

Appendix C.—Abstracts of Selected Entries From the Bibliography on CEA/CBA

Baker, C., and Way, L., "Clinical Utility of CAT Body Scans," *Am. J. Surg.* 136:37, July 1978.

This CEA of computed axial tomograph (CAT) body scans employs an efficacy scale which ranges from 1 point (given when the scan is deemed to have saved a patient's life) to 18 points (given when the scan is held to have led to a patient's death). In the course of the analysis, the sensitivity, specificity, and accuracy of CAT body scans are evaluated. The authors note that less expensive tests, most notably ultrasound, are bypassed or performed simultaneously with CAT scans. Analysis indicated that ultrasound and CAT scans are of about equal clinical value in any given situation, but ultrasound costs one-fourth as much as CAT scanning. The authors observe that clinicians, when employing CAT scanning, often seem to have no clear expectations that it can affect patient management. They also note that for most conditions about which CAT body scans are informative, insufficient information is not the major factor limiting the success of therapy. Though this study, limited to hospitalized patients, would have missed any decreased admissions for diagnostic tests which may have resulted from the use of CAT body scanning, its authors believe that few savings can be expected from replacing other diagnostic procedures with CAT scans. They recommend that CAT body scans be ordered only if 1) more information would truly affect patient management, 2) more cost-effective diagnostic tests have failed, and 3) the likelihood of disease is high.

The authors caution that their study was done as CAT technology was rapidly evolving. This evolution has obvious implications, including the likelihood that current use patterns (frequency and motivation) differ from what they will become if and when body scanning becomes standard practice. As such, the study fails to distinguish between cost effectiveness today and in a steady-state situation in the future. In addition, the study does not identify potential cost efficacy (i. e., cost effectiveness under optimal conditions). Despite these drawbacks, this study stands out as one of the very few which have attempted to identify and quantify patient management and health outcomes.

Barnes, B., "Cost-Benefit Analysis of Surgery: Current Accomplishments and Limitations," *Am. J. Surg.* 133:438, April 1977.

The general principles of CBA are presented, with a "good" program described as one in which the net discounted benefits exceed zero. The author says that CBA was first applied to health care in response to rapidly rising medical care expenditures. When conflict between individual and societal interests is discerned, the techniques of CBA **must** be applied with sensitivity to the individual and public interests involved. Limitations of CBA in health

care include difficulties in accurately accounting for the numerous complex costs and benefits encountered, in identifying and valuing long-range effects, and in determining a discount rate when costs and benefits are deferred many years.

CBA is described as applicable only where effects are nearly equivalent, so that the analysis becomes, in effect, a cost comparison. (However, one can also look at different effects resulting from equivalent costs.) Three examples of CBA as applied in health care are presented: 1) consideration of cholecystectomy for silent gallstones, 2) renal transplantation or chronic hemodialysis for end-stage renal dialysis, and 3) intensive care unit support for different illnesses.

The author states that the accomplishments of CBA and related techniques in health are largely those of more comprehensive understanding of the advantages or disadvantages of a particular therapy or policy. In itself, CBA is seldom definitive, but in conjunction with political and professional judgments, it can improve decisionmaking.

Bartlett, J., et al., "Evaluating Cost Effectiveness of Diagnostic Equipment: The Brain Scanner Case," *Br. Med. J.* 2:815, Sept. 16, 1978.

The bulk of this article is devoted to a comparison of the costs involved in five different options for implementing CAT scanning in a region of England. The net costs of CAT scanning are calculated as gross costs (e.g., purchasing, installation, staffing, etc.) minus savings from the decreased use of conventional neuroradiology and reduced bed days, presumed to result from the introduction of CAT. The article also includes a discussion of possible treatment improvements, unquantified and not included in the cost of calculations, that may result from the use of CAT. There is little discussion of the cost effectiveness of CAT scanning v. conventional neuroradiology, though the analysis of the five CAT implementation options seems based on the premise that CAT is more cost effective in certain circumstances. The authors acknowledge the lack of precision and uncertainty involved in the savings calculations, but contend that some savings do result from the introduction of CAT and must be assessed in any analysis.

Bennett, W., "Cost-Benefit Ratio of Pretransplant Bilateral Nephrectomy," *J.A.M.A.* 235:1703, Apr. 19, 1976.

