Planning. Prepared by the American Health Foundation for the National Center for Health Services Research, 1980. [This study will provide an HSA cost-effectiveness manual with particular emphasis on health promotion areas.]
4. M. M. Kristein and C.B. Arnold, "Mammographic Screening for Breast Cancer: The Economic Analysis, " paper presented at the American Public Health Association, Oct. 17, 1978.
5. M. M. Kristein, "Cost-Effectiveness of Various Smoking Cessation Methods," prepared by the American Health Foundation, 1978.
6. M. M. Kristein, "The Economics of Secondary Prevention: Screening for Disease: An Example From Colo-Rectal Cancer, " prepared by the American Health Foundation for the National Cancer Institute, 1978.

Appendix C.—Survey of the Resource Costs of CENCBA

Introduction

Formal CEA and CBA are applied to a wide range of topics in a number of diverse areas. A CEA or CBA can be performed by a single analyst or by a dozen or more. An analysis can take a few months to complete or may require more than a year. The size of the problem, the availability and quality of data, the complexity of the issues involved, and the presence of the right mix of professionals combine to exert considerable influence on the cost, the quality, the usefulness, and the credibility of the analysis.

A major focus of The Implications of Cost-Effectiveness Analysis of Medical Technology project was on the feasibility and implications of using CEAS and/or CBAS in the health care decisionmaking process. An important component of that focus are the direct resource costs of performing these types of analyses. The cost of producing a CEA or CBA will significantly influence the use of the methodologies. What are the resource needs, the problems likely to be encountered, and the time needed to produce quality analyses? What variables influence the resources used or needed to perform various CEAS or CBAS in the health care system? The feasibility and potential impacts of using CEA or CBA in the health care system are directly tied to such questions. To identify and discuss the range of answers, OTA conducted a survey of the resource costs of CEA/CBA.

Method

The survey instrument OTA used was a questionnaire designed to explore the types and amounts of resources required to perform a CEA and/or CBA of various health care technologies. (See the addendum to this appendix for a copy of the questionnaire.) Two types of information were desired: first, the resource costs of performing actual CEAS or CBAS that have been published in the literature; and second, the resources the sample group, comprised of the individuals who performed those analyses, felt would be required to staff a hypothetical research team responsible for conducting CEAS or CBAS on medical technologies on a regular and continuing basis. In essence, the survey sought a listing of resources that had been used for actual studies and estimates of resources needed to perform CEAS and CBAS on a routine basis.

The sample population was chosen on the basis of an analysis of the health care literature and discussions with analysts in the health care area. Thirty-five studies were selected by this process; no attempt was made to randomize the selection process. The survey was sent to analysts identified as having done "quality" work or whose studies were cited frequently in the health care literature. Not necessarily all quality studies were identified and selected by this process.

Twenty-two responses were returned. Eighteen respondents answered the questions pertaining to the resources actually used in performing the published analyses. Twenty-two respondents answered the questions related to the resource needs of the hypothetical CEA/CBA team described in the survey.

Results

Resources Used in Published CEAS and CBAS

The number of professional-level people directly involved in a single analysis ranged from 1 to 10 per study team, with the mode at 3 and the mean at 3.7. There was insufficient information to determine the degree of effort, or percentage of time, that the various professionals devoted to the studies. The responses were so varied and wide ranging that it was difficult to characterize the amount of time that, say, a physician or economist spent on a given analysis. The amount of full-time effort devoted to the studies ranged from as little as a single day to as much as a full year.

One trend that did emerge from the survey was the use of physicians on the study teams. Only one study did not have a physician directly involved in the analysis; the remaining studies had at least one physician, and several had two or more physicians, as part of their research group. The professions that were used in performing actual CEAS or CBAS on health care topics are summarized in table C-1.

The survey also attempted to identify the information used by the analysts. Their responses are summarized in table C-2. The types of data that the various analysts used tended to be study-specific. Responses included data on investment and operating costs, health education program costs, drug costs, screening costs, travel time to hospital, ambulance response time, physician fees, and on a host of other cost and benefit variables that cut across the four major categories listed in table C-2.

The final topic that the respondents were asked to address related to the data sources they used and the problems they had in obtaining their information. Their responses are summarized in table C-3.

Table C-1 .—Professions Used in Performing Health. Related CEA/CBAs

Personnel used	Frequency of response
Medicine	17
Economics	7
Epidemiology	6
Hospital administration	5
Systems analysis	5
Public health	4
Statistics	4
Operations research	4
Computer analysis	3
Computer programing	2
Public administration	2
Business administration	2
Medical student	2
Accountant	1
Actuary	1
Psychology	1

SOURCE. Office of Technology Assessment

Table C-2.—Types of Data Used in Health. Related CEAICBAS

Data used	Frequency of response
Morbidity	17
Mortality	16
Epidemiology	13
Health services utilization	8

SOURCE Office of Technology Assessment

Table C"3.—Availability and Sources of Data for Health. Related CEA/CBAs

Data characteristics	Frequency of response
Availability of data	
Readily available	11
Difficult to obtain	15
Almost impossible to obtain	6
Had to purchase data	0
Did original research	8
Collected from existing sources	18
Data was free	18
Data was inexpensive	7
Data was expensive	4
Sources of data '	
Data obtained from public sources	10
Data obtained from journals	16
Data obtained from books	8
Data obtained from Government reports, etc.	16
Data obtained from private sources (industry, insurance companies, etc.)	9
Data obtained from nonprofit organization	7
Data obtained from other sources	
State government	1
Expert opinion	2
Unpublished reports	1
Clinical trial information	2

SOURCE. Off Ice of Technology Assessment

Several respondents noted that, although the data may have been free, the time and effort required to obtain it was expensive. Another problem seemed to be determining whether the data existed, and if so, who had it.

Resource Needs of an "Ideal" CEA/CBA Research Team

The professions most frequently cited as being essential components of an office designed to perform CEAs and CBAs of health-care-related technologies on a regular and continuing basis are shown in table C-4.

Table C-4.—Professions and Support Services for an "Ideal" CEA/CBA Research Team

Professions/support services cited	Frequency of response
Profession cited	
Economics	21
Medicine	17
Statistics	14
Quantitative analysis[a]	13
Computer analysis	9
Epidemiology	9
Public health/public policy	7
Engineering	6
Psychology	4
Hospital administration	2
Medical sociology	2
Sociology/behavioral science	2
Medical student	1
Support services	
Secretary	16
Research assistant	15
Computer programer	9
Administrator	8
Librarian	3
Accountant	2

[a]Systems analysis, operations research, and decision analysis.

SOURCE: Office of Technology Assessment.

Several respondents specifically noted that provisions must be made for hiring the necessary consultants or experts as needed for assistance. Many respondents also cited the need for more than one of each professional (e.g., two physicians, three economists, several secretaries, six research assistants, etc.) to staff the research group.

The number of studies that this "ideal" office would be able to perform was estimated to be 6 to 10 studies per year with 8 to 12 full-time professionals and the necessary consultants, research assistants, and support staff present. The estimated cost of the research group was between $400,000 and $500,000 per year.

The estimated cost per study, not necessarily related to the aforementioned total office cost esti-

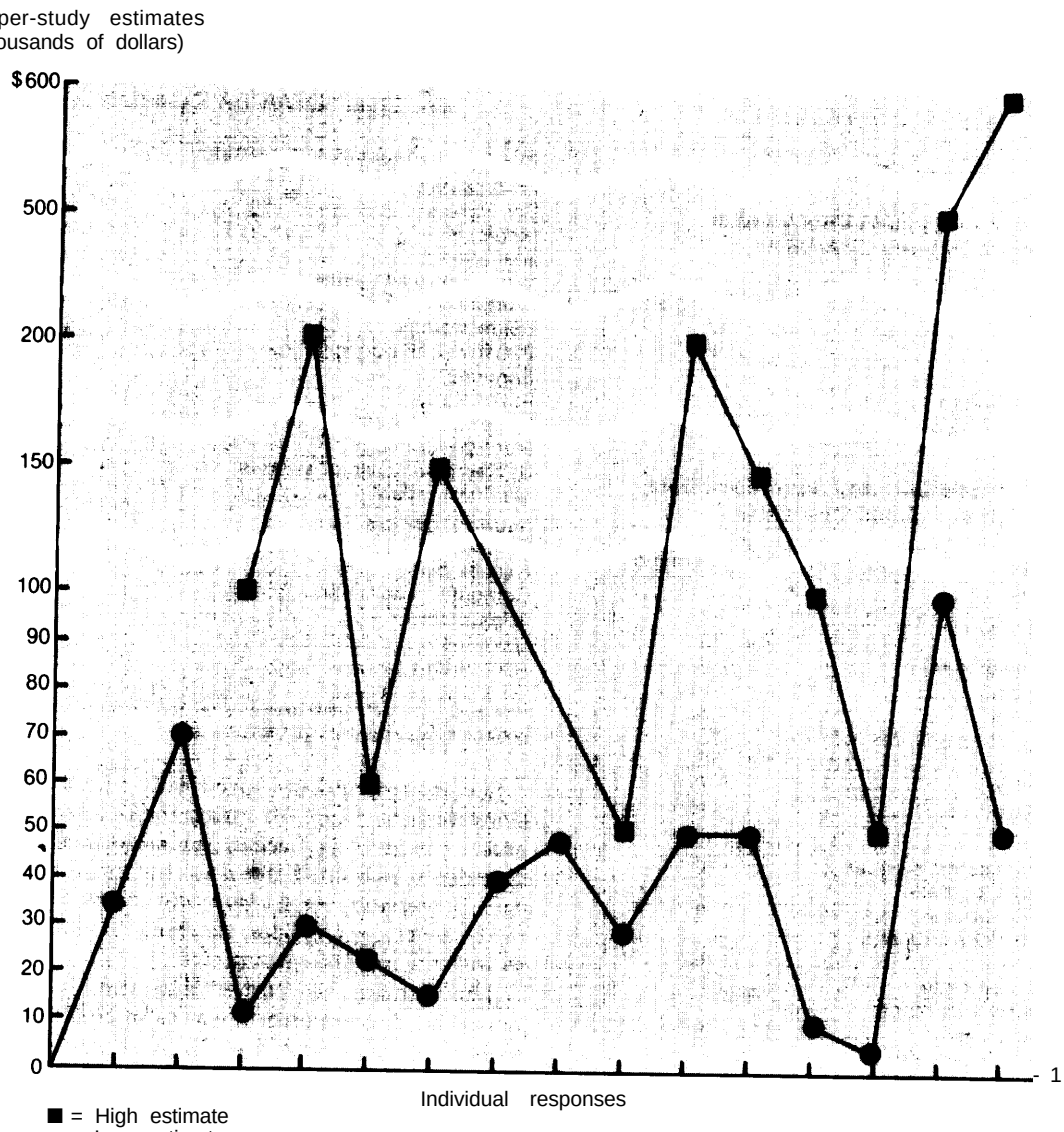
mates, covered a broad range. The graph in figure C-1 summarizes and illustrates the wide range and distribution of the cost-per-study estimates.

Many of the cost estimates were accompanied by reservations indicating a close correlation between the cost of the study and the extensiveness and severity of problems with information needs, the stage of the technology's development, the complexity of the problem being addressed, computer needs, and the costs to develop the study model. Though not the only variables cited, these problems received frequent mention by the respondents.

Discussion

The purpose of this survey was to identify information and personnel variables that might affect the "cost" of a CEA/CBA, solicit opinions regarding the

Figure C-1.–Range of Estimated Costs per CEA/CBA Study



aMost of the respondents Included a high and low estimate Of the cost-per-study,

SOURCE Office of Technology Assessment.

resources required to do CEA or CBAS on a routine basis, and to develop a range of estimates of the resources used to do published CEAS or CBAS. To a large degree, these goals were realized. Because of the survey's design, however, no firm conclusions are possible.

The scope of the survey was limited in the sense that the process used to select the sample population was restricted and arbitrary since the criteria used to identify the "quality" CEAS and CBAS were highly subjective. The survey sample was limited even further because its focus was restricted to CEAS and CBAS done only on health care topics, The survey sample may have been further distorted by the characteristics of the types of issues selected by researchers. Possibly, the CEA and CBA studies done to date have been "easy" ones. Analyses of health care topics may have been directed at areas that have had the most or best data available or that were the most highly visible in the public eye. On the other hand, a counterargument could be made. Since the field is relatively new, perhaps more work was required to develop the methods, more analysts were needed to perform quality work, and in essence, added effort was needed to establish the groundwork that other analysts can use. Whichever the case, it is difficult to know what effects these variables will have on the sample results.

The survey was broadly focused in that no attempt was made to differentiate between the various types of anaIyses. For example, no attempt was made to group the studies according to their technological focus (i.e., diagnosis v. therapy, procedure v. drug, or systems-based technology). Likewise, no attempt was made to determine if some analyses were considered more complex or sophisticated, or if some had more "value" to the research community or to the policy process, than others. Although these variables are very important, it was beyond the scope of this survey exercise to investigate them to the degree necessary to form estimates or conclusions.

