Formal Analysis, Policy Formulation, and End-Stage Renal Disease

April 1981

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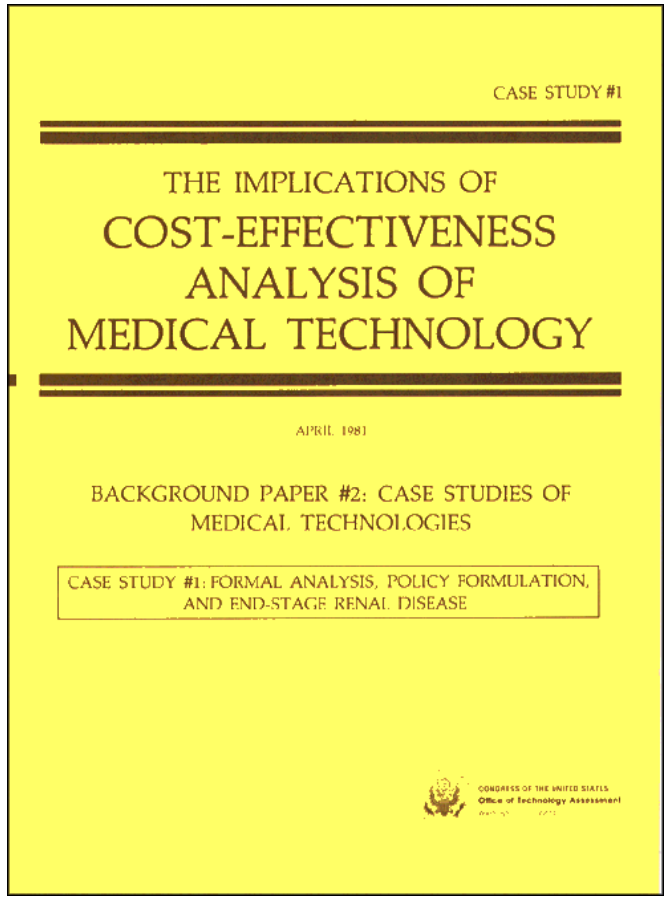

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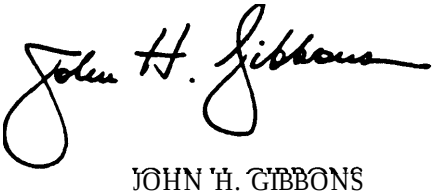
Foreword

This case study is one of 17 studies comprising Background Paper #2 for OTA's assessment, The *Implications* of *Cost-Effectiveness Analysis of Medical Technology.* That assessment analyzes the feasibility, implications, and value of using cost-effectiveness and cost-benefit analysis (CEA/CBA) in health care decisionmaking. The major, policy-oriented report of the assessment was published in August 1980. In addition to Background Paper #2, there are four other background papers being published in conjunction with the assessment: 1) a document which addresses methodological issues and reviews the CEA/CBA literature, published in September 1980; 2) a case study of the efficacy and cost-effectiveness of psychotherapy, published in October 1980; 3) a case study of four common diagnostic X-ray procedures, to be published in summer 1981; and 4) a review of international experience in managing medical technology, published in October 1980. Another related report was published in September of 1979: *A Review* of *Selected Federal Vaccine and Immunization Policies.*

The case studies in *Background Paper* #2; *Case Studies of Medical Technologies* are being published individually. They were commissioned by OTA both to provide information on the specific technologies and to gain lessons that could be applied to the broader policy aspects of the use of CEA/CBA. Several of the studies were specifically requested by the Senate Committee on Finance.

Drafts of each case study were reviewed by OTA staff; by members of the advisory panel to the overall assessment, chaired by Dr. John Hogness; by members of the Health Program Advisory Committee, chaired by Dr. Frederick Robbins; and by numerous other experts in clinical medicine, health policy, Government, and economics. We are grateful for their assistance. However, responsibility for the case studies remains with the authors.



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Preface

This case study is one of 17 topics being issued that comprise Background Paper #2 to the OTA project on the *Implicatiom of Cost-Effectiveness Analysis of Medical Technology. * The* overall project was requested by the Senate Committee on Labor and Human Resources. In all, 19 case studies of technological applications were commissioned as part of that project. Three of the 19 were specifically requested by the Senate Committee on Finance: psychotherapy, which was issued separately as Background Paper #3; diagnostic X-ray, which will be issued as Background Paper #.5; and respiratory therapies, which will be included as part of this series. The other 16 case studies were selected by OTA staff.

In order to select those 16 case studies, OTA, in consultation with the advisory panel to the overall project, developed a set of selection criteria. Those criteria were designed to ensure that as a group the case studies would provide:

- examples of types of technologies by function (preventive, diagnostic, therapeutic, and rehabilitative);
- examples of types of technologies by physical nature (drugs, devices, and procedures);
- examples of technologies in different stages of development and diffusion (new, emerging, and established);
- examples from different areas of medicine (such as general medical practice, pediatrics, radiology, and surgery);
- examples addressing medical problems that are important because of their high frequency or significant impacts (such as cost);
- examples of technologies with associated high costs either because of high volume (for low-cost technologies) or high individual costs;
- examples that could provide informative material relating to the broader policy and methodological issues of cost-effectiveness or cost-benefit analysis (CEA/ CBA); and
- examples with sufficient evaluable literature.

On the basis of these criteria and recommendations by panel members and other experts, OTA staff selected the other case studies. These 16 plus the respiratory therapy case study requested by the Finance Committee make up the 17 studies in this background paper.

All case studies were commissioned by OTA and performed under contract by experts in academia. They are authored studies. OTA subjected each case study to an extensive review process. Initial drafts of cases were reviewed by OTA staff and by members of the advisory panel to the project. Comments were provided to authors, along with OTA's suggestions for revisions. Subsequent drafts were sent by OTA to numerous experts for review and comment. Each case was seen by at least 20, and some by 40 *or* more, outside reviewers. These reviewers were from relevant Government agencies, professional societies, consumer and public interest groups, medical practice, and academic medicine. Academicians such as economists and decision analysts also reviewed the cases. In all, over 400 separate individuals or organizations reviewed one or more case studies. Although all these reviewers cannot be acknowledged individually OTA is very grateful for- their comments and advice. In addition, the authors of the case studies themselves often sent drafts to reviewers and incorporated their comments.

These case studies are authored works commissioned by OTA. The authors are responsible for the conclusions of their specific case study. These cases are not statements of official OTA position. OTA does not make recommendations or endorse particular technologies. During the various stages of the review and revision process, therefore, OTA encouraged the authors to present balanced information and to recognize divergent points of view. In two cases, OTA decided that in order to more fully present divergent views on particular technologies a commentary should be added to the case study. Thus, **following the case**

*Office of Technology Assessment, U.S. Congress, *The Implications of Cost-Effectiveness Analysis of Medical Technology*, GPO stock No. 052-003 00765-7 (Washington, D.C.: U.S. Government Printing Office, August 1980).

studies on gastrointestinal endoscopy and on the Keyes technique for periodontal disease, commentaries from experts in the appropriate health care specialty have been included, followed by responses from the authors.

The case studies were selected and designed to fulfill two functions. The first, and primary, purpose was to provide OTA with specific information that could be used in formulating general conclusions regarding the feasibility and implications of applying CEA/CBA in health care. By examining the 19 cases as a group and looking for common problems or strengths in the techniques of CEA/CBA, OTA was able to better analyze the potential contribution that these techniques might make to the management of medical technologies and health care costs and quality. The second function of the cases was to provide useful information on the specific technologies covered. However, this was not the major intent of the cases, and they should not be regarded as complete and definitive studies of the individual technologies. In many instances the case studies do represent excellent reviews of the literature pertaining to the specific technologies and as such can stand on their own as a useful contribution to the field. In general, though, the design and the funding levels of these case studies was such that they should be read primarily in the context of the overall OTA project on CEA/CBA in health care.

Some of the case studies are formal CEAs or CBAs; most are not. Some are primarily concerned with analysis of costs; others are more concerned with analysis of efficacy or effectiveness. Some, such as the study on end-stage renal disease, examine the role that formal analysis of costs and benefits can play in policy formulation. Others, such as the one on breast cancer surgery, illustrate how influences other than costs can determine the patterns of use of a technology. In other words, each looks at evaluation of the costs and the benefits of medical technologies from a slightly different perspective. The reader is encouraged to read this study in the context of the overall assessment's objectives in order to gain a feeling for the potential role that CEA/CBA can or cannot play in health care and to better understand the difficulties and complexities involved in applying CEA/CBA to specific medical technologies.

The 17 case studies comprising Background *Paper #2* short titles and their authors are:

Artificial Heart: Deborah P. Lubeck and John P. Bunker
Automated Multichannel Chemistry Analyzers: Milton C. Weinstein and Laurie A. Pearlman
Bone Marrow Transplants: Stuart O. Schweitzer and C. C. Scalzi
Breast Cancer Surgery: Karen Schachter and Duncan Neuhauser
Cardiac Radionuclide Imaging: William B. Stason and Eric Fortess
Cervical Cancer Screening: Bryan R. Luce
Cimetidine and Peptic Ulcer Disease: Harvey V. Fineberg and 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, and John P. Bunker
End-Stage Renal Disease: Richard A. Rettig
Gastrointestinal Endoscopy: Jonathan A. Showstack and Steven A. Schroeder
Neonatal Intensive Care: Peter Budetti, Peggy McManus, Nancy Barrand, and Lu Ann Heinen
Nurse Practitioners: Lauren LeRoy and Sharon Solkowitz
Orthopedic Joint Prosthetic Implants: Judith D. Bentkover and Philip G. Drew
Periodontal Disease Interventions: Richard M. Scheffler and Sheldon Rovin
Selected Respiratory Therapies: Richard M. Scheffler and Morgan Delaney

These studies will be available for sale by the Superintendent of Documents, U.S. Government Printing Office, Washington, D.C. 20401. Call OTA's Publishing Office (224-8996) for availability and ordering information.