This paper is an example of how titles can be misleading. Despite the title, there is not a single cost-benefit ratio in the entire article. The author compares the posttransplant course of patients who previously had had their kidneys removed to that of patients who had had no pretransplant surgery. The latter group experienced fewer rejections and better survival.

NOTE: Three types of entries are abstracted 1) many of the better known studies or methodology articles, 2) several examples of articles of varying technical sophistication (that is, a sample of the general literature) and 3) most of the case studies prepared or supported by OTA as part of the overall assessment (see app. D) Inclusion here does not imply that any particular study is one of the "best."

Bentkover, J. D., and Drew, P. G., "Cost-Effectiveness/Cost-Benefit of Medical Technologies: A Case Study of Orthopedic Joint Implants," in *The Implications of Cost-Effectiveness Analysis of Medical Technology/Background Paper #2; Case Studies of Medical Technologies*, prepared by OTA, U.S. Congress (Washington, D. C.: in press, 1980).

This study examines the feasibility and potential usefulness of undertaking CEA/CBA of orthopedic joint prostheses. Two specific issues are addressed: 1) whether it is feasible to evaluate carefully and completely the orthopedic joint implant technology within a CEA/CBA framework; and 2) how could such an evaluation be useful in formulating public policy.

The authors present a state-of-the-art study of CEA/CBA as it pertains to this technology. They do not try to assess the technology. The study includes a description of the technology (joint implants) based on a review of the literature, communications with selected medical specialists, and conversations with representatives of the orthopedic prostheses industry. The authors briefly discuss alternative forms of treatment for arthritis and point out an important difference between the alternatives (e. g., drugs) and joint implants: Most alternatives are only short-run measures, whereas joint implantation is a long-term measure.

Few data are available regarding the efficacy of joint implants. Data regarding the efficacy of hip replacements are better than the data for other joint implants or alternative measures. They may even be acceptable. Efficacy studies are in progress for some implants. The authors did not explore the possibility of producing the result (successful joint implantation) in the most efficient manner possible.

Potential benefits were put into two categories: direct and indirect. Potential direct benefits discussed include relief of pain, improved functional status of joint, measures included in the "Sickness Impact Profile" (e. g., social interactions, ambulation, sleep, leisure, and emotions), quality-adjusted life years (QALYs), and earnings. Potential indirect benefits include averted expenditures for the caring for, and treatment of, individuals handicapped with debilitated joints, e.g., those with severe arthritis. These potential benefits were only mentioned; none were quantified or measured.

Most costs mentioned were not distinguished from charges, and "avoidable" costs are not specifically identified. Some indirect costs, e.g., loss of productivity when patient is hospitalized, were identified. The author points out that both indirect and direct costs of complications associated with joint implants must be included as well as the costs of followup care and rehabilitation therapy.

The authors note that all projected benefits and costs should be discounted, but do not suggest any discount rate in particular. They do suggest that variables with uncertain values, e.g., discount rates, be subjected to a sensitivity analysis. Equity issues are not considered. The authors briefly mention some potential public policy implications of conducting CEA/CBA of orthopedic joint implants but their study does not contain specific results regarding the cost effectiveness of orthopedic joint implants.

Budetti, P., McManus, P., Barrand, N., and Heinen, L. A., "The Costs and Effectiveness of Neonatal Intensive Care," in *The Implications of Cost-Effectiveness Analysis of Medical Technology/Background Paper #2: Case Studies of Medical Technologies*, prepared by OTA, U.S. Congress (Washington, D. C.: in press, 1980).

This paper includes a review of the efficacy and effectiveness literature, as well as the "cost" and cost-effectiveness literature, on neonatal intensive care services. The authors note the rapid progress made in this area in the last is years, emphasizing the range and sophistication of care that hospitals can now offer. Their study centers on an examination of costs, personnel, technologies, and procedures used, and the efficacy and effectiveness of the intensive care services designed to provide advanced care to severely ill newborns.

Numerous problems involved in analyzing neonatal intensive care services are identified. First, the definitions are very tenuous. Neonatal services in many hospitals do not fit into the classifications used. Technological or personnel capabilities vary considerably in different hospitals, and regulatory and reimbursement policies create incentives for hospitals to classify their neonatal units inappropriately. Providers, paying units, and regulators disagree on uniform definitions that should be applied to different levels of care.