At best, the survey results suggest a lack of consensus on most aspects of funding or staffing resources required for CEAS or CBAS. A good example of this lack of agreement are the estimates of what it might cost to perform a single CEA or CBA. In large part, however, the many reservations that the respondents included regarding the effect of data problems and the complexity of the issue(s) being examined on the cost of the study would explain the wide range of estimates provided. Although there were few surprises regarding the types of professionals or support staff required to do CEAS or CBAS, there was an interesting change in views in the responses received for the questions related to the types of professionals actually used to perform specific analyses and those related to the types of professionals recommended to staff the hypothetical research team. All but one actual study included a physician as part of the research group, but only seven included an economist. The hypothetical research teams, however, leaned more heavily toward the inclusion of economists. This apparent shift toward economists may not indicate any real change in the perceived need for physicians, though, because several respondents indicated a need for physicians and scientists to serve as consultants to the hypothetical research group. Thus, some of the respondents to the survey listed physicians in another category. The shift to more economists, however, was not explained.

Data needs and problems appeared to be a significant factor for all the respondents. The large range of cost-per-study estimates was directly tied to the availability and quality of data. Information needs were cited much more often than factors such as complexity of the problem being studied and stage of development of the technology as variables that will affect the cost of a given study. To date, however, the respondents seem to feel that many of the data range from being easy to difficult, but not impossible, to obtain. The data also were very inexpensive to obtain and were available from public sources.

The results of OTA'S survey may simply provide a look at the types of resources analysts have used to perform health-care-related CEAS and CBAS and at the types of resources they feel are necessary to perform them on a routine basis. It is interesting to note the differences between the resources used and the resources that the respondents felt *were* required to do CEA and CBA on a regular basis. Several professions that were not used a great deal by the actual study teams in the past show up quite frequently on the "ideal" research team list. Disciplines such as economics, statistics, engineering, computer analysis, and computer programing were not frequently used in the actual studies, yet were cited several times by the respondents as being needed for the hypothetical research group. As the health care issues become more complex and as information needs become more demanding, the range of expertise needed to do analyses will broaden, and the costs of performing CEAS and CBAS will increase. These resource costs may become an important factor to be considered by governments and other institutions that must decide whether or not to do CEAS or CBAS or how many they can do in a given time period.

ADDENDUM

QUESTIONNAIRE USED IN THE

SURVEY OF THE COSTS OF CEA/CBA

PART 1

What is the general cost range that you feel is adequate to perform most cost-benefit or cost-effectiveness studies on medical technologies, techniques. procedures or systems?

_____

_____

_____

_____

We realize that the costs will vary according to different factors (e.g.. type of technology, stage of development). What are the factors that must be considered **and that are most important in effecting these cost differences, and how** would they affect the cost of analysis?

_____

_____

_____

Additional comments: _____

_____

Note: This aspect of the survey (Part 1) is very important to us; therefore we request that it receive priority should you find yourself short of time: however, we do urge you to complete all parts of this survey.

A. Hypothetical situation: You are asked to staff an office that would be responsible for carrying out cost-effectiveness or cost-benefit studies of

a range of medical-related technologies. techniques. procedures, and systems. What are the necessary disciplines to carry out this function? How many of each?

1. _____ 6. _____
2. _____ 7. _____
_____ 8. _____
4. _____ 9. _____
5. _____ 10. _____

What support services or personnel are needed? How many of each?

1. _____ 4. _____
2. _____ 5. _____
3. _____ 6. _____

PART 2

The intent of this part of the survey (part 2) is to obtain a range of estimates of resources that have been used to perform cost-effectiveness or cost-benefit studies. Since you have either performed or directed such a study, we ask for your help in providing us this information. At the bottom of the page, we have indicated the study or studies that we wish you to address.

Perhaps the easiest and quickest way for you to help us would be if you just send us the budget breakdown that was developed for the study or studies listed for contract or grant purposes. Should you wish to keep certain parts of it confidential, please black them out.

If you do not have a budget breakdown or would prefer not to send it, we ask that you fill out sections I and II below instead. For those of you who are sending the budget page, we ask that you also fill out section II only.

I. How many professional level people directly contributed to the analysis?

_____

A. **What** types of training or educational backgrounds did these people have (what did they consider their professional niche)? Please check off the disciplines involved; if more than one person in each discipline, please indicate how many. Please indicate level of education, B.S., M.S., Ph.D., etc.

1. Economics (Specialty _____)

2. Health care professional(s):

   a. Physician(s) ___ practicing or academic _____

   b. Public health ___

   c. Hospital administration ___

   d. Epidemiologist ___

   e. Scientist(s) ___

      What discipline(s) _____

      _____

3. Systems **analysis** ___

4. **Operations research** ___

5. **Computer analysis** ___

   a. computer programming ___

6. **Public administration** ___

7. **Lawyer** ___

8. **Other** _____

B. What level of effort did these people contribute to the study in terms of full-time equivalents or person days, weeks, or months devoted to

the analysis? (State type of professional and time spent.)

	Professional	Time spent on study
1.	_____	_____
2.	_____	_____
3.	_____	_____
4.	_____	_____
5.	_____	_____
6.	_____	_____

II. The information needs of the analysis:

A. What types of information did you need for your analysis? Please try to be fairly specific (for instance, equipment cost, personnel costs, drug costs, cervical cancer deaths per year, disability data, genetic disease prevalence data, etc.).

a. Direct cost(s) data _____

_____

_____

Direct benefit(s) data _____

_____

_____

c. Indirect cost data _____

_____

d. Indirect benefit data _____

_____

e. Morbidity data _____

f. Mortality data _____

/3* Epidemiological data _____

h. Health services utilization data _____

i. Other data needs _____

B. Was the data required for your study:

a. Readily available ___

b. Difficult to obtain ___

c. Almost impossible to get in a usable form ___

C. In order to obtain data in a usable form for your purposes, did you have to:

a. Purchase it (buy data tapes, compiled lists, etc.) ___

b. Do original research ___

c. Collect from existing sources ___

D. Was the data

a. Free ___

b. Inexpensive to obtain ___

c. Expensive to obtain ___

E. Was much or all of the data obtained from:

a. Public sources ___

1. journals ___

2. books ___

3. government studies or reports ___

4. other _____

b. Quasi-public sources, non-profit organizations, foundations. etc. _____

c. Private sources (industry, insurance firms, etc.) ___

F. Additional comments or explanations:

_____

_____

Appendix D.—Values, Ethics, and CBA in Health Care

by The Hastings Center
Institute of Society, Ethics and the Life Sciences
Hastings= on= Hudson, N.Y.

Introduction

The past two decades have been as hard on science as they have been on many other once venerated institutions, and for at least one of the same reasons: The idea that it consists exclusively of delineation of truths about the state of the world has been successfully challenged.

In some instances, scientific "facts" have been revealed to be deliberately falsified, Apart from tarnishing science's image, falsification has other consequences. If a cancer researcher fakes results, others may spend months or years and large amounts of public money attempting to replicate them. If a psychologist fabricates IQ studies of twins, the data may be used in other similar studies and reviews, and the cumulated erroneous data may be influential in formulating a worthless (at best), or even a repressive and openly racist (at worst), social policy.

It is one of the axioms of the scientific enterprise that the norms and internal controls of science guarantee that such abuses will ultimately be exposed. Even if that is true for flagrant violations, however, it is not clear that it is true or even can be true for the much more subtle distortions inherent in the daily practice of science. These generally involve nothing so egregious as deliberate falsification of data, but rather much less obvious effects of the researchers' biases and value commitments, many of which are unconscious.

In general, the argument that science's value-freedom is illusory has fallen less harshly on the "hard" or natural sciences than on the "soft" or social sciences. Economics, however, is almost in a class by itself. Since its methods are quantitative almost by definition, economics appears to be relatively "hard" compared to many of the other social sciences. Partly because of this, economics has been one of the least criticized social sciences.

In recent years, that situation has begun to change. Largely as a result of the "dismal science's" dismal record of forecasting, economics has been subjected to increasing criticism. That record has been increasingly frustrating for citizens and policy makers, both of whom must somehow cope with continuing uncertainties about inflation, unemployment, and other economic indicators. Deciding between big bang and steady state theories of the universe may ultimately have profound effects on our view of mankind, but most people are more concerned and more immediately affected by theories of economics. The impact of economics on government policy and daily life underscores the importance of accurate predictions and conclusions.

Among the currently fashionable methods of economics are CEA and CBA. A major text in the field of CBA was written by the economist E. J. Mishan, who believes economists doing CBA are really asking a question similar to that asked by a company accountant (410). Instead of asking whether the company will be better off engaging in one activity as opposed to another, the economist asks whether society would be better off by undertaking the project under study. In Mishan's view, CBA applies standard economics to help advance the social welfare. The rationale is based on the Pareto improvement, [1] which occurs if some economic arrangement makes people better off without making anyone worse off.

According to Herbert Klarman, a pioneer in using CBA in the health field, its use by experts, and the idea that it should influence public decisions, is only a generation old (317). There are few references to it in the literature before 1958. More recently, CBA has become a favorite tool among policymakers and has been applied in many areas of public life. Along with systems analysis, CBA has been widely used by the military and for major public works projects.[2]

Noting that investment in human capital was a popular theme in postwar economics, Mishan suggests that it was an easy step from investment in human productivity to investment in health (410).

NOTE: This appendix includes nearly all of a report prepared by The Hastings Center under contract to OTA. As with all reports published by OTA, its inclusion does not imply endorsement by the members of the Technology Assessment Board or by any advisory panels. Although the report focuses on CBA, the discussions apply in many instances to CEA as well. The report should not be considered an exhaustive treatment of ethical issues involved in the conduct and use of CEA/CBA.

[1] Named for the Italian economist and sociologist Vilfredo Pareto (1848-1923). "An equilibria is said to be 'Pareto-optimal' if (and only if) there is no possible movement from it that could make everyone better off" (531).

[2] One famous and very controversial British study, for example, used it to help find a site for a third London airport.

The rationale is simple: Disease and premature death are costly to an economy, so investment in medical care can bring significant economic rewards by increasing human capital.

An increasing number of cost-benefit and cost-effectiveness studies have been done in the health field in the last decade, although how influential such studies have been in the forging of policy is not clear. Rashi Fein has pointed out that the attractiveness of economic arguments derives from a belief that economics is value free and objective (194). That makes it seem particularly appropriate for fields dealing with social policy, such as health care, which are fraught with value conflicts and otherwise appear to lack rigorous guidelines for decisionmaking.

Sociologist Duncan MacRae suggests that the historical development of economics has narrowed the discipline's outlook, making it highly specialized and scientific (394). Whereas economists like Adam Smith all made contributions to disciplines other than their own, today's economists are either interested exclusively in economics, or they are interested in extending economic theory to other fields like political science or sociology.

Thus, MacRae argues, welfare economics is a much more closed system than its predecessors, more impervious to values that come out of other aspects of its practitioners' lives. According to R. M. Parish, this kind of narrow specialization is exactly what CBA needs least (475). Good CBA, he says, "seems to call for a combination of subtle theoretical analysis, imagination, and a lot of hard, pedestrian slugging—the capacities to do which are seldom combined in one individual."

The need for economists with diverse capacities may be especially great in the area of health. As noted by R. N. Grosse, there has been little analysis of the relationship between expenditures for and outcomes of social programs (265). At the very simplest level, we really do not know how medical services affect our health, in part because "health" is an abstract and fuzzy concept. Health programs often really cannot be evaluated. We lack agreement on their objectives and on how to achieve them. We find it hard to estimate measures even when we agree on them. We cannot sort out cause and effect relationships because there are many inputs and outcomes. Finally, we have very little information about programs whose effects occur over a long time.

To pick a program goal and then attempt to evaluate how well the program is achieving that goal requires normative assessments, evaluations, and assumptions. CBA, however, is simply descriptive. Norms, cutoff points, decisions about where to draw the line between good and bad outcomes, all are in the mind of the policy maker and need to be imposed on the data from outside. Norms are also in the analyst's mind, although analysts' commitment to the disciplinary ethos of quantification and objectivity may make them genuinely and sincerely unaware that norms are an inevitable part of their description.

In a book-length critique of CBA, Peter Self asserts that CBA depends on value judgments more than most types of information (556). Further, some of the major figures in economics, including many of that field's most thoughtful practitioners, share substantial agreement that economics in general and CBA in particular are anything but norm and value free.

This paper lays out major values underlying the quantitative techniques of cost-benefit and cost-effectiveness analysis in the health field. It shows how those values can result in ethical problems, and makes some suggestions for avoiding the difficulties the values engender. Though based on extensive reading of both the theoretical and technical literature in this field, the discussion is not exhaustive. Our brief for this exercise has been well stated by Fein, who observed that it is incorrect to suppose that measurement is neutral. "Cost-benefit analysis," he says, "is too important to be left to analysts or economists" (194).

Working Definitions of Cost-Benefit and Cost-Effectiveness Analysis

Definitions of CEA and CBA in the literature vary. OTA has defined them as follows:

Cost-Effectiveness Analysis (CEA): An analytical technique that compares the costs of a project or of alternative projects to the resultant benefits, with costs and benefits/effects not expressed by the same measure. Costs are usually expressed in dollars, but benefits/effects are ordinarily expressed in terms such as "lives saved," "disability avoided," or "quality-adjusted life years (QALYs) saved," or any other relevant objectives. Also, when benefits/effects are difficult to express in a common metric, they maybe presented as an "array."

Cost-Benefit Analysis (CBA): An analytical technique that compares the costs of a project or technological applications to the resultant benefits, with both costs and benefits expressed by the same measure. This measure is nearly always monetary.

E. J. Mishan says (410):

> The general question that a cost-benefit analysis sets out to answer is whether a number of investment projects, A, B, C, etc., should be undertaken and, if ingestible funds are limited, which one, two, or more, among these specific projects that would otherwise

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> qualify for admission, should be selected . . . in cost-benefit analysis we are concerned with the economy as a whole, with the welfare of a defined society, and not any smaller part of it.