Case Study #1

Formal Analysis, Policy Formulation, and End-Stage Renal Disease

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AUTHOR'S ACKNOWLEDGMENTS

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Contents

LIST OF TABLES

FIGURE

Case Study #1:
Formal Analysis, Policy Formulation, and End-Stage Renal Disease

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INTRODUCTION

The past two decades have witnessed increasing use of formal analysis in Federal Government policymaking. Most strongly identified with the Department of Defense in the early 1960's (8,15,16), formal analysis has now been extended to all areas of domestic public policy, including health (35,36). The use of formal analysis has also been extended from policymaking to program evaluation (44). In this case study, we analyze two instances of the use of formal analysis in the formulation of Federal Government policy for end-stage renal disease (ESRD).

The term formal analysis, as used in this study, refers to any explicitly analytical means of systematically examining the social costs and benefits of alternative policies for the purpose of choosing a preferred alternative in light of an a priori normative decision rule. Included are cost-benefit analysis (CBA), cost-effectiveness analysis (CEA), risk-benefit analysis, technology assessment, and other comparable means of analysis used in decisionmaking.

Much attention is given in the literature about formal analysis for policymaking to the general techniques and limits of analysis (23,30) or the results and implications of particular studies. Of critical importance, as well, however, are the institutional factors encouraging or inhibiting the use of formal analysis in policymaking. These institutional factors are a major concern of this case study.

This case study examines two instances of the use of formal analysis in the formulation of Federal Government policies toward ESRD.[1] Its focus is on the work of two committees, whose reports were an integral part of the ESRD policy formulation process in 1966 and 1967: 1) the Gottschalk committee, advisory to the U.S. Bureau of the Budget (BOB), and 2) the Burton committee, internal to the Public Health Service (PHS). Several questions are addressed. What factors gave rise to the demand for formal analysis within the policy process? What were the purposes of and constraints on the analyses? What did the analyses conclude? How were the results of these analyses used? What general lessons can be drawn from this experience?

Background information about ESRD is presented in the first section below. The subsequent section contains a review of the literature pertaining to ESRD, emphasizing the two major analytical studies—the Gottschalk report and the Burton report—which were pertinent to the policy formulation process. The role that those two studies played in that process is then examined in the next section. In the final section, we develop our conclusions.

[1]The general evolution of Federal policy in the area of ESRD has been analyzed elsewhere (32, 33).

THE ESRD PATIENT POPULATION

Treatment of ESRD by hemodialysis began in early 1960. By early 1967, according to estimates prepared by an advisory committee to BOB, approximately 750 to 1,000 patients had been treated by dialysis (31).

Data on patients treated in PHS-supported dialysis programs from March 1960 to March 1967 are presented in table 1. These data indicate that roughly 90 percent of these patients were distributed across the four age groups from 15 to 54 years. They also show an overall mortality rate for these patients of 17 percent, with considerable variation in the mortality rates for specific age groups.

Table 2 presents similar data for patients receiving dialysis in Veterans Administration (VA) hospitals from March 1963 through March 1967. By comparison with the PHS data in table 1, the data on these patients reveal a slightly higher patient mortality rate, fewer younger patients, and fewer elderly patients.

Time series data on dialysis began to be collected in 1970. Data from the National Dialysis Registry[2] are presented in table 3. These data show: 1) the proportion of women in the total patient population increased from 32 percent in 1970 to 40 percent in 1976; 2) the average age climbed steadily for both in-center and home dialysis patients; and 3) the proportion of home patients to total dialysis patients climbed to ap-

[2]The National Dialysis Registry was managed by the Research Triangle Institute from 1967 through 1976, under contract to the National Institutes of Health (NIH). The National Dialysis Registry was known to underreport the total U.S. dialysis population, because participation in the Registry was voluntary. Since the authorization of the medicare ESRD program in late 1972 created the expectation that both the National Dialysis Registry and the Human Renal Transplant Registry would be merged into a single system, it can be assumed that underreporting was exacerbated from 1974 onward.

Table 1.—Age Distribution of Dialysis Patients and Deaths in PHS-Supported Programs, March 1960-March 1967

Age group	All patients	Percent total patients	Number deceased	Percent deceased
Under 15	3	1.2	0	0.0
15-24	42	17.0	7	16.7
25-34	69	27.9	9	13.0
35-44	61	24.7	7	11.4
45-54	52	21.1	13	25.0
55-64	18	7.3	5	27.8
Over 65	2	0.8	1	50.0
Total	247	100.0	42	17.0

SOURCE *Report of the Committee on Chronic Kidney* Disease. September 1967

Table 2.—Age Distribution of Dialysis Patients and Deaths in VA Hospitals March 1963-March 1967

Age group	All patients	Percent total patients	Number deceased	Percent deceased
Under 15	0	0.0	0	0.0
15-24	8	3.5	2	25.0
25-34	54	23.4	9	16.7
35-44	99	42.8	18	18.2
45-54	62	26.8	14	22,6
55-64	8	3.5	4	50.0
Over 65	0	0.0	0	0.0
Total	231	100.0	47	20,3

SOURCE *Report of the Committee on Chronic Kidney Disease* September **1967**

Table 3.— Patients Alive on Dialysis in the United States, 1970-76

Report dated Jan. 1	Total number of patients alive	Number of men	Number of women	Number in home program	Average age In center patients	Average age Home patients
1970..	2,398	1,621	762	844	39	41
1971	3,463	2,256	1,184	1,361	40	42
1972	4,981	3,212	1,751	2,001	42	43
1973	7,498	4,741	2,696	2,703	44	45
1974	10,306	6.334	3,871	3,402	44	43
1975	13,417	8,231	5,098	3,814	45	44
1976	17,063	10,156	6,783	4,076	46	45

SOURCE Research Triangle Institute National Dialysis Registry, **Final Report.** August 1976

proximately 40 percent in 1971 and 1972, and declined steadily thereafter to 24 percent in 1976 (4). (By 1979, home dialysis patients accounted for approximately 13 percent of the total dialysis patient population.)

Worldwide data on kidney transplants from the early 1950's through May 1, 1976, are shown in table 4. Based on the Human Renal Transplant Registry, ' these data show a fairly stable number of transplants being performed in the United States since 1972 (l).

'The Human Renal Transplant Registry, supported by NIH and managed under contract by the American College of Surgeons, collected data on kidney transplants from 1972 through 1976. Before that time, such data were collected by the Kidney Transplant Registry of the Peter Bent Brigham Hospital, Harvard Medical School, Boston, Mass. (25).

Table 4.— Total Kidney Transplants Worldwide by Year, 1967-76'

	1953-66	1967	1968	1969	1970	1971	1972	1973	1974	1975	1976	Total
United States	1,146	462	702	872	1,139	1,676	2,232	2,417	2.218	2,399	438	15,701
Canada	93	64	83	83	126	200	165	211	177	72	–	1,274
Australia (to 4/30/75)	55	95	128	203	203	263	242	292	317	115	—	1,913
Other countries.	426	211	332	381	524	778	862	925	994	787	—	6,220
Total	**1,720**	**832**	**1,245**	**1.539**	**1,992**	**2,917**	**3,501**	**3,845**	**3,706**	**3,373**	**438**	**25,108**

'Includes all transplants recorded up to Ma, 1 1976
Total transplants 25 108
Total recipients *22261*

SOURCE American College 01 Physicians National Institutes of Health Organ Transplant Registry *May Newsletter 1976*

Medicare coverage for ESRD treatment by both dialysis and transplantation has been available for more than 90 percent of the U.S. population since July 1, 1973. The number of patients in the medicare ESRD program from its inception in 1973 through 1978 is shown in table 5. The proportion of patients 65 years old and above in the medicare ESRD patient population has increased from 5 percent in 1974 to basically 20 percent in 1977 and 1978.

The establishment of a data system for providing accurate, consistent, and timely data on ESRD patients has proved to be an extremely difficult task (32). The single Medicare Registry, which was to have combined both the National Dialysis and Human Renal Transplant Registries, existed briefly from 1975 through 1978 as a contractor-managed system, and it issued reports only in December 1976. The management contract for that Registry was terminated in 1978, and responsibility for the ESRD Medical Information System was transferred from the Bureau of Quality Assurance of PHS to the Medicare Bureau of the Health Care Financing Administration (HCFA). The new system began to issue reports in 1979.

Table 5.—Medicare ESRD Patient Population 1973-78

End of calender year	Total number of patients
July 1, 1973a	11,000
1973.	14$000
1974	23,000
1975	31,000
1976	38,000
1977	44.000
1978	50,000

[a]Inception of program

SOURCE Office of Financial and Actuarial Analysis, Division of Medicare Cost Estimates, Health Care Financing Administration, March 1979

FORMAL ANALYSIS LITERATURE PERTAINING TO ESRD

In 1967, two reports were published which constitute the primary formal analyses pertaining to ESRD. One of these reports, often known as the Gottschalk report,[4] was the work of an expert advisory committee to BOB (31). The other report, known as the Burton report, [5] was the work of a PHS task force which reported to the U.S. Surgeon General (17). Work on the Gottschalk report was begun in mid-1966 and completed "1 year later. The Burton report was prepared in 4 months in 1967. The two reports were released together in November 1967, at a press release called by PHS.

A derivative literature is associated with each report. Herbert Klarman, an economist who served on the Gottschalk committee, subsequently wrote abou: that committee's effort in several articles (18,19,20). Similarly, Robert Grosse briefly discussed the Burton report to the Surgeon General in several papers describing the Health, Education, and Welfare (HEW) program analyses in health done in the 1965-67 period (1 1, 12, 13).

A set of analyses on kidney disease was performed by Research Triangle Institute} (RTI). RTI's initial report to the Kidney Disease Control Program of PHS was prepared under a contract effective June 27, "1966 (14). A subsequent paper set forth the methodology for conducting benefit-cost analysis of kidney disease (10). The full report on work performed under RTI's contract was published later in 1968 (21). All this work was either supported by or derived from the Burton committee's efforts.