The major focus of the study is on efficacy, effectiveness, and costs of neonatal intensive care. Outcomes are defined in terms of improved mortality and morbidity rates and mental and physical development of critically ill newborns. Costs are distinguished from charges. The study addresses the average cost per day of caring for the critically ill newborn and reimbursement policies and procedures. No discount rate is used. Except in a very rough estimate of high and low figures for use and the cost effectiveness of caring for different birthweight infants, sensitivity analysis is not applied. Equity issues are not addressed.

The authors examine the incidence and severity of prematurity in the United States. They evaluate the social and biological aspects of prematurity, trends in infant mortality, and the incidence of underweight infants in the last two decades, and the effect of neonatal intensive care units (NICUS) on mortality and morbidity of premature infants at various birthweights. They also examine the use of NICUS via admission rates, estimated average length of stay, estimated total patient days, the number of hospitals with NICUS, and the number of intensive care beds.

Next, the authors examine the costs of neonatal intensive care, providing a caveat that the data on use and cost are very rough approximations. The authors derive the cost and use data from small, restricted population samples drawn from NICUS that vary in size, shape, and capability. Data were examined from three geographic regions and five individual centers, along with numerous studies on neonatal care. Costs associated with varying degrees of prematurity and severity of illness were examined, as well.

In general, costs are directly related to birthweight and prematurity—the lower the weight and/or the earlier the

birth, the higher the cost. The average cost per day in the hospital for critically ill newborns is \$267 for an average stay of 13 days. The average charge per day was about \$394. The study looks at the existing system of reimbursement for the cost of neonatal intensive care in five States and via five payers: commercial insurance, Blue Cross, medicaid, self-pay, and private insurance.

NICUS have been shown to reduce mortality rates, and all indications are that NICUS are cost effective. Nevertheless, more data is needed to determine their full impact. The authors review studies of the cost effectiveness or benefit of neonatal intensive care. They use a hybrid cost-effectiveness and cost-benefit analysis developed by Marcia Kramer to measure marginal costs of providing neonatal intensive care. They also compare methods of care in Great Britain and France with those in the United States.

The authors suggest that Federal policies need to be changed to reflect changes that have occurred in neonatal care. In particular, they suggest, guidelines that establish maximum numbers of beds per live births and minimum sizes of neonatal care units need to be revised. Also, medicaid and Social Security provisions for reimbursement of neonatal care costs need to be reexamined. The potential ethical implications of neonatal intensive care need more discussion.

Cretin, S., "Cost-Benefit Analysis of Treatment and Prevention of Myocardial Infarction," *Health Serv. Res.* 12:174, summer 1977.

This article, technically a CEA, compares the effects of three alternative methods for the treatment or prevention of myocardial infarction: 1) a coronary care unit, 2) a mobile coronary care unit, and 3) an intervention/prevention program aimed at reducing serum cholesterol levels. Effects are measured in terms of the total years of life added as a result of each alternative program. *Costs* are classified as direct and indirect. Costs and effects of each strategy are modeled on the basis of a cohort of 10-year-olds followed throughout their lifetimes. In addition, the manner of implementation is varied. Costs and effects are calculated for each alternative method assuming 1) the method is newly introduced alone, and 2) it is newly introduced with the other alternatives ongoing. "Cost-benefit" ratios are calculated as the dollar cost per added year of life for each alternative, introduced alone. The author illustrates changes in the ratios that result from varying the discount rate (i. e., performs sensitivity analysis), using rates of 0, 5, and 10 percent. She also discusses problems of selecting a discount rate for comparing alternative programs that incur costs and accrue benefits at widely separated times. The author finds the results of her analysis inconclusive. She notes that this and other modeling processes involve many simplifying assumptions and require that parameter values be estimated even when supporting data is scant.

Doherty, N., et al., "Cost-Effectiveness Analysis and Alternative Health Care Programs for the Elderly," *Health Serv. Res.* 12:190, summer 1977.