Mishan does not concern himself specifically with CEA, but his characterization of CBA is broad enough to encompass it.

For Prest and Turvey (487):

> Cost-benefit analysis is a way of setting out the factors which need to be taken into account in making certain economic choices, Most of the choices to which it has been applied involve investment projects and decisions—whether or not a particular project is worthwhile, which is the best of several alternative projects, or when to undertake a particular project.

This definition, too, is broad enough to encompass both CEA and CBA.

Not surprisingly, therefore, Weinstein and Stason observe (627):

> Cost-effectiveness analysis and benefit-cost (or cost-benefit) analysis are two related, but quite different, approaches to the assessment of health practices. Confusion frequently exists between the two approaches, and many analyses that are technically cost-effectiveness analyses are often labeled "cost-benefit" analyses, and vice-versa. The key distinction is that a benefit-cost analysis must value all outcomes in economic (e.g., dollar) terms, including lives or years of life and morbidity, whereas a cost-effectiveness analysis serves to place priorities on alternative expenditures without requiring that the dollar value of life and health be assessed.

According to Klarman, since CEA does not require valuing life and health in monetary terms, the dilemmas of valuation that arise with CBA can be avoided by substituting CEA (317). When we substitute CEA for CBA, though, we have lost the tool for setting priorities among several fields of activity.

Richard Layard who edited a major volume on CEA and CBA, holds that CEA is useful whenever CBA becomes impossible (349), The reason is that even if the planned benefit itself cannot be valued sensibly, it is still useful to compare the costs of providing the same benefit in different ways. Apart from providing a value for the benefits, he says, the procedures involved in CEA are exactly the same as those for CBA. Self, a major critic of the cost-effectiveness technique, says that cost-effectiveness can easily shade into CBA because goals cannot be described with precision (556).

In general, though, CEA aims to tell policy makers the least expensive way of achieving an already selected goal, whereas CBA seeks to help them select from among a choice of goals. Largely because it is considerably more ambitious, CBA presents more ethical difficulties than CEA (though the latter is certainly not free of them). This paper concentrates largely on CBA, but much of what is said is equally applicable to CEA.

Implications of the Conflict Between Equity and Efficiency

Among its practitioners, there is substantial agreement that efficiency is the chief value underpinning economics. Those practitioners also freely acknowledge that since this value is its central one, economics—and particularly CBA—gives short shrift to another important value: equity. The conflict between equity (often expressed by the terms equality, justice, fairness) and efficiency is an enduring one in both politics and in economics. This conflict is the most important and intractable value conflict in CBA and is also its most important ethical issue. How, in pursuit of the least wasteful way of spending the public dollar for a given commodity, can we also make it available to everybody?

Efficiency .—Economics is grounded in philosophy.[3] Its chief underpinnings came out of the philosophical school known as utilitarianism. Probably the most thoroughgoing attempt to construct a moral theory whose chief value is efficiency was made in the late l8th century by Jeremy Bentham (49). One of the earliest utilitarians, Bentham intended his system to apply both to the broad social arena encompassing politics and legislation, and to the action of individuals. The goal of Bentham's utilitarianism was to maximize pleasure and minimize pain for all who stood to be affected by an action.

Bentham's system presupposes an ability to quantify pleasures and pains. The process he described for judging outcomes required a precise specification of the tendencies of actions to produce pleasures or pains. All such tendencies are added together, and then totaled for all affected individuals until an optimal "balance" of pleasures over pains is reached. Bentham's scheme is sometimes called the "hedonic calculus" or the "felicific calculus. " The term "calculus" should be taken quite literally, since social decisionmaking using this method requires straightforward calculations—adding and subtracting units of pleasure and pain.

Utilitarians, after Bentham, subscribed to the basic idea of maximizing utility as a social goal, but' doubted the feasibility of constructing cardinal measures of utility. They sought to achieve the same result by employing an ordinal scale. Like Bentham's, their approach still allows for a meaningful notion of maximizing individual welfare by enabling comparisons

[3] 'As Kenneth Boulding has pointed out, Adam Smith was a professor of moral philosophy ***(67)***.

of states of affairs as "better" or "worse," although it is not committed to implausible measurements and calculations of pleasures and pains. The resort to ordinal measurement of utility, however, founders on the nettlesome problem of interpersonal comparisons of utility. Until we work out a common measure for one person's "better" and another's "worse," the achievement of social welfare will remain an elusive goal.

Equity.—In contrast to the value of efficiency that characterizes utilitarian theory, the central value in philosophical theories based on the concept of justice or fairness is equity. John RawIs's theory of "justice as fairness" (494) offers a compelling alternative to the philosophical foundations of CBA. Rawls criticizes utilitarianism's willingness to allow the greater gains of some to offset the lesser losses of others. In theory, all versions of utilitarianism could justify actions by a majority that would enhance the majority's position at the expense of a minority. Even if it could produce a total social welfare based on ordinal measures of satisfaction, however, Rawls would still object to utilitarianism on the ground that individual rights and liberties are liable to get lost in the aggregating shuffle. Although utilitarianism presents itself initially as an individualistic democracy of pleasures ("everybody to count for one, nobody for more than one'' —Bentham), Rawls observes, aggregating the desires of each individual into a total system of desire actually results in a morally defective impersonality that allows some peopIe to be used merely for the benefit of others.

Rawl's rival conception of equity is unique. Whereas some political theorists construe equity to mean equality (to everyone an equal share), and others treat it as a proportionality of some sort (to each according to need, merit, contribution, etc.), Rawls begins his notion of distributive justice with a preference for equality that is immediately hedged by a so-called "difference principle. " This principle permits significant differences in wealth and social status, but only those differences that benefit the least advantaged members of society. (This device makes interpersonal comparisons of utility unnecessary.) Thus, while it it permissible for a society to allow certain kinds of inequalities that raise everyone's standard of living—in fact, Rawk holds that it would be irrational to insist on equal shares of fewer goods—the difference principle rules out those inequalities that improve the status of the better-off at the expense of the worse-off. Once a society adopted the difference principle, individuals would be entitled to whatever goods or status they could obtain or achieve through voluntary transactions on the free market, gifts, and so on.

Two examples will suffice to illustrate the sorts of equity problems central to CBA: 1) the different ways in which people's lives and preferences are evaluated, and 2) the way in which the future is evaluated compared with the present.

Valuing Lives and Preferences

The problem of placing different values on different lives is keenly felt and discussed in all the theoretical literature on CBA, even by its friendliest adherents. This problem is particularly germane to the health field and is as old as CBA in that field. The 17th century estimates of the value of life by Sir William Petty illustrate a problem that remains unsolved (194). To strengthen his argument about what the government might save by transporting people out of the city and saving them from the London plague, Petty valued people at only t20 each, kecause that was the lowest price for a slave. For his largely illustrative and rhetorical purposes, setting the lowest possible valuation on labor effectively made the point. Petty's calculations of what the government might have saved was an underestimate; the actual amount would, of course, be much more, since not only "slaves" would be transported out of the city and saved from the plague, but "more valuable" persons as well.

In a 19th century sanitation study in Massachusetts, Lemuel Shattuck valued adult men differently from women and children (559)—a practice often adhered to today. Shattuck's estimates of the benefits of better sanitation in Massachusetts involved labor costs (calculated at an average of $50 a year) totaling almost $5.5 million, to which he added the cost of public support of dead laborers' widows and children. But the latter costs came to only an additional estimated $2 million.

The Issue and the Methods

Fein puts the whole issue nicely (194):

> Does the measurement of a person's worth in terms of his productive contribution really represent our social values? I believe that it does not do so. In particular, it fails adequately to take account of equity and distributional considerations (which many believe to be one of the major functions of government).

Fein points out that taking account of individual characteristics such as potential earnings could lead society to provide health care services to those with the highest potential income and to direct them away from those with low earning capacity and less education. Females in such a scheme are at a disadvantage compared to males, as are the old compared to the young, and blacks compared to whites. In each case,

the disadvantaged individuals are the ones with the lower incomes.

Klarman argues that some elements of the controversy over valuing lives have been settled by recent technical developments (317). Whereas 15 years ago a wife not employed outside the home did not count for very much in a CBA, he notes, recently housewives' services have been given a monetary value. The lives of housewives are now usually figured on that basis, though still, of course, at a lower rate than the lives of their husbands employed outside the home.

When cost-benefit calculations were begun at HEW in the middle 1960's, Agency officials realized that the benefits calculation discriminated against women and the elderly (265). According to Grosse, there were fears among some that too vigorous pursuit of the underlying logic would penalize not only health programs for the aged (medicare was just getting started), but programs for the poor, as well. He notes, however, that (265):

> In actual practice, for the particular programs studied, these concerns were not critical. The programs for cervical and breast cancer looked to be good despite their being for women. As for the poor, most of the programs considered, especially cervical cancer, syphilis and tuberculosis, were aimed primarily at them, and projects were usually located to serve low-income residents.

By implication at least, this is a way to get around some of the distributional problems inherent in CBA: If decisionmakers are committed to just distribution of resources and are interested in choosing the best programs aimed chiefly at the poor, i.e., if equity considerations are built in from the start, then the problem of equity can be dealt with. This is, of course, a big if,

Mishan believes that although distributional and equity considerations do offer grounds for objection to CBA, the objection is not a strong one (410). Even if a project is cost-beneficial, Mishan notes, it can always be rejected on grounds of inadequate attention to equity. Writing on the relationship of Christian ethics to CBA, Denys Munby agrees, arguing that the use of differential income data usually makes social decisions easier (416). If one found out that only millionaires would be affected by the destruction of a particular beauty spot, one might not worry very much about its destruction. "What is quite clear is that we can make our analysis as egalitarian as we want, " he says. What these views require, however, is that policy makers be committed to equity—and that they act on the basis of that commitment.

Another major objection to the usual way people are valued for purposes of CBA is that focusing on income ignores other indices, Mishan acknowledges that CBA tends to ignore indicators of social merit, but argues that we have no good way now of judging their worth (410).

CEA attempts to avoid the seemingly insoluble valuation problem of CBA by simply jettisoning much of it. Instead of comparing costs in dollars to benefits in dollars, CEA compares costs in dollars to benefits expressed in nonmonetary terms (e.g., numbers of deaths averted). As Weinstein and Stason note, however, a limitation of both CEA and CBA is (627):

> . . . that the benefits and costs to individual members of society need to be aggregated. If the inequitable distribution of benefits and costs across individuals or groups are of concern, a single cost-effectiveness measure will not do. However, as economists are wont to argue, over large number of programs and practices the inequities are likely to even themselves out and, with some exceptions, may reasonably be ignored.

Even assuming that some of the aforementioned problems could be solved, other problems in valuation would remain. In comparing the loss of a man's life with that of a woman, one is at least comparing two identical types of outcome. As Fein points out, however, problems arise in comparing apples-and-oranges types of outcomes, for example, lives saved in comparison with blindness prevented (194). Grosse agrees, noting that when dealing only with the cost per death averted, there is no way to compare or rank diseases that do not usually kill (265).

CBA has not yet found a common way of measuring different outputs from different programs so that they can be sensibly compared. Finding a common unit of measurement is very difficult. We are unable to measure units of satisfaction or of happiness generated by various government activities, nor are we able to compare one person's satisfactions with another's.

One way of dealing with this problem has been by using the concept of quality-adjusted life years (QALYs). Even the concept's chief proponents, however, concede that the weights it gives to varying states of health are subjective, so much so that (627):

> In a cost-effectiveness analysis at the societal level, it is therefore essential that a range of possible weights be used to reflect the spectrum of individual values . . . Most analyses avoid quality-of-life considerations entirely, quantifying only the change in life expectancy. Where the quality-of-life effects are believed to be important, however, the credibility of an otherwise effective analysis may be jeopardized. Tradeoffs involving quality-of-life considerations are made implicitly by health-care decisionmakers all the time; the role of the analysis is to make them explicit.

The difficulty here, as the authors implicitly acknowledge when they draw attention to the subjec-

tive nature of the QALY concept, is that such assessment reintroduces the problem of determining values that the use of CEA is supposed to avoid.

Mishan's text contains extensive material on the valuation of life and limb (410). Though philosophers could legitimately disagree with his view, Mishan regards saving life as symmetrical with losing it, so he bases his techniques almost exclusively on established legal methods of compensating victims. Self says this is actually an ethical rather than a legal position, a position that specifies that involuntary losses should be compensated (556).

Despite repeated expressions of dissatisfaction with it, the most common way of calculating the worth of a person's life is by discounting to the present that person's expected future earnings. Mishan discusses alternatives to this conventional way of valuing loss of life, one being to perform auxiliary calculations that take account of a victim's suffering or family's bereavement (410). He appears to favor evaluating the saving of life by referring to what each person would be willing to pay or to receive for an estimated change in the risk to his or her life. These calculations could change over time. Mishan argues that one might ultimately want to forgo bereavement calculations altogether because of the gradual loosening of family ties and the decline of emotional interdependence. In a wholly impersonal society, any member of the community is equally replaceable—in such a society there would be no suggestion of compensating for bereavement.

Weighting Schemes

One attempt to improve equity considerations has been to use utility rather than dollars, and to assume that income has diminishing marginal utility. This means each person's income is valued equally up to a fixed amount; income over that amount is given less importance in the calculations. One obvious difficulty with such weighting attempts is, **as** Mishan (410) and others (194,556,349) point out, to secure a consensus on the weighting. And, of course, a weighting scheme could still end up making the rich richer and the poor poorer.