Several other formal analyses have also been done. Pliskin, for instance, developed a health index for the selection of chronic renal disease patients (27) and a methodology for projecting needed hemodialysis beds (28). Barnes presented a simplified model of treating patients on chronic hospital dialysis, chronic home dialysis, living donor transplantation, and cadaver donor transplantation (2). Recently, Stange and Sumner have compared expected life-years gained from and costs of treatment by center dialysis, home dialysis, and transplantation (41).

In addition, there area few other brief formal analyses that attempt to compare the costs and effectiveness of the several means of treatment for ESRD. These add little that is new by way of data, methodology, or results to the literature cited above, however, and since the focus of the present study is on the institutional factors influencing the use of formal analysis, attention is directed mainly to the Gottschalk and Burton reports respectively.

[4]After its chairman. Dr. Carl Gottschalk, a professor at the University of North Carolina School of Medicine.

[5]After its chairman. Dr. Benjamin T. Burton, an associate director of the then National Institute of Arthritis and Metabolic Disease.

FORMAL ANALYSIS AND ESRD POLICY FORMULATION

In the more than 15 years during which Federal policy toward ESRD has developed, cost analyses have always been central to policy formulation. But only on one occasion have other forms of analysis been integral to the policy process. In 1966 and 1967, two efforts were made to apply CEA to kidney disease. The reports that resulted have already been mentioned: 1) *Report of the Committee on Chronic Kidney Disease* to BOB (the Gottschalk report) (31), and 2) *Kidney Disease Program Analysis: Report to the Surgeon General* (the Burton report) (17). These reports stimulated further analyses and also provided guidance to action.

Report of the Committee on Chronic Kidney Disease

The need for a review of Federal policies and programs related to ESRD was forcefully underlined for BOB by several events in 1965 and early 1966. First, VA submitted a budget request

to BOB for construction funds to create dialysis units in a number of VA hospitals and for additional funds to staff these units. VA's request prompted BOB to ask the Office of Science and Technology about the possibility of an external review of the VA dialysis program. That Office agreed that such a review would be useful, but urged that it include all Federal Government efforts in ESRD.

The attractiveness of such a review was reinforced when NBC News, on November 28, 1965, presented a l-hour television documentary, "Who Shall Live?" (26). Narrated by Edwin Newman, that program dramatically contrasted the lack of funds for dying individuals needing dialysis treatment to the hundreds of millions of dollars being spent for exploration of outer space and major weapons systems. Newman concluded the program by observing: "What is wrong is that a medical miracle has been achieved and we refuse to face its implications. We continue to argue over where the money is coming from—and we have the money." Representative Melvin Laird (R., Wis.), then ranking Republican member of the House appropriations subcommittee for HEW, was quoted: "We're spending billions of dollars to get to the moon, and it seems to me that these human problems, which we have right here on earth, need to be solved. " BOB, the Office of Science and Technology, and the White House all recognized that public pressures for more Federal funding of dialysis were building.

The expansion of dialysis-related activities was being actively promoted within PHS. Dr. James Kimmey, chief of PHS's Renal Disease Activity Branch, Division of Chronic Diseases, supported by certain congressional and medical allies, was advocating a dialysis program that would grow to an eventual level of $150 million annually. In March 1966, however, BOB staff discovered that Kimmey's plan had not considered either the VA program or the offsetting effects of providing transplants to some patients, although it did acknowledge that some dialysis patients would die (24). The need for a Government-wide review seemed increasingly clear.

In early 1966, therefore, BOB organized an advisory committee of outside experts to review Federal activities in dialysis. Dr. Carl Gottschalk, University of North Carolina, agreed to chair the committee and helped select the members from medicine, science, and ethics; BOB selected Klarman and Gerald Rosenthal, the economists.[6] The Gottschalk committee met seven times from July 1966 through May 1967, and submitted its report (31) to Charles Schultze, BOB director, in September 1967; the report was released to the public in November of the same year.

Central to any analysis is its purpose. BOB's charge to the Gottschalk committee **was** not fully articulated at the outset, but instead evolved over several months. Two concerns were foremost to BOB. One was national policy makers' substantial interest at that time in realizing clinical payoff from the Nation's large and continuing investment in biomedical research. That interest was dramatized on June 27, 1966, when President Lyndon B. Johnson called the Director of the National Institutes of Health (NIH) and the directors of the individual institutes of NIH to the White House to ask pointedly about the practical benefits the Nation was receiving from NIH-supported biomedical research (42).

BOB's second concern was to involve itself early in decisionmaking about new medical programs, especially those having major resource implications, so that policies could be shaped from a national perspective. Within BOB, Irving Lewis, Chief of the Health and Welfare Division, recalls: "We were very troubled by the VA request. There was a VA program, there was a PHS program, but there was no national approach to the problem" (22). The advisory committee of experts was convened to help BOB develop a national approach to ESRD.

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hlembt're nInIIt tee that BOB organized were I Dr Bernard Amos, Duke Un iversity Dr Lewis ~\' Bluemle Un iversit vot Pennsylvania Dr William A Greene Universit v ot Rochester Dr F Herbert Klarman Johns Hopkins Universit y I Dr John S Najarian University of Ca lifornia San Franc isc o Dr Gerald Rosenthat Brandeis Universit v Osc a r M Ruebhausen Debvoise Plimpton, Lyons, and Ga tes, New York; Dr George E. Schreiner Georgetown University; and Dr. Donald W Seldin University of Texas, Dallas. The advisors to the commit tee were Dr Robert W Berliner, National Heart Institute Dr Bernard Greenberg Universi t v ot North Carolina and Dr Arnold S Nash, Universi t y of North Carolina The BOB protessional supporting the committee was Pierre S Palmer he reported to Irving I Lewis Chiet of BOB s Health and Welfare Division

BOB expressed its two primary concerns at the Gottschalk committee's initial meeting in July 1966, but clear definition of the committee's mission came later. The committee's October meeting involved conversations with visiting foreign physicians attending the International Congress on Nephrology. In November, the group visited Dr. Belding H. Scribner, a pioneer in treating kidney failure by hemodialysis, in Seattle to learn about home dialysis and talk to patients. Not until the December meeting, however, did BOB's charge to the committee come into sharp focus.

Some on the committee wished to conduct a CBA addressing the relative priority "of investing resources in treatment of chronic kidney disease as against other needs in the health field" (37). One suggestion was to convene a group of health generalists to consider alternative claims on resources. A majority of those on the committee, however, held that determination of priorities rested with the Surgeon General, the Secretary of HEW, the BOB Director, and the President. Irving Lewis, responding to an inquiry from his BOB staff, concurred: "I think this is right. They should, however, give priorities within kidney disease."7

The Gottschalk committee's report, consequently, focused on those individuals with chronic kidney failure who required treatment by hemodialysis or kidney transplantation (31). These two forms of treatment, it said, "are now available as fruits of research and development programs and are life-saving." Though by no means optimal, these treatments could add a significant number of years to the lives of individuals "now dying because these treatment forms are not generally available. " The cost-effectiveness section of the Gottschalk report elaborated the reasons for not doing CBA, or weighing the funds used for treating chronic kidney failure against alternative uses of resources. The basic problem with CBA, it noted, was placing a value on human life. Furthermore, the report said, "in the absence of overwhelming costs (as reflected by an appreciable fraction of the GNP), " treatment for ESRD should be made universally available (31):

> The reasons for this decision are the irreversibility of the decision not to do so, as far as an individual is concerned; the existence for the first time of a technology capable of prolonging the lives of persons otherwise doomed to an early death; the relative youth of these persons; the prospects of further improvements in technology; and the fact that patients are known and identifiable, not members of a statistical distribution, enhances the community's interest in doing something in their behalf.

The committee's CEA took as it point of departure the committee's conclusion and recommendation that treatment be provided to all in medical need of it.

The Gottschalk report also dealt with the issue of prevention versus treatment. In transmitting the report to BOB Director Schultze, Gottschalk wrote (31):

> Prevention is obviously preferable to treatment of disease. Unfortunately, knowledge concerning the causes and prevention of end-stage kidney disease is limited and this is an area in which an expanded research effort is required. Furthermore, even if a completely successful method of prevention is developed it will have no significant impact on the numbers of people dying from end-stage kidney disease for many years. Therefore, the Committee recommends a national treatment program aimed at providing chronic dialysis and/or transplantation for all of the American population for whom it is medically indicated.

The conclusions of the committee's report were the following: 1) the high cost of ESRD treatment required Federal financial assistance;[8] 2) ESRD treatment should be universally available, not arbitrarily provided, say, to veterans, but denied to nonveterans; and 3) the Federal commitment to ESRD treatment necessarily had to be a continuing one. The Gottschalk committee also concluded that continuing medical research into the causes of kidney disease and

'Klarman recalls that he and Rosenthal spent much time in 1967 attempting, without success, to prepare a CBA,

[8]This conclusion was based on written and oral testimony received by the committee from Louis Reed, Social Security Administration, and from representatives of all major health insurance plans.

means of prevention was needed, though it did not consider this issue at any length.

The recommendations that followed from these conclusions dealt with the supply of and demand for ESRD treatment, and the management of Federal efforts in this area. On the supply side, the Gottschalk committee recommended Federal investment in establishing comprehensive kidney centers and satellite community dialysis units, as well as in training health professionals to staff such facilities. On the demand side, its recommendation was to provide for patient care financing by amending title XVIII (medicare) of the Social Security Act. Specifically, the committee recommended enacting the Johnson administration's pending amendment to extend medicare coverage to the disabled under 65 years of age and redefining disabled to include those with kidney failure even though they might continue to work as a result of treatment. On Federal management, it suggested "an appropriate mechanism" for coordinating governmental efforts.

The formal analysis in the committee's report included sections on treatment by hemodialysis and by transplantation, a discussion of the incidence and prevalence of chronic kidney failure, a projection of treatment needs, and CEA itself (31). The hemodialysis section addressed patient selection criteria. Ideal candidates for hemodialysis were then thought to be persons between 15 and 45 years of age, the upper age limit was expected to rise, however, and for analytical purposes, the committee used a 54-year upper age limit. Vascular diseases as medical conditions limiting treatment were briefly discussed, as were the limits of dialyzing children. It was also noted that the degree of rehabilitation of dialysis patients varied widely; existing data were of little use on the matter. It was expected that psychosocial problems of patients adapting to dialysis would increase as selection criteria were relaxed.