In this article, the authors discuss the method of CEA in general, contrast it with CBA, and illustrate it with an ex-

ample involving alternative programs of health care for the elderly (e. g., home care, day care, etc.). They also discuss the problems of measuring costs by market prices which may "obscure the real opportunity costs of resource consumption." With regard to effectiveness criteria, the authors note that many can be specified only in terms of ordinal numbers denoting rank, and they warn against the temptation "to add the nonadditive and to *compare the* incomparable." Costs in the analysis presented as an example are classified as primary, secondary, and tertiary, denoting program costs, other health-related service costs, and personal living expenditures, respectively. The authors explain and illustrate the tabular display approach to presenting data, in which effectiveness criteria are presented in columns and alternative programs are presented as row headings. It is unlikely, the authors conclude, that one alternative will emerge as preferred on the basis of all relevant criteria. In their example, day care is preferred on the basis of effectiveness criteria, while home care is preferred on the basis of cost criteria.

Eddy, D., "Rationale for the Cancer Screening Benefits Program Screening Policies: Implementation Plan, Part III," report to the National Cancer Institute, Blue Cross Association, Chicago, Ill., 1978.

This report describes the methods used to analyze the cost effectiveness of alternative cancer screening policy options and the rationale for a recommended insurance benefits program. Five cancer sites—breast, colon, cervix, lung, and bladder—were selected for full analysis. The model used translates the problem of screening-program effectiveness, and many variables that contribute to it, into quantitative terms and logical relationships. Probability formulas relating to the important variables are derived. The model, designed to be programed on a computer, traces the expected fate of a patient under various program options. It will accept information about patient characteristics (age, relative risk, previous history, incidence rates, etc.) and will program options and present information on the costs and effectiveness of a specified program. Different discount rates can be entered into it.

The author notes that creating a cancer screening program that is both medically effective and low in cost requires that many age, sex, and risk categories be used to define the optimal services and screening frequencies for various groups of individuals. Ideally, a program might include several screening protocols, each tailored to different categories. This is not possible, however, for a prepaid benefit program that will be purchased by a large heterogeneous population. Thus, one objective is to design a benefit program in which services do not vary greatly for those covered. Marginal effectiveness, rather than absolute effectiveness, was considered the effectiveness criterion, and on this basis, there is little difference in the cost-effective program for high-risk as compared to average-risk groups. The benefit program designed includes the following provisions: 1) a standard screening program will be provided every 4 years for persons aged 25 to 45; 2) a standard screening program will be provided every year for those over age 45; 3) an impregnated guaiac slide will

be provided every year beginning at age 45; 4) a Pap smear will be provided to women every 4 years beginning at age 25; 5) a mammography will be provided to women covered by the high-option benefit every 2 years beginning at age 50; 6) a proctosigmoidoscopy will be provided every 5 years beginning at age 50.

Eddy, D., "Screening for Colon Cancer: A Technology Assessment," in *The Implications of Cost-Effectiveness Analysis of Medical Technology/Background Paper #2: Case Studies of Medical Technologies*, prepared by OTA, U.S. Congress (Washington, D. C.: in press, 1980) .

This study focuses on the techniques that are available to screen for colon cancer—their development, evaluation, use, and cost effectiveness.

The author examines the three basic methods of techniques used in the detection of colon cancer: 1) the digital exam, 2) the sigmoidoscope, and 3) the test for occult blood in the stool. For each method, the author notes, there is either some degree of uncertainty regarding the sensitivity and specificity of the tests, or some degree of risk to the patient involved.

The study points out that there have been few, if any, clinical studies of the digital exam. Its effectiveness has been proven via the "time-honored" method of use and acceptable results at the patient-provider level. The effectiveness of sigmoidoscopes has been examined in a few clinical studies. The Hemocult test has been through, and is going through, a number of large clinical trials to evaluate its efficacy. To date, the results are inconclusive.

The author discusses the problems that exist in trying to apply CEA to colon cancer screening programs. He also examines a number of factors that affect CEA studies in the health care area in general. One is the need for, but absence of, information from formal randomized clinical trials regarding the effect and value of screening techniques. The information that is available is usually from uncontrolled studies that are burdened with **problems of their** own. Factors such as leadtime bias, patient-self-selection bias, and length-of-study-period bias **also** present data problems that must be considered. Another problem for the analyst are the quantitative aspects of trying to measure the costs, benefits, risks, or outcomes of the different colon cancer screening programs.