Klarman argues that weighting, though not wrong, is certainly judgmental (317). To make his point, Klarman cites as an example the weighting of earned income more heavily than the same amount of money received from public assistance. Such weighting is based on the assumption that money earned from work is somehow morally better than money received as public largesse. It may be legitimate to use such weighting in CBA, but as Klarman states (317):

> What must be recognized is that weights are judgmental, are likely to be arbitrary (at least initially), should be derived in the public arena, and, above all, must be clearly stated.

Layard favors CBA in which valuation is arrived at by asking people what they would pay to acquire the benefits or avoid the costs (349). Of course, one doesn't usually ask people such questions directly, although some economists advocate a questionnaire approach. Far more often, values are put on such programs indirectly by inferring what people value from their behavior, often from what they buy.

Munby approves attempts at valuation based on people's purchasing behavior (416):

> In principle, the whole analysis is entirely democratic; it starts and ends with what people actually want, as shown by their actual choices.

On the other hand, though, as SeIf argues, willingness to pay is in some sense related to ability to pay, so analyses in which this is used for the purpose of valuation are still biased toward the better-off (556).

The Veatch Critque

Robert Veatch asserts that valuing lives presupposes that we have decided the question of value to whom (603). In some theories of valuing lives, the relevant consideration is assumed to be value to society or aggregate value. In others, it is value to the individual (e.g., self-rating weighting systems), to some specific social group, or to the person who pays the bills (who might also be the U.S. taxpayer).

Veatch also points to a problem with the willingness-to-pay approach for weighting (603):

> At least up to a certain point, the older one becomes, the more valuable life becomes (because, subject to willingness to borrow, one is generally willing to pay more to avoid death risks since one has a higher income level). On the other hand, in human capital terms, the older one becomes, the less valuable life is, to the point that at retirement (at least for males who tend not to be involved in non-monetized domestic labor), life is "worthless". . . Any formula based on personal estimates of how much one would pay to avoid a certain risk of death accepts the status quo distribution of incomes in our society. Accepting it may be efficient—it may be utility maximizing—yet I am still convinced it is not just and it is not right.

Veatch argues that the Kantian maxim that the individual is to be treated as an end, and never only as a means, prohibits policies in which one individual or group benefits at the expense of another, even if the net benefits far outweigh the harms. Many economists have dealt with this and similar arguments by a pragmatic counterargument—that we are in fact making precisely such invidious comparisons with current policies arrived at on intuitional or other "nonscientific" grounds. By this argument, an important advantage of valuing lives for purposes of CBA is that it brings us face-to-face with the fact that, even

in a supposedly egalitarian society like the United States, we value different people's lives differently.

As Veatch says, however (603):

> If it is conceptually unsound to equate the real value of a life with the value of the labor that life will produce, it is also unsound to conclude that there is any necessary relationship between what I would pay for a program to add years of life, and what society ought to pay for that program.

Willingness-to-pay approaches favor those with greater ability to pay. Even willingness-to-pay measures that concentrate on percentage of income rather than absolute dollars really do not solve the problem, because selection of an acceptable percentage of income also has a very strong relationship to income level.

Finally, willingness-to-pay measures fail to tell us anything about peopIe's attitudes toward programs in which they have no particular stake, like prenatal diagnosis for women who have no plans to bear children and are not close to anybody who has such plans. Sheer self-interest dictates that one should be willing to pay for programs in which one is or may be personally involved. As Veatch notes (603):

> One might have a self interest in avoiding the witnessing of suffering around him, but this does not seem to be the real moral basis of a commitment to help programs designed to affect a condition one is virtually certain not to have.

Veatch here ignores the argument (based on CBA!) that such programs will save taxpayers money (e.g., by reducing the cost of institutions for the retarded).

Veatch identifies four different assumptions that can be used as the basis for valuing lives: 1) all lives are of equal value, 2) the prime of life is most worthwhile (because it is most productive), 3) youth is most worthwhile (because young people have more life ahead of them), 4) old age is most worthwhile (because older people are wiser?). Still Veatch says (603):

> . . . all have an equal claim to the health care needed to provide a level of health equal, insofar as possible, with other people's health . . . it will necessarily have to give way to other claims upon occasion, but it should be the decisive starting point for evaluating alternative public policy.

Thus, social usefulness, willingness-to-pay, future productivity, and other such measures are and ought to be irrelevant,

CBA is all right for some things, Veatch says, like teIling us that some diseases kill people in the prime of life. Whenever policy choices conflict with individual rights and justice, however, Veatch wishes to promote justice "even if that means lower aggregate indicators of utility" (603). Younger people should receive a higher priority than others, because "an essential part of the egalitarian principle of justice is that there should be, insofar as possible, an equal opportunity to live to the same age as others. " There should also be equality of opportunity to live life without suffering, Veatch believes. But here the policy objective should not be to achieve a maximum reduction in suffering (because that is an aggregate measure), but rather to reduce those conditions that produce the greatest suffering in the individuals who have them. Veatch believes that sometimes the needs of the poor may have to be placed ahead of those who may die relatively early. He also favors excluding or giving low priority to deaths for which the victim is personally responsible (e.g., death from lung cancer caused by cigarette smoking). According to Veatch, involuntary conditions should have higher priority than voluntary ones.

Discounting

Another important distributional problem that requires at least a brief discussion is selection of the discount rate and the related problem of comparing the future with the present, given inflation and the fact that, in general, people prefer benefits now to later. Self traces the problem, in part, to the Pareto principle itself, which he believes is "strongly biased towards the status quo" (556).

Layard (349) points out, and other analysts (317, 194) agree, that the question of the welfare of future generations is simply ignored in most CBAS. Layard believes most economists would argue that projects should be judged exclusively in terms of their effects on the welfare of those now living. He notes (349):

> If one takes the alternative view that cost-benefit analysis aims to throw light on what is right, it is difficult to think of any ethical justification for ignoring future generations. A practical argument is sometimes put forward for ignoring them—that we cannot know their preferences. However, there are many items (like life) where we do not know how they are valued by present generations, and many (like bread) that we can be fairly sure what future generations will feel.

The discount rate, which takes into account what a dollar invested today would earn in interest if it were not being spent on health care, tends to devalue the future. MacRae says economists' evaluations of the future may be wrong (394), Rawls has even suggested a zero discount rate to promote justice among generations (494). In his discussion of the discount rate, Mishan describes the political constraints on decisions about which discount rates to use (410). Klarman notes that Federal agencies have traditionally used a wide range of discount rates, usually without giving any kind of justification (317). The conse-

quences of choosing a high or a low rate, he says, are clear: Low rates favor projects or programs whose benefits accrue in the distant future. When the project is short-lived, the choice of a discount rate is minor. Nevertheless, Klarman says, there may even be some merit in employing a single number for all public human investment projects,

Conclusions

The distributional issues discussed above present both philosophical and practical problems for economists. According to Layard, most economists would argue that income should be redistributed by direct cash transfer rather than by juggling choices of public projects (349). But Layard believes that if we are reasonably certain that the cash will not be so distributed, then the poor person's dollar ought to be valued more highly than the rich person's.

As he sees it, the problem is less one of equity than it is of knowing which constraints should be taken as given and figured into the analysis. If a Government agency knows for certain that cash will not be redistributed, even if it should be, then the agency should allow for distributional factors when it evaluates a project. It should not allow for those factors if it can ensure that redistribution will be achieved by some more appropriate method. In practice, however, a Government agency cannot know this. Until this is settled, it may be impossible to rationally appraise a project. Privately employed economists, too, are free to incorporate distributional measures (e.g., no-cost transportation to neighborhood health centers for the elderly) into their equations.

According to Layard (349):

> A decision-maker should choose from his available set of actions the ones which maximize social welfare, subject to all the constraints over which he has no control.

It should be noted, however, that this approach allows economists enormous latitude without requiring that they incorporate distributional considerations. It can also be argued that such an approach completely subverts the purpose of CBA, which attempts to free the policy process from the individual beliefs and biases of the policy maker,

To summarize, the traditional approach to CBA excludes formal consideration of distributional effects such as equity and fairness, Since economists disagree about how to solve this problem, equity considerations are likely to continue *to* be underemphasized in practice. Mishan, for one, believes that the situation is unlikely to change in the foreseeable future (410).

Implications of Data Problems

After distributional/equity issues, the criticism most often made of CBA is that the data employed are simply not reliable. This criticism appears to be legitimate to an undetermined, possibly large, extent. It is a very serious criticism, chiefly because it conflicts with the quantitative and scientific trappings of CBA.

The general problem here, as Parish points out, is that CBA is a form of modeling, and, like all other attempts at modeling, expresses only certain aspects (and those perhaps not wholly accurately) of a very complex world (475). CBA is "irretrievably" second-best; that is, CBA seeks to explore the consequences of particular actions in the imperfect economy as it exists and in which the insights of theories applicable to a first-best world maybe misleading. According to Parish, "Its practitioners are frequently tempted to simplify their problem by making first-best assumptions . . . " (475).

The Problem of Defining Outcomes and Goals

Criticisms of data are made on a number of different grounds. Most obvious are the simple inadequacies of our current reporting and data systems. In a very real sense, of course, such inadequacies are technical problems that can be improved once they are identified and someone in a position of power decides to deal with them and improve the system.

Fein, however, has identified a number of much more difficult data problems (194). One concerns the measurement of outcomes which are conceptually amorphous (e.g., higher levels of health) and to which many factors contribute (e.g., housing, income, nutrition, environment, and medical care of all kinds). In addition, there are many factors whose relative contribution may differ for different persons, and whose proportional importance is largely unknown. Another problem is how to measure the goals of a health program that exist on a continuum. It is more difficult to measure continuous states with a wide range of effect, like pain or impairment of functional ability, than to measure discontinuous states like life and death. Furthermore, difficulties in measurement create a bias in favor of programs that have easily measurable goals.

Most discussions of CBA mention the selection of goals and objectives as a problem. Many critics and even some proponents of CBA feel that the goals are often vague and nonspecific (e. g., "health"). Kenneth Boulding points out that we often agree on major goals (67). We disagree on how to reach the goals, and in some cases, on what the alternative ap-

preaches might be. Mishan, for example, criticizes Klarman's study of syphilis by pointing out that the calculations were based on the goal of eradicating the disease (410). There was no comparison with a defined control program to reduce the disease to some prespecified level.

Ironically, Klarman himself is quite aware of the difficulty (317). He believes the costs of a particular health program can be estimated, but that the field's chief difficulty arises in formulating the contents and expected outcomes of programs before estimating their cost. In a study of the end-stage renal disease program, for example, CEA pointed to the superiority of kidney transplantation, with dialysis only for initial and backup support. Klarman believes that if a CBA had been performed, the shortage of suitable kidneys and the relative ease with which dialysis facilities could be expanded might have yielded a higher net benefit value for dialysis.

Economists may also make naive assumptions about the practicality of stated goals and outcomes, Klarman says (317). At least in the early days of CBA, he notes, there was a tendency by economists to attribute greater efficacy to medical care than we now believe is warranted.

The Problem of Uncertainty About the Future

The whole area of uncertainty about the future —guesswork about costs and benefits of technological innovations and unanticipated shifts in demand— continues to plague CBA. According to Mishan (410):

> The problem of how to make decisions in any situation where the past affords little or any guidance is not one that can be satisfactorily resolved either by logic or empiricism, and what moves have been formulated are either of limited application or of no practical value,

To cope with uncertainty, one can figure an arbitrary cutoff period, adjust the discount rates, or simply pick a subjective probability. A number of recent articles (627,564) recommend using sensitivity analysis (627):

> . . . In this method, the most uncertain features and assumptions in the cost-effectiveness calculation are varied one at a time over the range of possible values. If the basic conclusions do not change when a particular feature or assumption is varied, confidence in the conclusion is increased. If, instead, the basic conclusions are sensitive to variations in a particular feature or assumption, further research to learn more about that feature may be especially valuable . . .
>
> . . . Examples of sensitivity analyses that are often useful are to vary the estimates of the degree of clinical efficacy of the procedure in question, to vary the weights assigned to various quality levels in computing quality-adjusted life expectancy, and to test a range of discount rates, say, from *O* to 10 percent per year.

The Problem of Using Proxy Goals and Measures

Another problem comes from the use of proxy goals and measures that may bias the number and variety of options for action. Fein's cites as an example measuring the health of children by counting school days missed (194). A program designed to improve children's health so that they miss fewer days, he argues, is different from one that focuses so heavily on reducing days of absence that its success results in sick children's being sent to school.

In fact, some argue that one of the deficiencies of CBA is that it is a complete exercise in proxy measures, since, as Fein himself points out, monetary benefits are only a part (and in some cases a small part) of all benefits, and they do not represent a stable or constant fraction of all benefits. This problem has been obvious at least since Lemuel Shattuck's 1850 sanitation study in Massachusetts (559). By spending $3,000 on sanitation planning and technical assistance, he argued, the State would ultimately gain at least $7.5 million in productivity for the 6,000 unnecessary deaths averted by efficient sanitation. But Shattuck also understood, and said, that economic benefits were not the only ones that sanitation measures would yield. In addition, such measures would increase public happiness (194), CEA is an attempt to sidestep such problems, particularly through the concept of QALYs (627,563).