PHS and VA data were analyzed for some indication of mortality. Later in the analysis, projections would be based on 15-percent mortality of a dialysis population cohort in its first year, and a 10-percent mortality in each successive year. ' Costs for 16 center dialysis programs ranged from $10,000 to $21,000 per patient per year. Five home dialysis programs had costs ranging from $3,750 to $9,800 per year.

The section on transplantation outlined a strong case for that treatment relative to dialysis. The report noted an important shift over time in organ donor source—from identical twins to close blood relatives to nonliving (cadaver) donors. Cadaver organs, it said, were becoming the most important source of organs, owing in part to high success rates with them.

Worldwide mortality data from the Human Renal Transplant Registry, however, showed that survival rates declined with the shift from identical twins to siblings to parents to unrelated living donors to cadavers (25). The best l-year survival rate for cadaver kidneys was 38 percent. The report concluded that these worldwide data were of little predictive value, however, since they included the early experimental stage of transplantation. The report used only data for the 2 most recent years, broken down by large and small transplantation centers. These data showed l-year kidney survival rates of 65 percent for sibling donors and 39 percent for cadavers transplanted in large centers, results deemed "noteworthy" and "very encouraging. "

The committee's optimism about the future of renal transplantation was based on two subsets of data from the Human Renal Transplant Registry. The first excluded all deaths save those resulting from uncontrollable rejection: 3-year survival rates for siblings and parents were 70 percent and over 60 percent for cadavers. The second set included deaths from rejection and infection: l-year survival rates *were* 66 percent from close relatives and 54 percent from cadaver kidneys. The committee's report found it "impressive" that projections for fifth year survival for all cadaver kidneys, "if technical failures and deaths due to causes other than infection or rejection are excluded, " were 40 percent. The fu-

'Mortality assumptions were developed by Bernard Greenberg. Klarman and Rosenthal developed the detailed survivorship tables.

ture prospects for transplantation, the report suggested, were the following (31):

> From the subsets it is possible to extrapolate what might be expected from continuing experience and excellent management with existing methods of treatment, i.e., between **65** percent and 75 percent l-year survival for a closely related donor, between **40** percent and **50** percent survival for a cadaver donor. Additional known factors expected to improve the results further are (a) selection of antigenically compatible donors, (b) improved immunosuppression, and (c) the induction of specific immunologic tolerance.

Several features about these projections for kidney survival deserve mention. First, no estimates of the magnitude of the shift from living to cadaver donors were given in its report; nor were different projections developed for different degrees of shift. Second, the two subsets of Registry data on which the projections were based were not published. Finally, it was never clearly indicated how the assumptions based on these data subsets were used in the later CEA.

These matters are important because the optimism about transplantation, based on expert testimony heard by the committee, was never fully realized. One-year survival for cadaver transplants subsequently reached 55 percent in 1970 and then declined slightly (1,43). At the time of the committee's report, there were few who questioned the assumption that success with transplantation would improve with time.

Reported costs for transplantation from four centers ranged from $10,000 to $22,000, including preoperative and postoperative dialysis costs. One center reported an average cost of $13,300 for its 46 most recent transplants, and the Gottschalk committee judged that as the "most valid estimate. "[10]

The report contained a long section on incidence and prevalence of chronic kidney failure. Noting the absence of any periodic reporting on this disease, the Gottschalk committee developed two estimates of the potential dialysis cases for 1964, and from these derived an upper limit, lower limit, and most probable annual dialysis caseload. Mortality estimates were derived from actual data on dialysis: 15 percent mortality in the first year; 10 percent for each successive year. The kidney transplant failure rate was estimated to be 50 percent annually for the first 2 years and 5 percent each year thereafter. Projections of the dialysis caseload for the decade from 1968 through 1977 were made on the basis of varying levels of chronic kidney failure and varying numbers of patients transplanted. These projections are shown in table 6. If one compares the 1977 dialysis caseload for maximum level of transplantation and lowest level of total cases (29,201) with that for no transplantation and the maximum number of dialysis cases (53,633), it is obvious that projec-

[10] Klarman developed this estimate during a visit to Dr. David Hume, one of the kidney transplant pioneers, at the Medical College of Virginia in Richmond.

Table 6.—Predicted Caseload in Patient Years of Dialysis Under Varying Levels of Expected Cases of Chronic Uremia and Differing Amounts of Transplantation, 1968-77

	No transplantation, and number of cases is:			Probable number of transplantations, and number of cases is:			Maximum number of transplantations, and number of cases is:		
Year	Lower	Most probable	Upper	Lower	Most probable	Upper	Lower	Most probable	Upper
1968	5,530	6,436	7,541	5,354	6,261	7,365	5,333	6,239	7,344
1969	10,470	12,187	14,279	9,982	11,698	13,790	9,914	11,636	13,722
1970	15,038	17,503	20,506	14,158	16,624	19,626	14,037	16,503	19,506
1971	19,265	22,423	26,269	17,914	21,071	24,917	17,588	20,747	24,592
1972	23,191	26,994	31,624	21,293	25,096	29,727	20,584	24,387	29,017
1973 .....,	26,847	31,252	36,611	24,325	28,728	34,088	23,141	27,544	32,905
1974, ...	30,265	35,229	41,272	27,016	31,979	38,021	25,300	30,263	36,305
1975	33,468	38,956	45,638	29,367	34,856	41,538	27,043	32,532	39,213
1976	36,482	42,463	49,747	31,382	37,363	44,648	28,354	34,334	41,620
1977 ...,	39,332	45,779	53,633	33,053	39,498	47,351	29,201	35,649	43,505

SOURCE *Report of the Committee on Chronic Kidney Disease*, September **1967**

tions vary substantially as a consequence of their underlying assumptions. Such wide variation can obviously affect planning for facilities, if program decisions are to be based on the projections.

The heart of the Gottschalk report's CEA was a comparison of the expected life-years added by transplantation and by dialysis with the predicted costs of each. The critical assumptions for transplantation were the 50-percent kidney survival at the end of 2 years and a 5-percent annual failure rate thereafter. On the basis of these assumptions, transplantation led to an expected 17 additional life years (13.3 from transplantation and 3.9 from dialysis before and after a transplantation). Dialysis, on the other hand, based on a 15-percent first year mortality and lo-percent for each successive year, led to an expected 9 additional life-years.

The present value costs of 17 years of additional life from transplantation were calculated to be $44,500. The comparable present value costs of 9 additional life-years from dialysis were calculated to be $104,000 for center dialysis and $38,000 for home dialysis; if there were a 50-50 split between the two, the average estimated cost for dialysis would be $71,000. (The present value estimates were based on 4- and 5-percent discount rates, respectively, for center and home dialysis.)

The results of the comparison are presented in table 7. An adjustment for quality of life was made for transplantation based on the observation that an additional year of life from a successful transplantation is much better (1.25 times better) than an additional life-year from dialysis.

Table 7.— Increased Life-Years and Costs per Additional Life-Year by Means of Treatment

Modality	cost	Life-years gained	cost per year
Dialysis			
Center.	$104,000	9	$11,600
Home.	38,000	9	4,200
Average. . . .	71,000	9	7,900
Transplantation			
Unadjusted.	44,500	17	2,600
Adjusted for quality.	44,500	20.5	2,200

SOURCE: *Report 01 the Committee on Chronic Kidney Disease September* 1967

The conclusions of the Gottschalk report's CEA, not surprisingly, were these: 1) The maximum transplantation course was a better way to increase life expectancy for a given cost; 2) the value of a home dialysis program did not differ much from that of transplantation, emphasizing the proportion of transplantation costs attributable to dialysis; and 3) any shift in the dialysis population away from center dialysis and toward home dialysis offered substantial economic gain.

The committee's recommendation, therefore, was for a national treatment program aimed at building adequate treatment capacity and paying patient bills. Within that policy, the committee said, transplantation should be encouraged to the maximum extent possible, and home dialysis should be encouraged over center dialysis,

The estimated total costs, including both Federal and non-Federal funds, for the patient care portion of the national dialysis and transplantation program recommended by the Gottschalk committee were $40 million (low) and $49 million (probable) for fiscal 1970, the first year, and $157 million (low) and $205 million (probable) for fiscal 1974, the fifth year. The committee's table summarizing the cost estimates is reproduced as table 8. Estimates for total patients treated by both dialysis and transplantation were 3,662 in the first year and 18,500 in the fifth. The estimated costs to the Federal Government, from fiscal years 1969 through 1975, for construction or renovation, initial equipment, operating subsidy, and training personnel for kidney centers and community dialysis units, respectively, were $29 million, $22 million, $30 million, $30 million, $22 million, $23 million, and $15 million.