The author also discusses the special considerations that colon screening programs present to a CEA. These factors include patient characteristics and differences (in terms of effectiveness of screening programs), schedule (or history) and type of testing procedures used, varying accuracy of the different procedures, different origins of the cancer that require separate analysis, order and frequency of testing, and a host of other variables that must be included in a thorough evaluation.

Once, or if, these data and methodological problems are solved, the author feels the central issue can be addressed: What is the value of screening for colon cancer? The author sets out the basic format for addressing the problems involved in a CEA approach. An illustrative example is used to examine the costs and benefits of screening for col-

on cancer. A screening program for a 50-year-old average risk woman is evaluated using eight different combinations and frequencies of screening tests. The relevant factors (costs, screening regimen, efficacy data, outcome information, etc.) are examined by the author, using a sensitivity analysis approach to determine how the different variables affect the mortality rate and cost of the various screening programs. The result of the analysis is presented as a comparison between the decreasing probabilities of colon cancer's occurring with more frequent testing, improved life expectancy changes, increases in screening costs, and decreases in lost earnings as a result of the different levels of screening programs.

Fineberg, H. V., and Pearlman, L. A., "Benefit and Cost Analysis of Medical Interventions: The Case of Cimetidine and Peptic Ulcer Disease," in *The Implications of Cost-Effectiveness Analysis of Medical Technology/Background Paper #2: Case Studies of Medical Technologies*, prepared by OTA, U.S. Congress (Washington, D. C.: in press, 1980).

The study has two major goals: One is to assess the available evidence regarding the benefits and costs of cimetidine in the treatment of peptic ulcer disease; the other is to develop a widely applicable cost-benefit model for evaluation of medical technology. The study combines these two objectives by applying the model to the evaluation of cimetidine and ulcer disease. The authors approach the analysis in three parts: 1) a development and discussion of the cost-benefit model that they feel can be applied to medical interventions in general; 2) an overview of peptic ulcer disease in the United States; and 3) a discussion of the development, diffusion, and use of cimetidine to treat and/or manage peptic ulcer disease.

The foundation of their cost-benefit model is as follows: 1) There are two principal classes of effects—clinical effects and health system effects—and the specific components of these effects depend on the population and intervention being examined; 2) an evaluative model must apply to an identifiable patient population and specific health care interventions; 3) a patient population may be defined in terms of a diagnostic category, clinical signs or symptoms, risk factors, or complications of disease; and 4) clinical and health system effects interact to lead to an outcome (health status and/or resource costs).

The authors examine a host of studies dealing with the safety, efficacy, and effectiveness of cimetidine. Among the short-term clinical effects they assess are healing, pain relief, safety and adherence to the treatment plan, complications, recurrence, and recommendations for treating newly diagnosed, uncomplicated ulcers. The long-term clinical effects they examine are recurrence, safety, and complications. They also briefly discuss the pending approval by the Food and Drug Administration of cimetidine for long-term use.

The authors also examine the health system and outcome effects of cimetidine use. Among the variables evaluated are medication, diagnostic tests, physician visits, mortality, morbidity, and resource costs. These three areas—clinical effects, health system effects, and outcomes of

cimetidine use—are the primary elements of the CBA they perform.

The authors also examine and discuss the following findings: Cimetidine promotes healing and provides faster and more complete pain relief for duodenal ulcers; it may be more effective than placebos for patients with gastric ulcers; when used for up to 2 months, cimetidine appears to be a relatively safe drug; most known side effects are minor or reversible; cimetidine plus moderate amounts of antacid costs no more than a therapeutically equivalent course of intense antacid therapy; and maintenance treatment with cimetidine for as long as a year significantly reduced the chance of ulcer recurrence (compared to a placebo) during the period of treatment. Cimetidine, according to a few studies, also appears to have contributed to a sharp decline in surgery for ulcer disease in 1978, as well as to have helped patients to lose significantly fewer days of work than patients given a placebo.

These many findings and conclusions indicate that cimetidine provides a substantial benefit to cost ratio to the peptic ulcer patient and the health care system. The authors cite the findings of two other studies; one by the Netherlands Economic Institute in 1977 and the other by Robinson Associates, Inc., in 1978. The authors conduct an in-depth review and critique of the Robinson study.

Geiser, E., and Menz, F., "The Effectiveness of Public Dental Care Programs," *Med. Care* 14:189, March 1976.