For Fein, the choice of data is very important because budget officials will tend to focus on those outcomes that have numbers, or more specifically, economic values, attached to them (194). Programs that affect future productivity thus come to be overvalued because they can be quantified, and programs that relieve pain and suffering but do not affect productivity come to be undervalued. According to Fein (194):

> It may, indeed, be that programmed addressed to disabling conditions and to disease involving mortality, rather than to conditions that do not remove the person from economic activity, should be favored. That conclusion, however, should not be reached primarily because some things can be measured while others cannot. The analyst may discount the nature of the difficulty and the likelihood that this might occur, believing that his description of the items (particularly, benefits) that cannot be measured will suffice to alert the decision-maker to the inadequacy of the numbers. I suggest, however, that the analyst may underestimate the problem. He would do well to consider how compelling numbers are to finance officials

and how high a rate of discount is applied to words, however well-turned the phrases may be.

In an article on the Christian ethics of CBA, Denys Munby argues that the technique attempts (416):

> . . . precisely to take account of all the "social costs" that social reformers have (rightly) accused the pure market economies of ignoring . . . . Money is merely an abstract measuring rod; there is no other so comprehensive. It is not the use of money symbols that can be criticized, but the actual valuations have to be examined to see whether they correspond to the way we think things should be valued.

The Problem of Deciding on Inclusions and Exclusions in an Analysis

Klarman points out the great difficulty in deciding which factors to include and which to exclude in an analysis, and suggests that this problem is particularly acute in the health field (317). Other analysts agree. Grosse notes that the HEW study of the costs of automobile accidents and their prevention did not calculate the cost of seat belts (265), but he does not specify why. That particular omission seems very puzzling. Similarly not figured into the analysis was the possibility that lower auto insurance rates for everybody could result from a successful injury prevention program.

In genetic screening, where many CBAS have been performed, similar puzzlements crop up. A number of cost-benefit studies have been conducted on newborn screening for phenylketonuria (PKU), a very rare metabolic disorder leading to severe mental retardation (79). Mental retardation can be partially prevented by putting babies with PKU on a special diet shortly after birth. On paper, PKU-screening programs for newborns have been demonstrated over and over again to save society money by reducing the number of retarded people who need to be institutionalized.

But not one of the analyses of PKU-screening programs has included in its calculations a major long-term cost—that of finding and putting back on the special diet during pregnancy young women with PKU whose children will otherwise be severely retarded, Formerly, women with PKU lived in institutions and did not reproduce. As a result of the special diet initiated after birth, today many women who were born with the disorder Iead normal lives and have children.

The cost of a 20-year or longer followup for these women is enormous, and yet it is justified on both practical and ethical grounds. Having saved young women born with PKU from severe retardation, the state wants to prevent retardation in their offspring for which it might otherwise have to bear financial responsibility. In addition, it can certainly be argued that Government also has a moral obligation, once a public policy has been instituted, to help those people who are affected deal with new problems that arise in their lives as a result of State intervention.

The Problem of Infinite Externalities

Boulding points out that almost everything we do turns out differently from what we expect; both the bad and good are often unintentional (67). So, it is not surprising that a major problem with assessing cost and benefits are so-called externalities, otherwise known as external effects, neighborhood effects, side effects, spillover effects, or spillovers. Externalities include such things as the effect of building a road on esthetic sensibilities, on noise and pollution, on loss of life as a result of increased traffic accidents, and so forth.

What characterizes all these effects, Mishan says, is that they are unintentional and not subject to control by the people who experience them (at least not unless there is *some* way such people can spend money to avoid them) (410). Mishan suggests that the number of external effects in the real world is virtually unlimited. He believes society is obliged to limit them for the economist.

The number of effects that can be internalized into the pricing mechanism, Mishan says, is limited. According to Mishan, the costs of spillovers such as traffic noises, pollution, radioactive waste, and diseases of the nerves, heart, and stomach caused by high-tension living cannot be internalized. Internalization would require that the potential victim of the spillover have a legal property right to some measure of quiet or clean air, freedom from tension, and so forth. For that right to be enforceable, it would be necessary to delineate a territory around each individual that belongs solely to that person, so that an intrusion subject to legal compensation procedures could be identified. Since it is impossible to create a market, even an artificial one, that would make pricing them possible, such spillovers must ultimately be evaluated by the victims' subjective estimate.

Deriving his argument from John Stuart Mill, a 19th century British disciple of Bentham, Mishan thinks compensatory sums should actually be paid to victims of spillovers. He rejects the social engineering approach to spillovers which seeks to formulate tolerance levels for society (410):

> If the liberal economist rejects social engineering norms such as "tolerance level, " it is not merely because the choice of such a level for society is necessarily arbitrary, but because the adoption of such toler-

> ance norms on behalf of all members of society runs counter to the doctrine that each man is deemed to be the best judge of his interests, particularly in matters that affect him intimately.

A good portion of Mishan's book is devoted to a discussion of possible compensation and legal liability in such situations.

Prest and Turvey argue that CBA is not relevant to decisions on large investments because such large investments spill over so much that they may end up altering the whole economy (487). The example they use is the building of a dam in an underdeveloped country. Prest and Turvey's argument, however, might apply equally well to health care.

Grosse recounts that HEW's study of the possibilities for comprehensive health care programs for young children looked good enough that Congress became very interested (265). It was clear, however, that if children who then lacked access to good medical care were to be provided with conventional pediatric services, an acute shortage of doctors would result. Ways had to be found to use medical manpower more efficiently. Thus, the Social Security Amendments of 1967, which provided for early case finding and treatment of birth defects and chronic conditions in children, also provided for a research and demonstration program to train and use physicians' assistants.

Training physicians' assistants is an example of a side effect, because such training was not counted as a cost in the original HEW study. Nor was it anticipated how a CBA of this sort, translated by law into public policy, can influence the practice of medicine. This analysis led fairly directly to an increase in interest in physicians' assistants, which may change the whole hierarchical structure of medicine in the United States. It is not clear whether such a development should be counted as a cost or a benefit, or possibly a bit of each. Ordinarily, however, a massive (usually entirely unforeseen) ultimate effect such as this simply does not figure into the calculations.

The Problem of Pricing the Unpriceable

Finally, there is the stubborn problem of valuing intangibles, or pricirig the unpriceable. In discussing intangible costs like pain, discomfort, and grief, Klarman notes that one difficulty in valuing them is that they accrue partly to patients, but also partly to their friends, relatives, and to society (317). One way of valuing such intangibles is to ask what an individual would be willing to pay to avoid them. But Klarman believes the measurement problems here are major. Although he describes a number of different ways such intangibles have been valued, he is not very enthusiastic about any. Mishan points out that Klarman himself, when he did his 1965 calculations on syphilis, attributed more than 40 percent of the final benefit to "reduction of stigma, " which was valued (essentially arbitrarily) at either 1 or 0.5 percent of earnings subsequent to the discovery of syphilis (410).

Parish, along with most other commentators, also discusses the difficulty of valuing intangibles (475). It is true, says Parish, that such valuations are often impossible. Further, he notes (475):

> . . . ingenious, indirect means are often unconvincing and tend to discredit benefit-cost analysis. Also objectionable is the practice of finely calculating the more easily quantifiable elements while ignoring or drawing insufficient attention to the intangible ones. Indeed, this may be a more heinous event than to attempt to heroically quantify the unquantifiable.

Because intangibles are not sold in markets where consumers are forced to reveal their preferences, says Veatch, the willingness-to-pay principle is difficult to apply to them (603).

The Fictional "Facts"

All of these quite genuine measurement difficulties mean that CBA can never really tell the unvarnished truth, and that it is therefore at bottom a kind of systematic misrepresentation of the world it purports to measure. Self goes so far as to accuse cost-benefit analysts of (556):

> . , . unwittingly or not, playing a confidence trick with the symbols of monetary exchange. Of course the theoretical welfare economist is not a confidence trickster, in fact he is often high-minded, but he is committed to the discovery of some ideal ("optimum") set of economic conditions that transcend the ordinarily market economy, with the aid of which he can measure intrinsic value.

Many commentators believe that fact saddles economists with a heavy moral responsibility, and speak of it in exactly such terms. Mishan, for instance, ultimately characterizes CBA as horse-and-rabbit stew, the rabbit representing costs and benefits that really can be quantified, and the horse representing other considerations, including environmental spillovers (410). *"NO* matter how carefully the scientific rabbit is chosen, the flavor of the resulting stew is sure to be swamped by the horse flesh, " he concludes. Economists should resist the temptation to ignore the horse.

Implications of the Bias of Science Toward Rationality

Both distributional considerations and the truth or falsity of the assigned values in CBA are value/ethical problems with which many of the advocates of such analysis are all too familiar and with which they

are constantly struggling. Another value issue is more subtle and does not appear to have been previously identifed, no doubt partly because it underlies not just CBA, nor even economics, but the whole ethos of the scientific enterprise. That is the bias of science toward knowledge as a good for its own sake. This bias values "rational" decisions (usually defined as decisions made on the basis of "fact") over "intuitive" ones (usually characterized by a "gut feeling").

As Charles Lindblom and David Cohen emphasize, policy analysts tend to believe that all problems are best solved by gaining more information (361). Over and over again, CBA is justified not on grounds that it is accurate or fair, but that it represents information, and in that sense is and will always be preferred to the prior state of ignorance. Nobody puts the argument more succinctly than Prest and Turvey, who note that CBA forces those who conduct it to quantify costs and benefits insofar as possible (487):

> . . . rather than rest content with vague qualitative judgments or personal hunches. This is obviously a good thing in itself; some information is always better than none.

Mancur Olson attributes the quest for rationality not just to science in general, but to economics in particular (469):

> . . . Economic (more precisely macroeconomic) theory is in a fundamental sense more nearly **a** theory of rational behavior than a theory of material goods.

Boulding grants that the dollar "is a dangerously imperfect measure of the quality of human life and human values" (67). Nevertheless, he says (67):

> . . . it is a useful first approximation, and in these matters of evaluation of different choices it is extremely useful to have a first approximation that we can then modify. Without this, indeed, all evaluation is random selection by wild hunches.

Layard points out that all the writers in his edited volume assume that knowledge is better than ignorance (349).

Fein, who acknowledges serious problems in accurate valuation, nevertheless concludes "these problems leave us no worse off than we are in the absence of the evaluation effort" (194). In fact, he says, our awareness of the problems may leave us better off (194):

> . . . the cost-benefit analysis leads to a greater level of understanding of the deficiencies in our measuring techniques, of the vagueness of some of our goals. It does not make us ignorant but makes us aware of our ignorance. It forces us to question the "conventional wisdom" . . .

Although making comparisons between people or diseases is distasteful, Fein argues, such comparisons are being made all the time anyway, It is preferable to make the comparisons explicitly so that we can be aware that we are valuing the lives of airplane passengers, for example, more highly than those of coal miners.

Indeed, several writers justify CBA by arguing it makes us face squarely the moral implications of valuing different people's lives and preferences differently. Parish observes (in connection with the willingness-to-pay principle and the difficulty of valuing intangibles not sold in markets) that even to point out these difficulties is useful because it reveals an area of ignorance (475).

Mishan views the economists doing CBA as having a much more active role than that of a simple technician (410). To him, the cost-benefit analyst is a moral agent with an obligation to incorporate some of the deficiencies revealed by the analysis into policy formulation. Even when analysts cannot bring some important but unquantifiable item into the calculus, they can at least clearly reveal an area of ignorance. They can also help valuation by providing a physical description of the spillovers and their significance and offering a guess or range of guesses about the value of the damage. Analysts can also provide contingency calculations and estimate the critical magnitude for the spillovers that will just offset a project's benefits. They can even use questionnaires, says Mishan (410):

> Surveys based on the questionnaire method maybe suspect for a number of reasons, but they are sometimes better than guesswork, and assuredly better than no information at all.

Again and again, in discussion after discussion, commentators state the better-than-nothing argument. Despite these expressions of faith, the question remains whether revealing our ignorance will, in fact, force us to face and remedy it. Grosse observes that the HEW study of maternal and child health programs was a difficult one to do because hard information on the state of children's health was difficult to obtain (265):

> Surprisingly, estimates of improvement in general health attributable to medical care are almost nonexistent. It is not easy to demonstrate statistically that children who see doctors regularly are healthier than children who do not.

This statement is an indication of what we can probably take to be Grosse's belief that visits to the doctor do indeed improve health; he is frustrated only by his inability to demonstrate that statistically. The alternative view, both plausible and increasingly fashionable, is that visits to the doctor do not contribute appreciably to the general health of children. That possibility appears not to have occurred to Grosse,

though entertaining it might certainly give policymakers quite a different view of what kinds of priorities to accord to health care.

One of the most extensive criticisms of this rationalism-oriented mind set has been lodged by Veatch. He argues that systems analysis, rationalized centralized planning, and quantification are not value-neutral, but are instead attractive to people who have certain values (603). Veatch criticizes systematic, data-based analysis of policy problems on two grounds. One is practical— he has doubts about its outcome. So many things in practice turn out to be unquantifiable, Veatch says, that it may be better not to pursue such analysis at all because the possibility of error may be great. Furthermore, Veatch says, systematic, data-based analyses of policy problems are contrary to a sense of human freedom or spontaneity and are overly rationalistic (603):

> It is sobering to realize that basic lifestyle choice is at stake here. One cannot systematically or rationally prove that systematic, rational policy analysis is a preferable style of living in a social community.

For Lindblom and Cohen, the problem is not style but substance (361). They believe that more knowledge will be of little use in many situations and that many problems are best solved "interactively," that is, by means of negotiation or the political process. Even when knowledge is of value, it is usually only one element in a more complex political and human situation. Moreover, they argue, interactive problem solving can be just as rational in its methodology as the "scientific" approach.