Kidney Disease Program Analysis: A Report to the Surgeon General

In the early 1960's, under Defense Secretary Robert McNamara, CEA was introduced into national security decisionmaking with a flourish. In 1965, Henry S. Rowen left his position as

Table 8.—Total Estimated Federal and Non-Federal Patient Care Costs of a National Dialysis and Transplantation Program

	In-center dialysis			Home dialysis			Transplantations			Total program	
Fiscal year	Patients treated	Cost/ patient year	Estimated Cost[a] ($000,000)	Patients treated	Cost/ patient year	Estimated cost[a] ($000,000)	Patients treated	Cost/ trans-plant	Estimated Costa ($000,000)[l]	Patients treated	Estimated cost[a] ($000,000)
1970											
Low estimate	1,456	$10,000	$15	1,456	$4,000	$17	750	$10,000	$ 8	3,662	$40
Probable . . .	1,456	“14,000	20	1,456	5,000	19	750	13,000	10	3,662	49
1971											
Low estimate	2,194	“ 0,000	22	2,194	4,000	16	1,250	10,000	13	5,638	51
Probable . .	2,194	14,000	31	2,194	5,000	18	1,250	13,000	16	5,638	65
1972											
Low estimate	3,731	10,000	37	3,731	4,000	30	1,750	10,000	18	9,212	85
Probable	3,731	14,000	52	3,731	5.000	34	1,750	13,000	23	9,212	109
7973											
Low estimate	5,878	10,000	59	5,879	4,000	45	2,550	10,000	26	14,307	130
Probable	5,878	14,000	82	5,879	5,000	51	2,550	13,000	33	14,307	166
1974											
Low estimate	7,575	10,000	76	7,576	4,000	47	3.350	10,000	34	18,501	157
Probable	7,575	14,000	106	7,576	5,000	55	3,350	13,000	44	18,501	205
1975											
Low estimate	8,925	10,000	89	8,926	4,000	49	4,150	10,000	42	22,001	180
Probable	8,925	14,000	125	8,926	5,000	58	4,150	13,000	54	22,001	237

[a]Rounded to nearest million
[b]Based on appC tables 40 and 41 Excludes patient years of dialysis needed for transplants (table 4 1) because the cost of this dialysis is Included in the cost per transplantation For 197375 the number of patient years of dialysis for new cases orginating in these years have been deducted because they are capability rather than requirements they have been replaced by most probable number of new pal ients needing dialysis (table 40) translated to patient years by using 925 percent the first year 87 2 percent second year and 87 6 percent third y ear
[c]From table 3
[d]Includes initial home dialysis equipment and training costs of $10 000 for new patients on home dialysis 1970 $11 2 million 1971 $74 million 1972 $15 4million 1973 $21 5 mill ton 1974$17 mi lion 1975 $135 million Assumes that equ ipment provided for home dialysis patients who have died or been transplanted will be made avai l able for new pat ients starting on home dialysis

SOURCE *Report of the Committee on Chronic Kidney Disease* September 1977

Deputy Assistant Secretary of Defense for Systems Analysis to become the Assistant Director of BOB. In that position, aided by a formal declaration from President Lyndon B. Johnson, he promoted the use of CBA and CEA throughout the domestic agencies of the Federal Government. Also in 1965, John Gardner became the Secretary of HEW and took a number of steps to increase his control over the far-flung HEW bureaucracy. One step was to create the position of Assistant Secretary for Planning and Evaluation to do for HEW what the systems analysts had done for the Defense Department. The first occupant of that post, William Gorham, came from the Pentagon. Early in 1966, Gorham and his staff sought to promote the use of CEA and CBA in HEW (34).

In response to these developments at HEW, the U.S. Surgeon General, William H. Stewart, in March 1967 established health program analysis groups to address disease control issues related to air pollution, cancer, health care facilities, kidney disease, manpower, and narcotic addiction.[11] For each issue, these groups were to discuss: 1) goals and objectives and ways of measuring them; 2) alternative a$_p$-proaches to the same objectives; 3) the cost “of reaching various points on the continuum of objectives by means of alternative approaches;” and 4) assumptions underlying conclusions, the uncertainties affecting the estimates, and the issues not resolvable at that time. Each alternative approach was to consider “the relative mix of research, prevention, and control. ” The program analysis groups were to complete their work by May 30, 1967, and the resulting reports were to guide the development of the HEW’s 1969-1973 5-year program and financial plan, as well as the fiscal 1969 budget and legislative proposals (9).

[l] A 1966 Program Memorandum on Selec ted Disease Control Programs, dratted 1 year earlier, was cited as an exercise t o gai n experience i n applying ana l y t ical methods t o t he study of disease control programs.

The kidney disease program analysis group, chaired by Dr. Benjamin T. Burton, was composed of individuals from various parts of PHS.[12] Its charge from the Surgeon General was a general one given to all the program groups. The kidney disease group, therefore, translated this general guidance into a charge to perform "a logical analysis of programs leading to a solution or amelioration of the problem of kidney disease" (17). The group identified traditional disease control mechanisms for such a task as prevention, diagnostic and therapeutic techniques, laboratory and clinical research, manpower training, public education, and the construction of specialized facilities. It made the assumption that no "single program component would lead to a major reduction in the national kidney disease problem, " so a mix of approaches would have to be employed. It further assumed that "unlimited Federal funds" were not available for any given disease problem, such as kidney disease; therefore, "a rational balance" had to be struck between various approaches "to derive maximum benefits from any current or possibly extended future Federal efforts. " These controlling assumptions affected the definition of the problem for analysis, the analysis itself, and the implications of the analysis.

The Burton report to the Surgeon General (17) was organized into six chapters: the summary of the report and chapters dealing successively with the major types of kidney disease; current kidney disease control programs, both Federal and non-Federal; research methods; the program analysis; and the cost of treating all patients with chronic kidney failure.

[12]The kidney disease group was chaired by Dr. Benjamin T. Burton, National Institute of Arthritis and Metabolic Diseases (NIAMD). Its members were: Dr. Kent N. Gershingarn, NIAMD; Dr. William R. De Cesare, NIH; Dr. Gerald A. Escovitz, Bureau of Health Manpower; John O'S. Francis, Bureau of Disease Prevention and Environmental Control (BDPEC); Dr. Richard B. Freeman, BDPEC; Ezra Glaser, NIH; Dr. William H. Goldwater, NIH; Dr. Norman A. Hilmar, BDPEC; Dr. Donald E. Kayhoe, National Institute of Allergy and Infectious Diseases (NIAID); and Dr. Robert van Hoek, Bureau of Health Services. Liaison with the Office of the Surgeon General was provided by William M. Anderson. There were three consultants to the group from the Research Triangle Institute: Albert V. Alhadeff, Jerome B. Hallan, and Dr. Edgar A. Parsons.

The Burton group's analysis was based on a breakdown of kidney disease into four primary types:

1. infectious diseases,
2. hypersensitivity diseases,
3. diseases associated with hypertension, and
4. end-stage disease.

The progression of each of the first three types was to the same functional equivalent—end-stage—where irreversible deterioration of kidney function had proceeded so far that untreated individuals would die.

Each of the four types of kidney disease was analyzed in terms of four hypothetical programs, delineated by time, funding level, and state of clinical practice. The four different programs were described as follows (17):13

1. hypothetical program at the current HEW expenditure level (essentially $47 million), based on the current state of the art;
2. hypothetical program at an intermediate HEW expenditure level (roughly 2 1/2 times the current level), based on the current state of the art;
3. hypothetical program at an accelerated HEW expenditure level (roughly six times the current level), based on the current state of the art; and
4. hypothetical program for fiscal year 1975, at "an accelerated HEW expenditure level (roughly six times the current level), based on the expected advanced state of the art in 1975.

Within each of the resulting 16 analyses, attention was given to "a rational mix of program components" —prevention, diagnosis and treatment, research, training, and facilities. The fourth program, it should be noted, was based on numerous assumptions about scientific and clinical progress by 1975; it is highly sensitive to those assumptions and consequently its projections are not discussed below.

[13]Freeman recalls that the intermediate' projections were close to real numbers and the ' decelerated project ions were totally idealized

The estimated annual costs to HEW are shown in table 9 below. The totals for each of the four resource levels, respectively, are $47 million, $117 million, $290 million, and $293 million. The corresponding total annual national costs including HEW costs (not shown in the table) are $241 million, $333 million, $526 million, and $813 million. (All cost figures are in constant 1966 dollars.)

Short- and long-term benefits from these various expenditure levels were estimated. Short-term benefits were calculated for reductions in annual mortality, number of new cases, and

Table 9.— HEW Cost Summary (in thousands of dollars)

Program level	Cost by kidney disease category				Total	
	Infectious	Hypersensitivity	Hypertensive	End-stage	cost	Percent
Current expenditure level[a]						
Diagnosis, prevention, treatment:						
Prevention (including education and administration)	$3,803	$1,500	**$4,000**	—	**$9,303**	**19.920/.**
Diagnosis and treatment	—	—	—	$7,240	7,240	15.50
Subtotal	3,803	1,500	4,000	7,240	16,543	35.42
Research	4,000	5,250	3,800	12,100	25,150	53.85
Training	400	560	380	1,000	2,340	5.01
Facilities	1,000	170	1,000	500	2,670	5.72
Total	$9,203	$7,480	$9,180	$20,840	$46,703	100.00%
Intermediate expenditure level[b]						
Diagnosis, prevention, treatment:						
Prevention (including education and administration)	$ 5,929	$ 3,000	$ 8,057	—	$16,986	14.47%
Diagnosis and treatment	—	—	—	$ 30,000	30,000	25.56
Subtotal	5,929	3,000	8,057	30,000	46,986	40.03
Research	5,500	8,250	4,650	18,000	36,400	31.01
Training	750	750	500	5,500	7,500	6.39
Facilities	8,000	8,000	8,000	2,500	26,500	22.57
Total	$20,179	$20,000	$21,207	$56,000	$117,386	100.00%
Accelerated expenditure level[b]						
Diagnosis, prevention, treatment:						
Prevention (including education and administration)	$ 9,919	$ 3,000	$10,114	—	$ 23,033	7.94%
Diagnosis and treatment	—	—	—	$171,000	171,000	58.98
Subtotal	9,919	3,000	10,114	171,000	194,033	66.92
Research	6,500	10,125	5,500	24,000	46,125	15.91
Training	975	750	1,425	10,000	13,150	4.54
Facilities	10,000	10,000	11,600	5,000	36,600	12.63
Total	$27,394	$23,875	$28,639	$210,000	$289,908	100.00%
Accelerated expenditure level—1975[c]						
Diagnosis, prevention, treatment:						
Prevention (including education and administration)	$11,308	$43,000 / 13,000 [d]	$11,732	—	$ 76,040	25.94%
Diagnosis and treatment	—	—	—	$132,225	132,225	45.11
Subtotal	11,308	56,000	11,732	132,225	208,265	71.05
Research	7,410	12,450	9,500	1,500	30,860	10.53
Training	1,110	1,870	3,000	5,000	10,980	3.75
Facilities	11,400	10,000	11,600	10,000	43,000	14.67
Total	$31,228	$77,320	$35,832	$148,725	$293,105	100.00%

[a] Attributable to renal disease associated with hypertension.
[b] Current state of the art.
[c] Advanced state of the art.
[d] The $43,000 estimate is contingent on the development of a vaccine for hypersensitivity; $13,000 is the estimated cost of prevention without a vaccine.

