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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.