Economics, like the rest of science, however, is tied irrevocably to the latter. As Boulding observes (67):

> The fundamental principle that we should count all costs, whether easily countable or not, and evaluate all rewards, however hard they are to evaluate, is one which emerges squarely out of economics and which is at least a preliminary guideline in the formation of the moral judgment, in what might be called the "economic ethic. "

Boulding freely acknowledges that some things simply cannot be measured, particularly what he calls the "heroic ethic" (bravery in the military, religious feelings, and so forth) (67):

> The attack on economics is an attack on calculatedness, and the very fact that we think of calculating as cold suggests how exposed economists are to romantic and heroic criticism.

The problem is to retain both heroic and economic elements in our institutions and in our decisionmaking, but in proper balance (67):

> Economic man is a clod, heroic man is a fool, but somewhere between the clod and the fool, human man, if the expression may be pardoned, steers his tottering way.

Though most instinctively prefer even a flawed systematic analysis to none, it is well to keep Fein's warning in mind (194):

> While it is better to know something than to know nothing . . . we dare not minimize the danger that in knowing something we may behave as if we know everything.

Implications of the Conflict Between Freedom and Paternalism

The conflict between freedom and paternalism is one not much discussed in the literature on CBA. But individual freedom is an issue particularly important in the United States, and paternalism one particularly important in medicine. The conflict between freedom and paternalism is thus a perennial one in U.S. health care. Clearly, CBA, with its quantitative methods and scientific respectability, can force choices.

Nevertheless, it is anything but clear that CBA has done this. One of the early analyses performed at HEW and described by Grosse (265), for example, examined the value of fluoridation of drinking water to help reduce cavities. Grosse says that on paper (265):

> . , . fluoridation looks like a very attractive program. It was so attractive that one can assume that a program as cheap as this is not bring inhibited by a lack of financial support by the Federal Government; there are other factors at work.

He does not specify the nature of those other factors, but most of us know they are political. There is strong local opposition to fluoridation of water, often on grounds that it is simply wrong to force everybody in a community to undergo a medical treatment with no way of opting out.

Public policy, particularly health policy, has often been criticized as paternalistic. The Government is often accused of meddling in people's lives, trying to get them to behave in certain ways for their own good. CBAS are, in a way, a response to that criticism. Today, health programs are justified less on grounds that the Government ought to be doing good things for its citizens than in terms of the tax savings and other economic benefits that will result. Thus, screening newborns for PKU is no longer (or rarely) supported on grounds that families ought to be spared the miseries of having to cope with a retarded child who need not have been so retarded, but rather on grounds that the Government will save money on institutional costs for the retarded by underwriting such a program.

It is probable, however, that in a way CBA functions partly as a mask for the old paternalism. The extensive analyses of genetic screening, for instance, offer a respectably scientific and economic justifica-

tion for continuing programs whose origins are actually traceable to a combination of political and humane impulses. This illustrates that the important problem here may not be the paternalism of economists, but that of the policy maker. The Government policymaker orders and pays for the analysis. Because of this, the policy maker will often have much to say about what goes into the analysis, for instance, concerning goals and outcomes. Furthermore, the policy maker is also in a position to apply the analysis to the making of social decisions. This position not only invites paternalism, it almost demands it, since in some sense policymakers are by definition in a somewhat paternal role with respect to the people for whom they make policy.

Of course, a CBA can also be influenced by the degree of paternalism subscribed to by economists. For example, many analyses employ shadow pricing in which, for a variety of reasons, the value of some item in the analysis is set at something other than its current market value. As Layard points out, highly paternalistic economists may very well substitute their own valuation for that revealed by answers to survey questions if they believe that the public's valuations may not be accurate—particularly when valuing future possibilities (349).

Conclusions

The issues of equity, efficiency, freedom, paternalism, the value of knowledge, and rationality—by truth-telling and deception—are irretrievably imbedded in the techniques of CEA and CBA. There is no way to change the techniques to eliminate those values and their inevitable conflicts. Certain technical adjustments (e. g., putting the services of housewives into dollar terms) have been made in the past, and these have improved the situation somewhat. Similar adjustments will continue to be made in the future, but essentially they will be small adjustments that will have no effect on the fact that the issues described are simply inherent in the techniques themselves or in their translation into policy.

Should we not use cost-benefit techniques in forging health policy? No, discarding CBA is not the answer. In an important sense, the defenders of the technique are correct when they argue that policy decisions in health care are being made daily on shaky grounds anyway. CBA is at least an attempt, however imperfect, to ground those decisions in real needs and real possibilities.

The problem is not that CBA lacks objectivity and is not value free, but rather that objectivity and value freedom are unjustifiably attributed to it. To their credit, most theorists of CBA cited here are quite aware that their techniques are anything but objective and value free. The problem comes with lesser practitioners who see themselves simply as technicians or—and this may be the real problem—with policy makers who take the scientific aura of CBA for truth, failing to realize CBA's limitations.

Many, discussions of CBA have paid little attention to policymakers. Yet, virtually all analyses are undertaken at the behest of policy makers, and the methods employed in a specific analysis may depend on their goals and values. Weinstein and Stason point out, for instance, that a health maintenance organization may care about total costs, whereas a group practice may care chiefly about nonreimbursable costs; such differences have obvious and important implications for society's efforts to contain costs (627).

To understand the pros and cons of CBA studies, one must also understand the policy level on which they are commissioned and applied. Who is the policymaker? The Secretary of DHHS, the Senate Subcommittee on Health and Scientific Research, the State health department, the health systems agency, the hospital administrator, the school nurse, the private physician? Each of these policy makers may very well use CBA to make policy. The decision may be different in every case, however, because individual policymakers, like economists, will have personal values, institutional biases, and a somewhat different constituency from others'.

Harold Green notes that optimism or pessimism can sometimes be "a function of statutory mandate or agency mission" (258). The Food and Drug Administration, for instance, automatically regards any food additive as hazardous until it has been demonstrated to be safe. The importance of the policymaker's perspective is illustrated, as well, by the unsettled question of whether a physician ought to depart from concern for the patient's welfare and begin to incorporate social values into the decision to, for example, order an additional test that has only very small chance of giving additional information.

For some analysts, the question of who the policymaker is does not seem to be very important. If as an analyst you follow Mishan's recommendations (410), for instance, you will behave quite explicitly as a moral agent, with a duty to point up all the relevant effects of a program that do not enter the cost-benefit computation no matter who your employer is. Mishan also argues that if economists have reason to believe that a project will be unambiguously regressive, it is their duty to mention this; for Mishan, economists should be just as committed to equity as to efficiency. That commitment should obtain, he implies, regardless of the level on which policy is being made.

It is not clear whether Mishan's view is widely shared by economists, or whether instead most economists see themselves as simple hired hands. At a minimum, though, it seems sensible to assert that there are some kinds of information that ought to be made explicit as part of a CBA. These include clear indications of the data sources on valuing lives, what kinds of equity considerations spring from the use of particular data (e.g., whether whites are valued more highly than blacks), and how and why the discount rate was selected.

Conventional assumptions, disclaimers, and cautions also ought to be an explicit part of each CBA. Greater use of sensitivity analysis would probably be illuminating. In addition, people need to be made more aware of the limitations of CBA. That probably includes people within the economics profession itself, certainly includes policymakers who use CBA, and may even include the general public.

CBA should certainly never be used as the sole basis for major health policy decision. Such use, however, may not be as much of a problem as has often been charged. Grosse for instance, says the HEW analyses were used to give the Department a "feel for what were relatively high- and low-priority programs, and then to feed these insights into the decisionmaking process, which also considers other viewpoints, the existing commitments, the political situation, the rate of spending, and the ability to get people moving on programs, and so on" (265). In Grosse's view, CBA is clearly a useful tool, but only one of many.

Swint and Nelson apparently share this view, observing (593):

> Many of the criticisms of (cost-benefit analysis) come from the people who have (perhaps inadvertently) set up a "straw man" by claiming it cannot provide the sole decision-making criterion. The point is that (cost-benefit analysis) is not intended to provide the sole decision-making criterion; that has only been incorrectly imputed to it by non-practitioners. What it does do is provide economic information that must be combined with distributional, sociopolitical, humanitarian and other information by the decision maker (vis-a-vis the analyst) for net evaluation. We see no point in depriving decisionmakers of one piece (the economic) of the several sets of information needed to make rational decisions.

For Prest and Turvey, who were writing, after all, in the comparatively early days of 1965, CBA is "only a technique for making decisions within a framework which has to be decided upon in advance and which involves a wide range of considerations, many of them of a political or social character" (487). They argue that the case for using CBA is strengthened, not weakened, if its limitations are openly recognized and emphasized. CBA may then function as a "sensible antidote to the wilder excesses of particular lobbies. " CBA may also cause questions to be asked that would not otherwise have been raised. Prest and Turvey also agree with Grosse that even if CBA cannot always give the right answers, it can sometimes play the purely negative role of screening projects and rejecting those that are clearly less promising (487,265).

On the other hand, one should certainly not underestimate the power of politics. Klarman points out that politics is one of the foremost barriers to systematic analysis (265). CBA implies a delineation of goals and an articulation of values, whereas the political process, he says, may require the blurring of differences and conflicts in order to facilitate coalition building so that particular ends can be achieved. According to Martin Rein, because of that requirement of politics, it is necessary to put planning beyond the reach of shortsighted political considerations: ". , . at some stage, and to some degree, decisions must be taken out of the political context and handed over to the social scientist for analysis" (501). Real exploration and analysis of alternative choices, he suggests, can occur only in the absence of short-term political pressures.

How decisions can be taken out of the political context for analysis is not clear. Nor is it entirely clear that they should be. Green argues that sometimes nonscientific negotiation and compromise may be the best course for policymakers (258,361). He asks (258):

> Is it not more important to resolve controversy in the optimum manner than to produce an objectively correct result? If so, is it not possible that optimum resolution of controversy may require acceptance as true of something that we know is untrue?

Even some of CBA's best-known and most sensitive practitioners acknowledge that, at times, the political process may respond better to our needs than even the most rational and careful CBA. Rashi Fein believes in CBA. He thinks that policymakers need to be reminded that there are economic returns to health programs, that good health can be supported on investment grounds, and that poor health costs a lot. On the other hand, Fein comments (194):

> At present, in the United States, beset as we are by divisions and by tensions, distributional considerations lie at the heart of many of our problems. The healing of social wounds (not an unimportant objective even if its benefits cannot be quantified in monetary returns) may, today, be more readily accomplished by providing the services that people believe to be important than by providing that which the analyst has tentatively determined is most beneficial. The healing of social wounds, is, at this moment, I believe more vital than the healing of disease.

Appendix E.— Legal Status of CEA/CBA

Most of the existing statutory authorities that guide Federal health-care-related agencies allow the agencies to use or support CEA/CBA. Recently, however, two legislative initiatives—Public Law 95-623 establishing the National Center for Health Care Technology (NCHCT) and the National Health Planning and Resources Development Amendments to Public Law 93-641—placed special emphasis on the use and support of CEA by NCHCT and by health systems agencies (HSAS). Although these laws are not mandates, they are explicit suggestions to do CEA or CBA studies. The language of Public Law 95-623 calls for NCHCT to "give appropriate emphasis" to cost effectiveness (among a number of other criteria) while conducting and supporting research on health care technology. Similarly, the health planning amendments state that cost effectiveness should be one of the criteria that HSAS use while carrying out their mandate to review the appropriateness of existing facilities (see ch. 7 for a detailed discussion). These two laws are as close as Congress has come to requiring a health-care-related agency to perform or support CEA or CBA studies as part of its mission.

At the other end of the legislative spectrum from these two laws are the statutes that authorize and guide the efforts of the Food and Drug Administration (FDA). FDA has traditionally interpreted its mandate as excluding or strongly discouraging the formal evaluation and incorporation of economic costs and social benefits in its regulatory proceedings (127a). Although, FDA has done a small number of costs and benefits type comparisons of proposed initiatives, those analyses were conducted in response to executive orders calling for: Inflationary Impact Statements (E.O. 11821, 1974), Economic Impact Statements (E.O. 11949, 1977), and Improving Government Regulations (E.O. 12044, 1978). Those orders applied to executive agencies that proposed major regulations, i.e., regulations having an annual impact on the economy of $100 million or more or causing a major increase in costs or prices for individual industries, levels of government, or geographic regions. Any agency that proposed a regulation falling into one of these categories was required to examine the costs and benefits of the proposed action.

One section of the food additive amendments to FDA's mandate, usually referred to as the "Delaney clause," (The Food, Drug, and Cosmetic Act, 21 U. S. C., sec. 409c3a, 1976) explicitly prohibits the balancing of risks and benefits in a proposed regulation of a food additive that has been shown to be carcinogenic (127a). The inflexible language of this amendment has stimulated a number of legislative proposals to repeal or alter the "Delaney clause," and/or alter FDA's authorizing legislation to allow or require the balancing of risks and benefits in proposed regulatory or rulemaking initiatives. [1] The most prominent of the initiatives is the bill introduced in the 96th Congress as the "Drug Regulation Reform Act of 1979."

The legal status of CEA and CBA in the decisionmaking processes of other agencies of the Federal Government is in a much greater state of conflict, confusion, and flux. In the past 10 years, numerous court rulings have attempted to define and clarify the role of CEA and CBA in the decisionmaking processes of regulatory agencies. Increasing congressional attention is being focused on the potential usefulness of CEA and CBA techniques, especially as they pertain to the decisionmaking efforts of several regulatory agencies. Over 65 bills were introduced in the last two Congresses that would require various agencies to incorporate CEA, CBA, or risk-benefit techniques into their formal decisionmaking procedures (600). For many agencies, the legislation would simply formalize a CEA/CBA process that is now informal. In a number of other agencies, a formal or informal process of using CEA/CBA does not exist. In any case, many of the proposed changes would make explicit that which past legislation has left vague and open to judicial and agency interpretation.