SOURCE: *Kidney Disease Program Analysis: A Report to the Surgeon General* (Washington, D.C.: DHEW, July 1967).

days of morbidity. Long-term benefits were calculated in terms of eventual annual reduction in number of cases reaching ESRD. Long-term, though never specified, is essentially the 20th year of program effort. For ESRD, only short-term mortality reduction is shown, since that is all that is possible. The calculated benefits are shown in table 10.

What conclusions are to be drawn from the Burton study? First, additional resources in prevention beyond current levels promised little additional benefit in reduced deaths and rather modest additional benefits in reduced prevalence and morbidity. Second, the prevention of hypertension offered an important means for reduction of deaths from ESRD. Third, only the direct treatment of ESRD offered the best prospect for reducing deaths from that type of kidney disease. Although the Burton report itself nowhere reaches these conclusions in so crisp a fashion, careful perusal of table 10 provides the basis for these inferences.

Actually, the Burton report itself gives an impression somewhat at variance with the aforementioned conclusions. Figure 3 of the report, reproduced as figure 1, discusses the changing "state of the art" due to advances from scientific research. The clear implication is that better knowledge about preventing and treating primary kidney disease would cut deeply into the

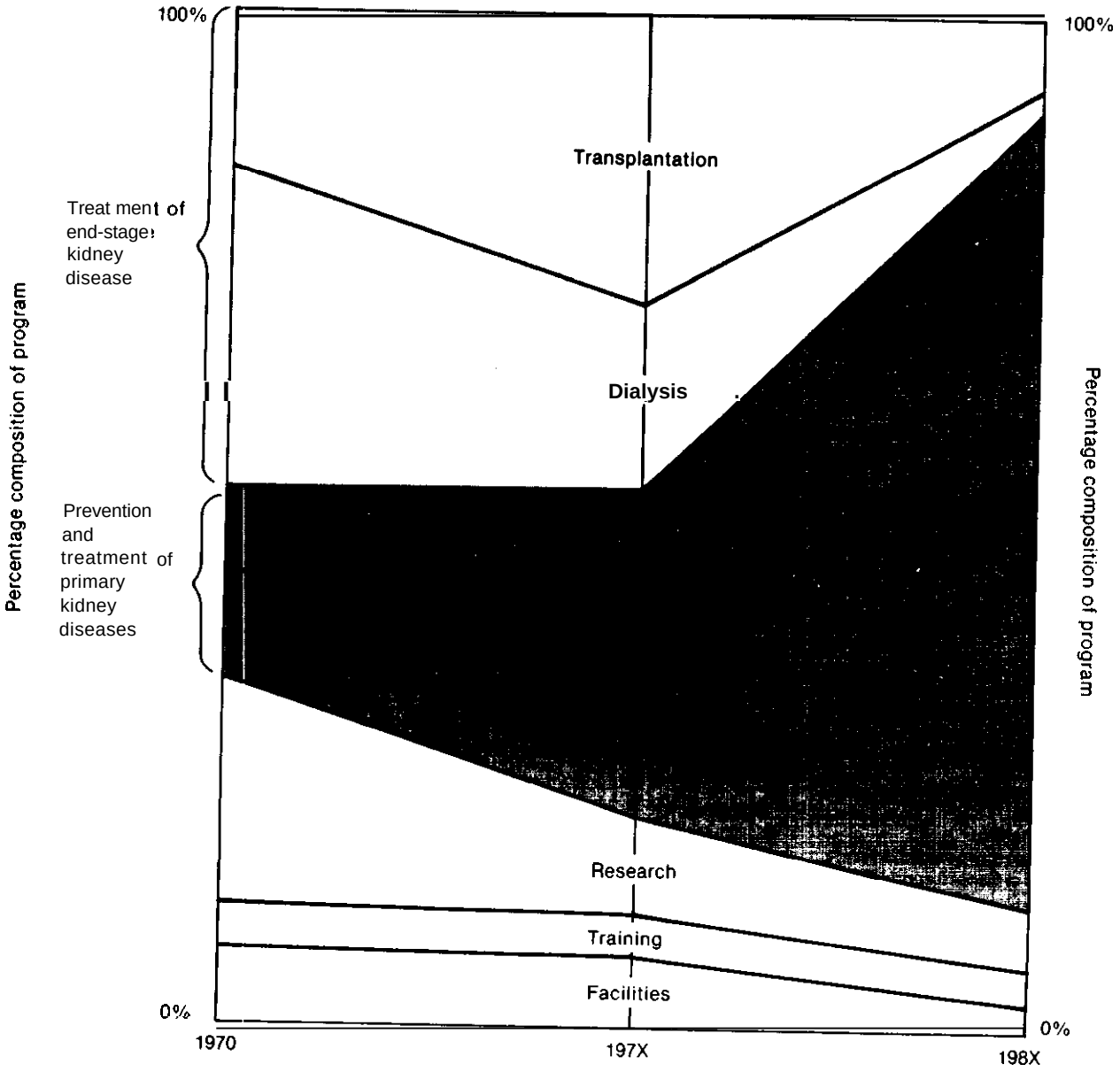
Table 10.—Program Benefits

Program level	Benefits by kidney disease category: Infectious	Hypersensitivity	Hypertensive	End-stage	Total
Current expenditure /eve/[b]					
Short-term benefit-reductions					
Mortality. ., ...	70 deaths	610 deaths	2,190 deaths	690 deaths	3,560 deaths
Prevalence	3,231,260 cases		27,000 cases		3,258,260 cases
Morbid days	15,962,420 days		1,802,000 days		17,764,420 days
Long-term benefit-reductions					
Annual	1,750 deaths		4,330 deaths		6,080 deaths
Cumulative.	25,850 deaths		86,560 deaths		112,410 deaths
Intermediate expenditure level[b]					
Short-term benefit-reductions					
Mortality.	70 deaths	610 deaths	2,270 deaths	1,560 deaths	4,510 deaths
Prevalence	3,243,860 cases		39,880 cases	—	3,278.740 cases
Morbid days.	16,273,640 days		2,056,820 days		18,330,460 days
Long-term benefit-reductions					
Annual	1,770 deaths		4,820 deaths		6,590 deaths
Cumulative.	26,190 deaths		96,300 deaths		122,490 deaths
Accelerated expenditure level[b]					
Short-term benefit-reductions					
Mortality. ., ...	70 deaths	610 deaths	2,380 deaths	7,675 deaths	10,735 deaths
Prevalence	3,292,860 cases		42,750 cases	—	3.335,610 cases
Morbid days	17,483,880 days		2,311,340 days		19,795,220 days
Long-term benefit-reductions					
Annual	1,870 deaths		4,820 deaths		6,690 deaths
Cumulative.	27,480 deaths		96,300 deaths		123,780 deaths
Accelerated expenditure level[c]					
Short-term benefit-reductions					
Mortality.	80 deaths	770 deaths	*9,300* deaths	27,399 deaths	37,549 deaths
Prevalence	5,630,720 cases	62.250 cases	289.690 cases		5,991,723 cases
Morbid days	26,064,430 days	2,610,000 days	5,578,860 days		34,253,290 days
Long-term benefit-reductions					
Annual	4,125 deaths	8,610 deaths	9,480 deaths		21,090 deaths
Cumulative.	76,500 deaths	320,000 deaths	189,660 deaths		586,160 deaths

Short term benefits – reduction in *annual* mortality, etc when program is fully operative
Long term annual benefits—evenlual *annual* reduction in number of cases reaching end stage kidney disease
Long-term cumulative benefits— sum total of long term annual benefits
[a] Renal disease associated with hypertension
[b] Current state of the art
[c] Advanced state of the art

SOURCE *Kidney Disease Program Analysis A Report to the Surgeon General* (Washington D C DHEW July 19671

Figure 1.— Effect of the Advancing State of the Art on Future Program Composition (percentages are wholly arbitrary and merely serve to illustrate shifting trends)



SOURCE. *Kidney Disease Program Analysis A Report to the Surgeon General* (Washington D C DHEW July 1967)

need to treat ESRD. That this development has not come to pass by 1980 (as the Gottschalk report's optimism about transplantation has also failed to materialize) should be a reminder to all about the dangers of relying on "inevitable" scientific advance in analyzing the costs and benefits of medical care.

The final chapter of the Burton report dealt with the cost of treating all patients threatened

by end-stage kidney failure, either by dialysis or transplantation. Though this issue was not part of the original charge to the group, the report argued, it was a logical corollary of the previous analysis. The policy question was stated in the report in the following way ***(17):***

> In any consideration of possible programs for the amelioration of the kidney disease problem, the overall expense of treating all patients threatened with a uremic death regardless of the possible costs, for humanitarian reasons, represents one extreme in a broad spectrum of possible programs. It should therefore be ascertained to serve as a maximal benchmark for any intensive attempt at program analysis or planning.

Unlike the Gottschalk committee, which took treatment as its point of origin, the Burton committee took it as one extreme in the range of possible programs.

The Burton committee assumed that 50,000 patients were vulnerable to ESRD year after year, of whom 10,000 could be treated by "conservative management" (primarily dietary control). Of the remaining patients, 11,000 were candidates for transplantation, ***2,000*** ideal candidates, and another ***9,000*** satisfactory. The assumption was made, therefore, that 29,000 new patients were available for and would be placed on dialysis each year.

Estimated annual outcomes for transplantation among the ideal candidates were that 80 percent would be "cured," 10 percent would fail and be placed on dialysis, and 10 percent would die; the corresponding values for satisfactory candidates were 30, 30, and 40. For the dialysis patients, it was estimated that 50 percent would die in the first year, and there would be a 90-percent survival rate for those remaining in each successive year.