This CBA examines the costs and benefits of a public dental care program designed to "maintain the integrity of the natural teeth" in school-age children. Benefits are calculated by estimating the number of teeth "saved" in 15-year-olds that are a result of the program, and then multiplying it by the cost of replacing a natural tooth with an artificial bridge. The current costs of saving a permanent tooth are used as a cost measure. Data from two actual public dental care programs are examined. The authors conclude that public dental care programs must be administered over a relatively long period of time (6 to 7 years) before net benefits begin to accrue on an annual basis. An even longer period of time (11 to 14 years) is required before the programs generate sufficient total benefits to cover total costs. The discounted present values of the program, with use of an 8-percent discount rate, were found to be particularly sensitive to changes in the cost of care and the value of saving a tooth. Extensive sensitivity analysis is performed on the variables involved, making this article an excellent illustration of the use of sensitivity analysis in handling uncertainty.

Grosse, R., "Cost-Benefit Analysis of Health Services," *Ann. Am. Acad. Polit. Soc. Sci.* 399:89, January 1972.

This article, a general review rather than an analysis, presents an explanation of the rationale behind the use of CEA and CBA in the allocation of health resources and describes an application by HEW. Costs are described as forgone benefits: "The cost of saving a human life is not to be measured in dollars, but rather in terms of alternative lives

to be saved or other social values sacrificed." The problem of incommensurability of benefits is discussed. HEW's calculations of the cost per death averted and of productivity and medical treatment savings in various cancer control programs are presented and compared to other health programs (e.g., motor vehicles safety and arthritis). The article illustrates the changes in program priority that can occur when the criterion is changed from deaths averted to savings from avoided medical treatment and loss of productivity (measured as discounted lifetime earnings). The problem of uncertainty is discussed, and a matrix composed of relative payoffs and the certainty of results is presented as one method of handling it. The final section of the article describes in detail the HEW maternal and child health program analysis.

Hagard, S., et al., "Preventing the Birth of Infants With Down's Syndrome: A Cost-Benefit Analysis," *Br. Med. J.* 1:753, Mar. 27, 1976.

The costs and benefits of providing routine prenatal diagnosis, with termination of affected pregnancies, are examined. In the event of pregnancy termination, two situations are considered: 1) Termination is followed by a further pregnancy, assumed to be normal (replacement); and 2) termination is not followed by a further pregnancy (no replacement). Since such prenatal testing could diagnose fetal *myelocoele*, the costs and benefits involved in preventing this disease are also taken into consideration. For Down's syndrome, during the period 1975-94, the following numbers are estimated: 1) the number of births by 5-year maternal age groups, 2) survival rates and the degree of handicap of survivors, 3) costs to society of caring for survivors, 4) characteristics, including the number of affected births prevented, of a prenatal diagnostic program, and 5) the costs of such a program. The benefit of preventing the birth of infants with Down's syndrome is calculated as the cost to the community of their care. In the case of replacement, this is the difference between the cost of caring for a handicapped person and the cost of caring for a normal person. In the case of no replacement, this is the cost of caring for a handicapped person. Results of the study indicate that the benefit-cost ratio of prenatal diagnosis is greater than 1 for women over 40 years old, equal to 1 for women between 35 and 45 years, and less than 1 for women under 35 years. The problems associated with different results for different age groups are discussed. A discount rate of 10 percent is used. The authors examine the changes in the results of the analysis that would occur if, after genetic counseling, only half of the women accepted amniocentesis and possible termination of pregnancy.

Harris, G., "Introduction to Cost-Benefit Analysis Applied to New Health Technologies," prepared by Arthur D. Little, Inc., for Bureau of Health Planning and Resource Development, Health Resources Administration, Hyattsville, Md., December 1977.

This document describes the steps in CBA as the following: 1) articulation of the problem, 2) enumeration of alternatives to address the problem, 3) identification of their achievable effects, 4) measurement and valuation of the

achievable effects, and 5) application of the economic decision criteria. Objectives are described as cost reduction and/or enhancing of benefits. Costs and benefits are classified as direct, indirect, or intangible. The need to focus on incremental, rather than total, costs and benefits is explained. Discounting to present value and the problem of choosing a discount rate are discussed. Five criteria of preferredness are described: 1) net present benefit, 2) internal rate-of-return, 3) benefit-cost ratio, 4) payback period, and 5) average rate of return. The advantages, disadvantages, and appropriate use of each criterion are presented. Threshold analysis, sensitivity analysis, and probability/risk analysis are described as methods of dealing with uncertainty. Common problems encountered in analysis, such as incomplete data, transitional costs, scope, and externalities, and the issue of equity and distribution are discussed.