In the past decade, a number of health, safety, and environmental laws have added to the Federal Government's involvement in the regulation of highly controversial areas of society and business. [2] In many instances, the specific purpose and intended outcomes of the legislation were inadequately defined after the general goals were established. In addition, the types of procedures allowed or required to initiate agency action, or the level of "evidence" required to support a given initiative, were left vague in the authorizing legislation. In many cases, these things were left vague with good reason. The uncertainties

[1] Most of the reaction was stimulated by the use of the "Delaney clause" to initiate a ban of saccharin, an artificial sweetener used extensively in this country. For a detailed analysis of the technical issues involved in the proposed saccharin regulation, see *Cancer Testing Technology and Saccharin* (466), prepared by OTA.

[2] Included among these laws are (127a): 1) Safety and Health Act (Public Law 91-596); 2) Consumer Product Safety Act (Public Law 92-573); 3) Amendments to the Pesticide Act (Public Law 92-516, Public Law 94-140, Public Law 95-396); 4) Solid Waste Disposal Act (Public Law 94-580); 5) Toxic Control Act (Public Law 94-469); 6) Medical Device Amendments (Public Law 94-295); 7) Safe Drinking Water Act of 1977 (Public Law 95-190); 8) Clean Air Act Amendments of 197? (Public Law 91-604, Public Law 95-95). Perhaps the most well known is the National Environmental Policy Act of 1969— NEPA (U.S.C. sec. 4321 et seq., 1970). It has been responsible for a significant number of legal challenges regarding the decisionmaking methods of a host of Federal agencies.

involved in the issues, the imprecise nature of the best available data, and many other factors required that the authorizing statutes remain flexible.

The broadly worded statutory language, however, has left the door open for procedural challenges, differing interpretations of the laws, and litigation. The practical result of this problem is that the courts have been repeatedly forced to clarify and define what is required of the agencies in establishing a rule or regulation. Numerous court cases have challenged the decisionmaking procedures of the Environmental Protection Agency, the Consumer Product Safety Commission (CPSC), the Nuclear Regulatory Commission, the Occupational Safety and Health Administration (OSHA), and others.[3] One of the results of this litigation is an increasing pressure on the agencies to include more formal and explicit costs v. benefits comparisons in their decisionmaking and evidence-gathering procedures.

Neither the courts nor Congress are totally unfamiliar with CEA or CBA techniques. The Federal courts have been concerned with agency actions using costs and benefits since the early 1940's (526). Congress has required the Corps of Engineers to justify water resource projects with cost-benefit calculations since the late 1930's. Recent years, especially the last 10, however, have witnessed an increasing awareness of the use of CEA/CBA in the regulatory decisionmaking process. Enactment of the National Environmental Policy Act (NEPA) in 1969 and the issuance of the executive orders mentioned above marked the beginning of significant judicial involvement in the area of procedural and substantive review of CEA/CBA use in the regulatory process.

Despite a decade of litigation, conflict, and debate, the role of formal CEA/CBA in the regulatory process still remains ill-defined and unclear in many, if not most, decisionmaking situations. The courts have had, and continue to have, a difficult time in producing a coherent body of law that establishes uniform standards of judicial review in this area (this is especially true for NEPA-related issues). They have not been able to reconcile the conflicting standards that have been placed into various statutes that indirectly require many agencies to balance the social costs and benefits of their regulatory initiatives (526). The courts review agency initiatives with three basic criteria in mind: 1) Did the agency conform to the procedures set out in the law? (the procedural test); 2) Was the cost-benefit comparison arbitrary or capricious~ (a substantive and due process test); and 3) What is the "substantial evidence on the record as a whole?" (also a substantive test). Putting these criteria into practice has not always been easy or successful. The limitations of the methods of CEA/CBA, the vagueness of the legislative language and intent, the differing interpretations of the level of sophistication or rigor required in the cost-benefit comparisons, and the reluctance of the courts to substitute their judgments for agency expertise have contributed to the continuing confusion that surrounds the use of formal CEA/CBA techniques in the decisionmaking process.

A number of recent and pending Federal District Court rulings may be able to give further indications of the direction in which the courts are moving with respect to requiring CEA or CBA techniques in the decisionmaking process. One should keep in mind, however, that the courts' opinions may be specific to an agency, may be subject to later reinterpretation by a higher court, or may be applicable only to the specific statutory wording on which the rulings turn. OTA is not suggesting that these opinions will have significant impact on the health-related agencies examined in this assessment. As judicial signals that may very well broadcast to a number of executive agencies the extent to which formal CEA/CBA techniques might be used or required in their decisionmaking procedures, however, these opinions are worthy of note.

The litigation many observers may be watching most closely is the "benzene case" now before the Supreme Court.[4] That case involves a number of issues, but the primary focus is on two questions: 1) Is OSHA required to use a costs v. benefits comparison to support its proposed standards? and, 2) If so, is the analysis OSHA claims to have performed adequate to support its decision? A lower court answered yes to the first and no to the second, and overturned OSHA'S proposed benzene standards for the workplace (20). The lower court held, in part, that OSHA had failed to properly or adequately compare the benefits expected from the proposed regulation to the anticipated costs of compliance to determine if a "reasonable relationship" existed.[5]

[3] This appendix does not attempt to provide a comprehensive discussion of the development and interpretation of the case law that guides this area of regulatory behavior. At best, an attempt is made to touch on the recent judicial highlights that have built upon earlier rulings. For a more complete analysis of the courts' involvement in this area, see references **41, 42,** and **526.**

[4] *1ndustria/ Union Department, AFL-C1O v. American Petroleum, et al. No. 78-911)* and *Marshall u American Petroleum Institute, et al. (No. 78-1036).*

[5] The language in OSHA'S mandate (Occupational Safety and Health Act, 29 U .S.C. sec. 652 et seq. (1975)) requires that OSHA has the obligation to enact only standards that are "reasonably necessary" or appropriate to provide safe or healthful workplaces and that standards dealing with toxic materials be "feasible, " OSHA must determine whether benefits expected from a standard bear a reasonable relationship to costs imposed by the standard. The Circuit Court rulings in the CPSC and OSHA cases hinge on these phrases; "reasonably necessary, " "feasible," and whether benefits expected bear a "reasonable relationship" to the costs expected.

Circuit Court rulings involving CPSC were cited by the court in the benzene case to illustrate the evidence requirements that the court felt are necessary to determine if a proposed initiative is "reasonably necessary" when the proposed standard's benefits are balanced against the costs, or burden, of compliance with the standard. [b]The Supreme Court will determine if OSHA'S statutory language requires it to go beyond the data it has used in the past and follow the guidelines established by the CPSC rulings for the use of costs v. benefits comparisons in its decisionmaking procedures.

The available evidence strongly indicates that there is mounting legislative and judicial pressure to formalize the use of CEA/CBA in many decisionmaking areas in the Federal Government. To date, most of this pressure has been focused on the regulatory agencies. It is uncertain at this time if these pressures will expand into the health care system.

[b]The rulings cited in the OSHA opinion as guiding the determinations were:

1. *Aqua Slide 'N' Dive* v. *Consumer Product Safety Commission*, 569 F. 2d. 832 (5th Cir. 1978). and
2. *D. D. Bean & Sons* v. *Consumer Product Safety Commission*. 574 F. 2d 643 (1st Cir. 1978).

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Appendix F.— Description of Other Volumes of the Assessment

In addition to the present report, several background papers were prepared in conjunction with OTA'S assessment The Implications of Cost-Effectiveness Analysis of Medical Technology, and these are available separately. The findings and options of the assessment are in large part derived from the information generated by the background efforts.

Background Paper #1: Methodological Issues and L.iterature Review, includes an in-depth examination of the decisionmaking context and methodology discussions presented in this report. A critique of the literature, a bibliography of over 600 items, and abstracts of over 70 studies and other articles are also included.

In order to investigate 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 in a collected volume, Background Paper #2: Case Studies of Medical Technologies. Some of the cases represent formal cost-effectiveness analysis (e.g., the case on bone marrow transplants), while others represent net cost or "least cost" analysis (e.g., the case on certain respiratory therapies). Other cases illustrate various issues such as the difficulty of conducting cost-effectiveness analysis 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
  Nancy Barrand
  Lu Ann Heinen
Nurse Practitioners
  Lauren LeRoy
  Sharon Solkowitz
Orthopedic Joint Prosthetic Implants
  Judith D. Bentkover
  Philip G. Drew
Periodontal Disease Interventions
  Richard M. Scheffler
  Sheldon Rovin
Selected Respiratory Therapies
  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 cost-effectiveness and cost-benefit analysis 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 for OTA 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:

United Kingdom
Barbara Stocking
Canada
Jack Needleman
Australia
Sydney Sax
Japan
Joel Broida
France
Rebecca Fuhrer
Germany
Karin A. Dumbaugh
Netherlands
L. M. J. Groot
Iceland
David Gunnarson
Duncan vB. Neuhauser
Sweden
Erik H. G. Gaensler
Egon Jonsson
Duncan vB. Neuhauser

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 cost-effectiveness analysis was performed.

Appendix G.—Acronyms and Glossary

ACRONYMS

ADAMHA	Alcohol, Drug Abuse, and Mental Health Administration (PHS)
AHA	American Hospital Association
AIP	annual implementation plan
AMA	American Medical Association
ANDA	abbreviated new drug application
AoA	Administration on Aging
ASH	Assistant Secretary for Health (DHHS)
ASPE	Assistant Secretary for Planning and Evaluation (DHHS)
BC/BS	Blue Cross and Blue Shield Associations
BCHS	Bureau of Community Health Services (Health Services Administration)
BHF	Bureau of Health Facilities (HRA)
BHP	Bureau of Health Planning (HRA)
BHPr	Bureau of Health Professions (HRA)
BMD	Bureau of Medical Devices (FDA)
BMS	Bureau of Medical Services (Health Services Administration)
BOB	Bureau of Biologics (FDA)
BOD	Bureau of Drugs (FDA)
BOE	Bureau of Epidemiology (CDC)
BOL	Bureau of Laboratories (CDC)
BRH	Bureau of Radiological Health (FDA)
CBA	cost-benefit analysis
CBO	Congressional Budget Office (U.S. Congress)
CDC	Center for Disease Control (PHS)
CEA	cost-effectiveness analysis
CEA/CBA	cost-effectiveness analysis/cost-benefit analysis (when referred to as a class of analytical techniques)
CFR	Code of Federal Regulations
CHP	Comprehensive Health Planning
CMHC	community mental health center
CON	certificate of need
CPSC	Consumer Product Safety Commission
CT	computed tomography (scanner)
DARP	Drug Abuse Reporting Program (NIDA)
DES	diethylstilbestrol
DESI	Drug Efficacy Study Implementation (project)
DHEW	Department of Health, Education, and Welfare (now DHHS)
DHHS	Department of Health and Human Services (formerly DHEW)
DOD	Department of Defense
DOE	Department of Energy
DOL	Deparment of Labor
DRG	Division of Research Grants (NIH)
EFM	electronic fetal monitoring -
EPA	Environmental Protection Agency
EPSDT	Early and Periodic Screening, Diagnosis, and Treatment (Program) (HEW)
ESRD	end-stage renal disease
FDA	Food and Drug Administration (PHS)
FR	Federal Register
FY	fiscal year
GAO	General Accounting Office (U.S. Congress)
GHAA	Group Health Association of America
GHC	Group Health Cooperative; Seattle, Wash.
HCFA	Health Care Financing Administration (DHHS)
HEW	Department of Health, Education, and Welfare (now DHHS)
HMO	health maintenance organization
HRA	Health Resources Administration (PHS)
HSA	health systems agency
HSI	health status index
HSP	health systems plan
HSQB	Health Standards and Quality Bureau (HCFA)
IDE	investigational device exemption
IHS	Indian Health Service (Health Services Administration)
IND	notice of claimed investigational exemption for a new drug
IOM	Institute of Medicine (NAS)
IPA	individual practice association (HMO)
KFMCP	Kaiser Foundation Medical Care Plan
MCE	medical care evaluation studies (by PSROS)
NAS	National Academy of Sciences
NASA	National Aeronautics and Space Administration
r4CHCT	National Center for Health Care Technology (OASH)
NCHS	National Center for Health Statistics (OASH)
NCHSR	National Center for Health Services Research (OASH)
NCI	National Cancer Institute (NIH)
NDA	new drug application
NEI	National Eye Institute (NIH)
NEPA	National Environmental Policy Act
NHLBI	National Heart, Lung, and Blood Institute (NIH)
NIA	National Institute on Aging (NIH)
NIAAA	National Institute on Alcohol Abuse and Alcoholism (ADAMHA)

NIAID	National Institute of Allergy and Infectious Diseases (NIH)
NIAMDD	National Institute of Arthritis, Metabolism, and Digestive Diseases (NIH)
NICHD	National Institute of Child Health and Human Development (NIH)
NIDA	National Institute on Drug Abuse (ADAMHA)
NIDR	National Institute of Dental Research (NIH)
NIEHS	National Institute of Environmental Health Sciences (NIH)
NIGMS	National Institute of General Medical Sciences (NIH)
NIH	National Institutes of Health (PHS)
NIMH	National Institute of Mental Health (ADAMHA)
NINCDS	National Institute of Neurological and Communicative Disorders and Stroke (NIH)
NIOSH	National Institute for Occupational Safety and Health (CDC)
NLM	National Library of Medicine (NIH)
NSF	National Science Foundation
OASH	Office of the Assistant Secretary for Health (DHHS)
OASPE	Office of the Assistant Secretary for Planning and Education (DHHS)
ODPHP	Office of Disease Prevention and Health Promotion (OASH)
OHDS	Office of Human Development Services (DHHS)
OHMO	Office of Health Maintenance Organizations (OASH)
OHRST	Office of Health Research, Statistics, and Technology (OASH)
OMAR	Office of Medical Applications of Research (NIH)
OPE	Office of Planning and Evaluation (OASH)
OPEL	Office of Planning, Evaluation, and Legislation
OPPR	Office of Policy Planning and Research (HCFA)
OSHA	Occupational Safety and Health Administration (DOL.)
OTA	Office of Technology Assessment (U.S. Congress)
OTC	over-the-counter (Drug Review Program) (FDA)
PDP	product development protocol
PGP	prepaid group practice (HMO)
PHS	Public Health Service (DHHS)
PPB	planning-programing-budgeting
PMA	Pharmaceutical Manufacturers' Association
PSRO	Professional Standards Review Organization, or Office of PSRO (HCFA)
QALY	quality-adjusted life year
RFP	request for proposal
RMP	Regional Medical Program
SHCC	State Health Coordinating Council
SHPDA	State health planning and development agency
SSA	Social Security Administration (DHHS)
TOPS	Treatment Outcome Prospective Study (NIDA)
VA	Veterans Administration
ZBB	zero based budgeting

GLOSSARY

Ambulatory medical care: Medical goods and services rendered to a patient not admitted to a hospital or other inpatient health care facility, including such items as physician office visits, outpatient laboratory diagnostic services, and outpatient prescription drugs.