Costs for the first 15 years of the program were estimated by the Burton committee using "present cost" figures of $16,000 for a transplant operation and $15,000 per year for dialysis treatment. (No home dialysis was projected.) Based on first and successive year cohorts of 40,000 patients, the projected costs were:

1st year	$611 million	40,000 patients
5th year	$1,044 million	102,000 patients
15th year	$2,702 million	179,000 patients

Having laid out a program for treating all those vulnerable to kidney failure and having developed the corresponding cost estimates, the Burton report then *limited* the domain of applicability of its analysis. It noted that "supply" constraints of trained personnel and facilities would limit the rate of program buildup. More significantly, it reduced the "demand" substantially from 40,000 vulnerable to an annual cohort of 8,000 to 10,000 individuals ideally suited for dialysis and transplantation.

Finally, on the penultimate page of the Burton committee's report to the Surgeon General, it was noted that (17):

> With this general framework in mind and utilizing the elements and computations in Section V of Chapter 5, it is possible to arrive at practical and feasible predictions concerning the cost of such a program and the overall number of patients that could be accommodated by it during each of the first few years, the number of patients who are likely to graduate from this program permanently each year because of successful transplantation, the number of individuals who would have to be maintained permanently with the aid of chronic dialysis, and the number of new patients which such a program could accommodate each year.

Having calculated costs for the much less likely 40,000-patient annual cohort, however, the report stopped abruptly without making any calculations for the smaller, more probable, 8,000 - to 1(),000-patient cohort!

Overall, the Burton committee report had a number of problems, some of which were stylistic. First, there was a somewhat naive belief in "decisions based upon a thorough, dispassionate, and logical analysis" and in the possibility of allocating resources "on the basis of logical priorities. " Second, the 16 different scenarios make the analysis entirely too complex to be of much use by policy makers. Third, each scenario is highly complex in its own right, built upon numerous assumptions about the clinical feasi-

[a] "A future cost projection, using $12,000 for each transplant and $ 10,000 for dialysis per year was also made, on the assumption that costs would decline over time due to increased efficiencies in providing treatment

bility of the various components or forms of intervention, the appropriate "mix" of components, and the relative shares of Federal and non-Federal funds. Fourth, the narrative tends toward a repetitious presentation of calculations based on certain assumptions, justified by relatively little description of clinical feasibility, and often supported only by citations to "best estimate" or "informed medical judgment ."

The Policy and Bureaucratic Contexts

The Gottschalk and Burton committees worked at approximately the same time; they addressed similar subject matter; and their reports were issued simultaneously. It is worthwhile to compare and contrast the two committees, and their respective reports, and the policy and bureaucratic contexts in which they operated.

The two committees differed in their initial stimulus and central purpose. The Gottschalk committee was a direct response to the existence of expensive, lifesaving therapies developed largely from publicly financed medical research and to the mounting pressures to make those treatments available to the general public. That committee was not asked to address the question of whether treatment for ESRD should be provided relative to alternative uses of resources; it was asked how such treatment should be made available if the basic policy choice was to go forward.

The Burton committee arose from the general effort within HEW to use program budgeting, systems analysis, and CBA for allocating resources to major programs. In general, the commissioned studies were undertaken in order to provide guidance for HEW's 5-year program and financial plan and for fiscal year 1969 budget and legislative proposals. An attachment to a memorandum from Secretary Gardner stated, "Of particular importance this year is the question of kidney diseases. The future of Federal support for hemodialysis, transplantation, and prevention needs decisions based on thorough analysis" (29). No further clarification of purpose was given to the committee, nor did any subsequent interaction occur between the committee and the Surgeon General, the Assistant Secretary for Health, and the Assistant Secretary for Planning and Evaluation which might have sharpened the focus of the committee's study.

The two committees differed in composition. The Gottschalk committee's members and consultants included three renal physiologists, two nephrologists, an immunologist, a transplant surgeon, a psychiatrist, two economists, a lawyer, a biostatistician, and a professor of ethics. These people came primarily from universities, and especially academic medicine, but also from Government and the private practice of law. The Burton committee's members and consultants included one nephrologist, the director of the National Institute of Arthritis and Metabolic Diseases' (NIAMD) artificial kidney program, the director of the National Institute of Allergy and Infectious Diseases' transplant immunology program, two professionals from the PHS kidney disease control program, several other PHS officials, and three operations analysts. Members were drawn exclusively from the Government, consultants from a nonprofit research firm, Scientific and clinical competence was greater on the Gottschalk committee.

The duration of the two committees differed substantially. The Gottschalk committee met first in mid-1966 and a total of seven times over the next year. A draft report was circulated for comment in the summer of 1967 and a final report was presented to the Director of BOB that September. The Burton committee met initially in late March 1967. Its final report was transmitted to the Surgeon General in late July, only 4 months later.

In substantive terms, the reports generally agreed about the value of research, differed somewhat on the value of prevention, and differed greatly on the treatment of ESRD. The Gottschalk committee concluded that "continuing and expanded research in the fields of transplantation immunity, organ procurement and storage, and dialysis is essential for optimal development of these methods of treatment" (31). The Burton committee, less directly but more frequently supported research as critical to dealing with kidney disease. It tended to go beyond a limited endorsement, however, and suggested

that research was likely to generate substantial future reductions in deaths from ESRD.

On prevention, the Gottschalk committee declared that a screening program for pyelonephritis, glomerulonephritis, and polycystic kidney disease made very little sense. The Burton committee essentially said the same thing, but couched its findings in language which obscured that conclusion. The Burton committee did, however, emphasize the prospective benefits in reduced deaths from ESRD due to prevention of hypertension, a matter on which the Gottschalk commit tee was silent.

It was on the issue of treatment for ESRD, however, that the two committees diverged most sharply. The Gottschalk committee used the treatment of ESRD as its point of departure. That committee focused exclusively on patients who were dying from chronic kidney failure, hence candidates for treatment by hemodialysis or transplantation. The two forms of therapy, its report declared, were "sufficiently well advanced today to warrant launching a national program" (31). For the Burton committee, by contrast, a universal treatment program represented one "extreme" of policy possibilities, the "maximal benchmark" of how far the Government might go.

The Gottschalk committee estimated 3,662 patients in the first year of a treatment program, increasing to 18,500 in the fifth year. The corresponding costs were $40 million to $49 million for the first year, and $157 million to $205 million in the fifth. Cost estimates were based on the assumption that half of all dialysis pati - nts were treated at home at an annual cost of $4,000 (low estimate) or 5,000 (probable estimate). The estimated annual costs for center dialysis ranged from $10,000 (low estimate) to $14,000 (probable estimate). Roughly 20 percent of the total beneficiary population, i t was estimated, would receive a transplant, the costs of which ranged from $10,000 to $13,000. Estimates about the patient population (table 8) explicitly assumed that growth would be constrained by available facilities.

The Burton committee established the costs of a treatment program for 40,000 first-year patients at $611 million, and of a fifth-year population of 102,000 at slightly more than $1 billion (in 1967 dollars). In fact, as noted above, the committee's report to the Surgeon General indicated that an annual cohort of only 8,000 to 10,000 individuals would be serious candidates for a treatment program "in the near future" (17). Neither patient population nor associated cost estimates for this most likely annual patient cohort were developed in the report, however, so the astronomical cost estimates based on the unlikely 40,000 annual patient cohort stood by implication as the committee's contribution to the policy discussion.

These discrepancies between the Gottschalk and Burton committee reports might have been unimportant t save for the circumstances of their release to the public. Initially, BOB had planned to keep the Gottschalk committee's report secret and present it only to the BOB Director. News of the committee and its work leaked out to the press, however, resulting in some publicity in April 1967 and the certainty that the final report could not remain secret (38). But BOB itself, confronted with the fiscal implications of a national ESRD treatment program, and increasingly feeling the effect of Vietnam war demands on funds for the Great Society programs, walked away from the Gottschalk committee's report.

BOB's retreat from the Gottschalk report was manifested in several ways. Dr. Burton recalls that BOB was unwilling to publish the report, and he authorized payment for publication of .500 copies out of the budget of NIAMD's artificial kidney program. The cover of the report carried only the title and date, Report of the Committee on Chronic Renal Disease, September 1967. Inside was a transmittal letter from Dr. Carl Gottschalk to BOB Director Charles Schultze, but no official acknowledgment of receipt by the Director. Finally, the manner of the report's release revealed BOB's desire to put distance between itself and the report from its commit tee of experts.

The Burton report to the Surgeon General was completed in late July, the Gottschalk report to BOB in mid-September. The political

question became how to release these reports in a way to minimize public pressures for an expanded ESRD treatment program. A "low key" approach was agreed on, and strategy was coordinated between Joseph Califano on the White House staff, BOB Director Schultze, and HEW Secretary John Gardner (3). The two reports were released simultaneously at a PHS press conference on November 3, 1967.

The press conference itself engendered some hard feelings. Dr. Gottschalk was notified of the press conference by telephone in Chapel Hill, N. C., only 12 hours before it was scheduled, and he was unable to attend. Dr. Robert Berliner, then intramural research director of the National Heart Institute, and technically an "adviser" to the Gottschalk committee, was present to represent the expert committee. Since BOB had refused to take any position on the Gottschalk report, however, Berliner was unable to make other than perfunctory comments about it. Dr. Burton, on the other hand, was under fewer inhibitions regarding the report from his committee. The reporter who covered the story for Medical World News wrote that the feature of the briefing was not the report of the Budget Bureau's expert committee, but the PHS report to the Surgeon General which emphasized research on preventing kidney disease rather than treating it. Dr. Burton reportedly called the Gottschalk committee report "shortsighted." "In the long run, " he was quoted as saying, "we want to get rid of the infectious mechanisms leading to kidney disease" (6). "

Policy and Program Effects

What were the policy and program effects of these two formal analyses? The Burton report had little direct effect on PHS policies or programs in kidney disease. For one thing, the Surgeon General's charge precluded the study group's making any recommendations. In general, the Burton report reinforced NIH research programs related to dialysis and transplantation and supported PHS efforts in prevention. It countered, to some degree, the pressures from within the PHS kidney disease control program to promote a greatly expanded treatment program.