Klarman, H., et al., "Cost-Effectiveness Analysis Applied to the Treatment of Chronic Renal Disease, " *Med. Care* 6:48, January-February 1968.

The authors attempt to determine the best mix of center dialysis, home dialysis, and kidney transplantation in examining the costs and effects of treating chronic renal disease. A quality-of-life adjustment is made to account for the differences in lifestyle between patients on dialysis and those with effective transplants. (The freedom associated with the latter is valued at 0.25 of a life-year.) The calculations in the analysis are based on survivorship tables for transplant and dialysis cohorts of 1,000 each. The authors warn that, at the time of the analysis, there had not been enough experience with any of the three treatment modes to generate an expected life table with great accuracy. The discount rate used is net of an anticipated inflation rate, resulting in a discount rate of 4 percent for transplant and center dialysis and 5 percent for home dialysis. No sensitivity analysis is presented for the discount rate, the anticipated inflation rate, or life expectancy. The authors conclude that kidney transplantation is more cost effective than the other two alternatives. Choice of the preferred treatment modality is independent of the quality-of-life adjustment because transplantation dominates even without the adjustment.

Korenbrodt, C., Flood, A., Higgins, M., Roos, N., and Bunker, J., • "Elective Hysterectomy: Costs, Risks, and Benefits, " in *The Implications of Cost-Effectiveness Analysis of Medical Technology/Background Paper #2: Case Studies of Medical Technologies*, prepared by OTA, U.S. Congress (Washington, D. C.: in press, 1980) .

This study examines elective hysterectomy as it is used for sterilization and cancer prevention. The focus of the study is a review of the literature and the issues surrounding the costs, risks, and benefits of elective hysterectomy. The study does not attempt to establish the cost effectiveness of hysterectomy. The authors examine the significant

side effects of hysterectomy, such as change in medical utilization and psychological effects following surgery.

The authors review selected studies that evaluate the efficiency and cost effectiveness of elective hysterectomies. Not taking a cost-benefit approach, these studies do not attempt to value the saving of life in monetary terms. The first two efficiency studies that the authors' review contrast the direct costs of hysterectomy with the net lifetime costs of gynecological care. Future costs are discounted at rates varying from 3 to 6.5 percent. Another study the authors review examines the use of hysterectomy as a sterilization device v. the direct costs of tubal ligation plus the expense of future gynecological care which would have been averted by hysterectomy.

The effectiveness of hysterectomies in preventing pregnancy and cancer is not an issue; but the health risks of the procedure are. Efficacy/effectiveness of alternative means to accomplish these objectives are assessed, but not in the cost-effectiveness studies reviewed. Additionally, the cost-effectiveness studies which are reviewed do not attempt to identify, measure, or place a value on the side effects of surgery.

Costs are distinguished from charges and issues of equity are discussed. The authors do not employ a sensitivity analysis. Conclusions are drawn with respect to the cost effectiveness of elective hysterectomies as they are used for the separate purposes that are examined.

Leroy, L., and Solkowitz, S., "Costs and Effectiveness of Nurse Practitioners, " in *The Implications of Cost-Effectiveness Analysis of Medical Technology/Background Paper #2: Case Studies of Medical Technologies*, prepared by OTA, U.S. Congress (Washington, D. C.: in press, 1980) .

This study reviews the literature on the cost effectiveness of nurse practitioners to provide primary medical care services. Only limited data are available, and much of the information deals with other types of physician extenders. In addition, many of the data have been gathered in the developmental stage of introducing nurse practitioners; the relevance of these data for present policy purposes is unclear.

At least theoretically, nurse practitioners offer the potential to reduce the costs of health care and improve access to the health care system. Nurse practitioners can perform basic and routine medical care tasks, allowing physicians to focus their efforts on serious illness problems. Training costs and pay are less for nurse practitioners than for physicians, so costs should be lower for routine care if nurse practitioners are used. There are a number of problems in directly extrapolating to lower costs, however, and, depending on the system within which nurse practitioners operate, cost savings may or may not be realized.