Biomedical **and behavioral research: A combination of biological, medical, psychological, social, and physical scientific investigations focused on eradicating disease and generating new scientific knowledge.**

Cavitation financing method: The method of paying for medical care on a fixed, periodic prepayment basis per individual enrolled in a health plan. Payment by "cavitation" implies that the amount paid by the individual is independent of the number of services that individual has received.

CEA/CBA: A composite term referring to a family of analytical techniques that are employed to compare costs and benefits of programs or technologies. Literally, the term as used in this assessment means "cost-effectiveness analysis/cost-benefit analysis."

Certificate of need (CON): A regulatory planning mechanism required by the National Health Planning and Resources Development Act to control

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large health care capital expenditures. CON applications by institutions are reviewed by local health systems agencies, who recommend approval or disapproval; they are denied or approved by State health planning agencies (SHPDAS).

Consensus development conference: A process in which knowledgeable biomedical researchers, practicing health professionals, and others, as appropriate, are brought together by NIH to explore publicly the scientific background, state of knowledge, proper use(s), and any other issues pertinent to the technology under consideration.

Controlled clinical trial: An experimental design by which human or animal subjects are assigned, in accordance with predetermined rules, either to an experimental group (in which subjects receive a clinical intervention or dosage level of uncertain efficacy or safety) or to a control group (in which subjects receive some other intervention or dosage level, usually the customary or conventional one, or a placebo). If the predetermined rules specify that the subjects are assigned to groups randomly, the result is a randomized controlled clinical trial, The vast majority of randomized clinical trials are also controlled trials.

Cost-benefit analysis: An analytical technique that compares the costs of a project or technological application to the resultant benefits, with both costs and benefits expressed by the same measure. This measure is nearly always monetary.

Cost-effectiveness analysis: An analytical technique that compares the costs of a project or of alternative projects to the resultant benefits, with cost and benefits/effectiveness not expressed by the same measure. Costs are usually expressed in dollars, but benefits/effectiveness are ordinarily expressed in terms such as "lives saved," "disability avoided," "quality-adjusted life years (QALYs) saved," or any other relevant objectives. Also, when benefits/effectiveness are difficult to express in a common metric, they may be presented as an "array."

Device (medical): Any physical item, excluding drugs, used in medical care (including instruments, apparatus, machines, implants, and reagents).

Discount **rate:** A factor used in economic analysis to reduce to present value those costs and effects that occur in future years. Discounting is based on two premises: 1) individuals prefer to receive benefits today rather than in the future, and 2) resources invested today in alternative programs could earn a return over time.

Drug: Any chemical or biological substance that may be applied to, ingested by, or injected into humans in order to prevent, treat, or diagnose disease or other medical conditions.

Effectiveness: Same as efficacy (see below) except that it refers to ". . . average conditions of use."

Efficacy: The probability of benefit to individuals in a defined population from a medical technology applied for a given medical problem under ideal conditions of use.

Fee-for-service: A method of paying for medical care on a retrospective basis by which each service actually received by an individual bears a related charge.

Health maintenance organization: A health care organization that acts as both insurer and provider of comprehensive medical services by a defined set of physicians to a voluntarily enrolled population paying a prospective per capita fee (i.e., paying by "cavitation"),

Health status index (HSI): A composite measure of well-being of an individual or a population, An HSI may include any two or more measures such as morbidity, mortality, level of function or mobility, and potential for developing disease.

Health services research: A field of inquiry that focuses on the structure, production, distribution, and effects of delivering personal health services.

Health systems agency (HSA): One of the local health planning agencies which are charged with developing local health planning goals and implementing plans in consonance with State and National health care goals. HSAS are federally funded and are governed by a body which is broadly representative of both provider and consumer interests, the latter being in the majority. The country is divided into areas each of which is served by an HSA.

Human capital: An economic concept used to assess "livelihood," or the earnings potential of an individual. It has often been used as a proxy for the value of life in terms of an individual's productive capacity to society.

Investigational new drug (IND) application: Short for "notice of claimed investigational exemption for a new drug." An IND application is submitted to FDA by a drug's sponsor. It is a request for permission to use the drug in humans in order to investigate the drug's safety and efficacy for specific conditions. It must include the results of toxicity studies in animals, the qualifications of the investigators, and the design of the proposed clinical studies.

Marginal benefit: An economic concept referring to the additional benefit achieved by incurring an additional unit of cost.

Marginal cost: An economic concept referring to the additional cost of achieving one more unit of benefit.

Medical care evaluation studies: Intensive retrospective reviews of patients' records to evaluate the quality, variation, and utilization of health care services delivered to the patient.

Medical technology: The drugs, devices, and medical and surgical procedures used in medical care, and the organizational and support systems within which such care is provided.

Morbidity: Illness, injury, impairment, or disability in an individual.

Mortality: The death of an individual; often used in epidemiological studies where mortality rates for a population for a certain disease or injury are calculated.

Net cost analysis: A form of CEA / CBA (see above) that concentrates on costs, with less attention paid to analyzing outcomes in terms of health benefit. When alternatives are under study, their efficacy is often assumed to be equal.

New drug application (NDA): An application to FDA by the sponsor of a new drug for permission to market the drug. The NDA must provide information that demonstrates the safety and efficacy of the drug.

Procedure: A medical technology involving any combination of drugs, devices, and provider skills and abilities. Appendectomy, for example, may involve at least drugs (for anesthesia), monitoring devices, surgical devices, and physicians', nurses', and support staffs' skilled actions.

Professional Standards Review Organization: Community-based nonprofit agencies directed by physicians that monitor the quality and appropriateness of institutional health care provided to medicare and medicaid beneficiaries.

Quality-adjusted life **year (QALY):** A health status index in which 1 year of life is adjusted for various types and degrees of disability to yield 1 year of healthy life. QALYs are sometimes used to measure in common terms the effects on morbidity and mortality of health care technologies or programs.

Risk: A measure of the probability of an adverse or untoward outcome's occurring and the severity of the resultant harm to health of individuals in a defined population associated with use of a medical technology applied for a given medical problem under specified conditions of use.

Risk-benefit analysis: The formal comparison of the probability and level of adverse or untoward outcomes versus positive outcomes for any given action. The comparison of outcomes does not take into consideration the resource costs involved in the intended action.

Safety: A judgment of the acceptability of risk (see above) in a specified situation.

State Health Coordinating Council (SHCC): The State advisory body to the State health planning and development agency (SHPDA). SHCCS consist of a consumer majority, mostly from health system agencies' representatives who are appointed by the Governor, and are responsible for approving the State health plan which is submitted by the SHPDA.

State health planning and development **agency (SHpDA):** state-level health planning organizations which are federally mandated to develop and implement statewide health plans in consonance with national goals and local planning objectives. SHPDAS must approve or disapprove health system agencies' recommendations regarding certificate of need for Iarge new capital investments.

Willingness-to-pay: An economic concept used to assess the monetary value of life in terms of what an individual is "willing to pay" to prolong life or postpone death. The willingness-to-pay technique is used to try to assess how much an individual values his or her own life. Sometimes, the technique is also used to assess how much an individual is willing to pay to decrease risk to others or to prolong others' lives.

Appendix H.— Health Program Advisory Committee, Authors of Case Studies of Medical Technologies (Background Papers #2, #3, and #5), and Acknowledgments

HEALTH PROGRAM ADVISORY COMMITTEE

Frederick C. Robbins, Chairman
Dean, School of Medicine, Case Western Reserve University

Stuart H. Altman
Dean
Florence Heller School
Brandeis University

Robert M. Ball
Senior Scholar
Institute of Medicine
National Academy of Sciences

Lewis H. Butler
Health Policy Program
University of California, San Francisco

Kurt Deuschle
Professor of Community Medicine
Mount Sinai School of Medicine

Zita Fearon
Consumer Commission on the Accreditation of Health Services, Inc.

Rashi Fein
Professor of the Economics of Medicine
Center for Community Health and Medical Care
Harvard Medical School

Melvin A. Glasser
Director
Social Security Department
United Auto Workers

Patricia King
Professor
Georgetown Law Center

Sidney S. Lee
Associate Dean
Community Medicine
McGill University

Mark Lepper
Vice President for Inter-Institutional Affairs
Rush-Presbyterian Medical School
St. Luke's Medical Center

Frederick Mosteller
Professor and Chairman
Department of Biostatistics
Harvard University

Beverlee Myers
Director
Department of Health Services
State of California

Mitchell Rabkin
General Director
Beth Israel Hospital

Kerr L. White
Rockefeller Foundation

AUTHORS OF CASE STUDIES OF MEDICAL TECHNOLOGIES

Nancy Barrand
Health Policy Program
University of California, San Francisco

Judy Bentkover
Arthur D. Little, Inc.

Peter Budetti
Health Policy Program
University of California, San Francisco

John P. Bunker
Division of Public Semices Research
School of Medicine
Stanford University

Morgan Delaney
School of Medicine
George Washington University

Philip G. Drew
Arthur D. Little, Znc.

David Eddy
Department of Engineering-Economics Systems
Stanford University

Harvey V. Fineberg
School of Public Health
Harvard University

Ann B. Flood
School of Medicine
University of Illinois

Eric Fortess
School of Public Health
Harvard University

Lu Ann Heinen
Health Policy Program
University of California, San Francisco

Michael Higgins
Division of Health Services Research
Stanford University

Carol Korenbrot
Health Policy Program
University of California, San Francisco

Lauren LeRoy
Health Policy Program
University of California, San Francisco

Deborah P. Lubeck
Division of Health Services Research
Stanford University

Bryan R. Luce
Office of Technology Assessment
Congress of the United States

Peggy McManus
Health Policy Program
University of California, San Francisco

Lawrence Miike
Office of Technology Assessment
Congress of the United States

Duncan Neuhauser
School of Medicine
Case Western Reserve University

Frederick L. Newman
Systems Research Unit
Eastern Pennsylvania Psychiatric Institute

Laurie A. Pearlman
School of Public Health
Harvard University

Richard A. Rettig
The RAND Corp.

Noralou Roos
Faculty of Medicine
University of Manitoba

Sheldon Rovin
School of Dental Medicine
University of Pennsylvania

Leonard Saxe
Department of Psychology
Boston University

C. C. Scalzi
School of Public Health
University of California, Los Angeles

Karen Schachter
School of Public Health
Harvard University

Richard M. Scheffler
Department of Economics
George Washington University

Steven A. Schroeder
Health Policy Program
University of California, San Francisco

Stuart O. Schweitzer
School of Public Health
University of California, Los Angeles

Jonathan A. Showstack
Health Policy Program
University of California, San Francisco

William B. Stason
School of Public Health
Harvard University

Judith L. Wagner
The Urban institute

Milton C. Weinstein
School of Public Health
Harvard University

Brian T. Yates
Department of Psychology
The American University

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Shari Cretin
University of California at Los Angeles
Palmer Dearing
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National Center for Health Statistics
Erik Farag
Health Resources Administration
Paul Gertman
Boston University
Donald Goldstone
National Center for Health Services Research
Michael Goran
Rockridge Health Care Plan
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James Haug
American College of Surgeons
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Harvard University
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National Institutes of Health
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University of California at San Francisco
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American Hospital Association
Dorothy Rice
National Center for Health Statistics
Wayne Roe
Health Industry Manufacturers' Association
Noralou Roos
University of Manitoba
Gerald Rosenthal
National Center for Health Services Research
Louise Russell
The Brookings Institution
Robert Sauer
Food and Drug Administration
Stephen Schiaffino
National Institutes of Health
Stephen Schoenbaum
Peter Bent Brigham Hospital
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Donald Shepard
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George Silver
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American College of Physicians
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