The Burton report was well received by William Gorham, the HEW Assistant Secretary for Planning and Evaluation, by Dr. Alice Rivlin, his deputy, and by Dr. Philip R. Lee, the Assistant Secretary for Health. Others in HEW were less responsive. Dr. Burton recalls briefing Secretary Gardner on the study for 1 1/2 hours shortly before the Secretary's resignation in early 1968 and receiving only a noncommittal "Thank you very much" in return. Robert Grosse, an economist involved in HEW's effort to improve resource allocation by using formal analysis, described the kidney disease group's work favorably but uncritically in several papers (11,12,13).

One effect of the Burton report, noted above, was to engender hard feelings among some members of the Gottschalk committee and those in the medical community who were favorably disposed to the Gottschalk report's recommendations. These hard feelings arose from the belief that the Burton study group, by its implicit attack on a national treatment program and by the circumstances surrounding the public release of the two reports, had sought to sabotage the report of the Gottschalk committee.

Three factors contributed to this impression of sabotage. First, the reasons behind the PHS study were never clear. Why study kidney disease when it was known that a BOB committee was at work on the subject, many reasoned, if not to prevent PHS from being outflanked on an issue of some importance to it? PHS was not entirely clear on this point. It recognized that the real policy problem was the treatment issue, but did not address this matter directly in the charge to the study group. The PHS study group, moreover, acknowledged that the cost of a national dialysis and transplantation treatment program was beyond its charge, but nevertheless chose to conclude its report on that subject. The purposes and motives of PHS were thus called into question.

"Burton recalls speaking of Immunological" mechanisms in connection with glomerulonephritis

Second, BOB's motives and behavior in walking away from the report by its committee of experts were not widely understood. Irving Lewis recalls the occasion when Dr. Gottschalk briefed BOB Director Schultze on the report: Schultze praised the work of the committee and its report, but said that resources for new domestic initiatives were increasingly scarce as a result of the impact of the Vietnam war (22). BOB, consequently, sought to dampen the impact of the Gottschalk committee's report in the ways described above.

Finally, hard feelings arose from the absence of communication between the two committees. Interaction between the two groups did occur through several formal and informal means, but these were limited in scope and duration. A single meeting of the two committees in the spring of 1977 would have clarified for each other their respective missions and perhaps would have facilitated a more clearly understood division of labor.

The Gottschalk committee and its report recommending a national ESRD treatment program had greater effects than the Burton committee and its report, because the Gottschalk committee was a group with high status in academic medicine, and advisory to BOB in the Executive Office of the President. The committee's effects were of four kinds. The first was to neutralize opposition to dialysis and transplantation within the academic medical research community by making clear that these therapies were established and not experimental and by convincing the community that the patient load, and thus the economic burden, was predictable. Dr. George Schreiner recalls that representatives of foreign countries who testified at the committee's second meeting, held at the same time as the International Congress of Nephrology, all reported that the incidence of good dialysis candidates each year was in the range of 56 to 58 patients per million population. Certain predictabilities existed. The committee's work, Schreiner remembers, "gave a much broader vista to the scientific types like Gottschalk, Seldin, and Berliner" (40).

The converse effect of the Gottschalk committee's work was to sanction the work of clinicians who were treating patients. Controversy existed throughout the early 1960's about whether dialysis and transplantation, especially the former, were experimental or established treatment. The report of the Gottschalk committee declared that dialysis and transplantation were "capable of prolonging life" and were "sufficiently well-advanced today to warrant launching a national program, " The impact of this declaration was summarized several years ago by one prominent clinician in this way: "It was not until 1967 and the Gottschalk report that you had for the first time an expression of national opinion that dialysis and cadaveric transplantation were therapy" (39).

The third effect of the Gottschalk report was on the VA's renal program, which had precipitated the formation of the committee. There was created within VA a home dialysis program which came to dialyze a significant portion of the veteran dialysis patients. A secondary effect on VA was to encourage the development of its transplantation program in a select number of VA hospitals and to encourage the advocacy of transplantation programs within the PHS program as well.

The question arises about the effect of the Gottschalk report on subsequent legislation, especially upon the extension of medicare coverage to ESRD through the inclusion of section 2991 in Public Law 92-603, the Social Security Amendments of 1972. After the committee's report, several bills that incorporated the report's recommendations were proposed in Congress, but none was enacted. In 1970, the heart disease, cancer, and stroke legislation was amended to include kidney disease, the only legislative enactment on the subject occurring before Public Law 92-603.

Dr. George Schreiner, member of the Gottschalk committee, served as president of the National Kidney Foundation from 1969 through 1971. In that capacity, and aided by Charles Plante, Washington representative of the foundation, Schreiner used the report, in his words, "as a two-by-four to get the mule's attention. " In numerous one-on-one discussions with individual members of Congress and their staff, the Gottschalk report was cited as evidence of the

importance of treating ESRD. That report, therefore, helped clinicians advocating a treatment financing effort. Schreiner was emphatic, however, that legislation would never have occurred because of the report (40).

Within the Bureau of Health Insurance of the Social Security Administration, Irwin Wolkstein, Deputy Director for Program Policy, remembers reading the Gottschalk report and talking about it with Klarman. The Bureau of Health Insurance as an organization, however, had deep reservations about extending health insurance coverage on a categorical disease basis. The Gottschalk report per se generated no advocates for its recommendation within medicare (45). Neither the staff of the House Ways and Means Committee nor the Senate Finance Committee had read the report at the time of the 1972 legislation.

The 1972 legislation, however, did extend medicare coverage to those with ESRD under the general extension of coverage to the disabled. In this respect, the statute was consistent with the Gottschalk report's recommendation. The statute and implementing regulations for the medicare renal program differed in two respects, however, from major recommendations of the report. First, whereas the Gottschalk committee recommended a strong emphasis on home dialysis, the medicare program inadvertently introduced disincentives to that form of treatment (32). Second, the report recommended a Federal investment in the "supply" side of treatment facilities and trained medical professionals. In 1972, though the needs on the supply side were less than those 5 years earlier, no consideration was given to this Federal assistance, and the statute focused exclusively on augmenting the "demand" side by financing patient care.

In summary, the Gottschalk committee report had little direct effect on the 1972 resolution of the policy question of financing patient care. The effects on the scientific and clinical communities, of course, may have indirectly influenced the subsequent course of events.

CONCLUSIONS

What general conclusions can be drawn from this study about the use of formal analysis in policy formulation? First, policy toward ESRD was always governed by two rudimentary facts concerning treatment for ESRD: Such treatment was costly and lifesaving. These facts were unchanged by analysis; nor did analysis alter perceptions of what policy ought to be. Some shrank from the large cost implications of a national treatment program: How can we afford such an expensive effort for so few? Others reacted to the benefits: How can we withhold lifesaving therapy simply because people lack the means to pay for it? Analysis did not alter the fact that the policy issue was a basic political choice between scarcity on the one hand, and the priceless value of life on the other (s).

Second, it is important to recognize that expectations of progress infuse practically all domains of science and technology, including medicine, Indeed, in this analysis, we witness several instances of underlying optimism about probable developments in medical research and practice. The Gottschalk committee was overly optimistic about transplantation, the Burton committee about research and prevention. If analysis is based on limited and inadequate data, as it usually is at the early stages of policy formulation, it is then quite difficult to separate realistic from overly optimistic expectations about the future. A critical review of basic assumptions by experts not involved in the analytical effort may be the best corrective to this source of bias.

Third, formal analysis on allocating resources for lifesaving therapy cannot be done in secret. Though the original intention of BOB was to secure expert judgment from the Gottschalk committee on a confidential basis, leaks to the press, the resultant publicity about a "secret" effort, and congressional inquiries eventually forced the analyses to be released (7). PHS, on

the other hand, always intended to release the Burton committee's report, and its intentions placed further pressure on BOB to release the Gottschalk report. It is the case, however, that the Government has substantial discretion in how it releases reports, and can choose to do so in ways that enhance or diminish the impact of recommendations.

Fourth, the absence of major policy action in response to the Gottschalk and Burton reports does not mean that these efforts had no impact. Certainly, these reports did raise the consciousness of policy makers at the highest levels of Government to the substantial cost implications of action, Given that the Vietnam war was absorbing increasing proportions of national resources at the time, it is not surprising that nothing happened.[16] The effects of greater eventual policy consequence were those on the scientific and clinical communities, including strengthening clinician advocacy on behalf of treatment financing.

Fifth, analysts have their greatest effect when they have direct access to key policy makers (23). The expert advisory committee to BOB had access to the BOB Director, to the Office of Science and Technology, and to the White House. The members of the PHS study group had access mainly to the higher officials in HEW. An additional, and closely related, point is that analysis has its greatest effect when policymakers wish to use it and have access themselves to instruments that affect policies and programs. The influence of the Gottschalk committee report, in large measure, was confined to VA, because BOB could influence VA directly through the budgetary process and because it consciously shrank from the report's larger implications.

Finally, it should be noted that analysis done for purposes of policy formulation is likely to be limited by a number of factors. The statement of the problem will control the analysis and its outcomes. Data are likely to be inadequate, especially for issues just coming to policy prominence. Assumptions are required on critical issues. Estimates are based on assumptions and inadequate data. Normative preferences will be found explicitly or implicitly throughout the analysis. Projections over time are highly sensitive to the estimates, assumptions, and data. Uncertainty may inhere in clinical practice which cannot be reduced by formal analysis but only by the increase of scientific knowledge. Given these limitations, the large conclusions of formal analysis are likely to be most important to policy formulation if the estimates, assumptions, and data appear to be reasonable. Formal analysis may augment policymaking; it cannot substitute for it,

[16]Gottschalk recalls that BOB officials told him early in the work of the committee that the analysis was undertaken, in part, to create a policy option in the event peace arrived in Southeast Asia. Lewis has no such recollection".

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