A key question examined by this study deals with the nature of the services nurse practitioners perform and how they affect costs. In general, they provide complementary and substitute services, although the nature of these services is difficult to document because data often indicate only "office visit." Complementary services would include

● With the technical assistance of Mitchell LaPlante

treatment such as "well baby care," while substitutive services refers to such treatment as "physicals."

In terms of quality of care, nurse practitioners appear to provide care that is of as high quality as that of physicians (with whom they usually work and are compared). There is some evidence that nurse practitioners, working in close conjunction with physicians, provide superior care when compared to solo practitioners. Productivity is more difficult to assess and depends on how nurse practitioners are used. There seems to be clear evidence that the use of nurse practitioners improves physicians' productivity, but it is not clear how this improved productivity affects costs. Supervisory time, duplicative work, and the fact that nurse practitioners spend more time per patient must be considered.

The data needed to conduct a CEA of nurse practitioners include employment costs, training costs, and medical care costs. Unfortunately, each of these factors may be subject to changes as a result of alterations in another part of the system. The employment costs of nurse practitioners, for example, is a function (in part) of the demand for their services. Even more difficult to determine is price. Because they are most often hired by physicians or health institutions which have already established fee systems, any cost savings may be absorbed by the physicians or institutions and may not be reflected in the price of services delivered.

The case concludes by cautioning against the use of current data to determine new policy. Based on changes in the way nurse practitioners are used, costs could vary widely. This is a case in which an actual CEA may provide misleading policy advice, although the identification of variables required by the CEA may be very helpful.

Lesourd, D., et al., *Benefit-Cost Analysis of Kidney Disease Programs*, PHS publication No. 1941, prepared by the Research Triangle Institute (Washington, D. C.: U.S. Government Printing Office, 1968).

This analysis was one of a series of federally sponsored efforts to assess the costs and benefits of alternative approaches to the problem of kidney disease. The approaches included screening, prevention, and three treatment modalities (home dialysis, center dialysis, and transplantation). Employing a variety of assumptions (e. g., risk population for the screening programs, size of treatment facilities), the authors concluded that early detection dramatically dominated the treatment approaches with respect to economic benefits and costs. Depending on the population screened, the former had benefit-cost ratios of 30:1 and greater. By contrast, the treatment alternatives produced benefit-cost ratios in the vicinity of 1:1. This ratio varied according to: 1) the treatment method (transplantation producing the highest ratios); 2) the scale of operation; 3) the allocation of research costs; and 4) high, low, and best cost estimates in the two instances of dialysis. To estimate indirect benefits (i. e., productivity losses avoided), the authors assumed that 70% percent of the dialysis patients would be capable of resuming a normal earning capacity; the remaining 30 percent were assigned half the expected income of a comparable but healthy individual.

The qualitative findings of this analysis were supported by other studies undertaken at the same time. Despite the consensus that prevention and early detection were the most cost-effective approaches to dealing with the kidney disease problem, Federal policy was directed toward the alternative which appeared least economically desirable, center dialysis.

Lubeck, D. P., and Bunker, J. P., "The Artificial Heart: Costs, Risks, and Benefits," in *The Implications of Cost-Effectiveness Analysis of Medical Technology/Background Paper #2: Case Studies of Medical Technologies*, prepared by OTA, U.S. Congress (Washington, D. C.: in press, 1980).

This study examines the many factors that have played a role in the development of the artificial heart: factors that are affected by, and in turn affect, three areas of public policy—R&D, reimbursement, and regulation.

The authors provide a backdrop of the history of the artificial heart development program. They also examine the safety and efficacy determinations that have been arrived at through experiments and clinical trials. The current and potential technological developments that are or will be, part of the artificial heart are described, and the numerous R&D needs that must be met before an artificial heart can be successfully used are examined.

The authors examine the economic aspects of the artificial heart from the patient's perspective and from a societal view, focusing on the costs of diagnosis, implantation, and postoperative care. These costs are compared to the costs associated with related procedures: cardiac pacemakers, aortocoronary bypass surgery, and heart transplants. The renal dialysis program is used to illustrate the possibility of the Federal Government's financing artificial heart procedures and the distribution of services to the population.

The authors also examine four social cost areas: increased social expenditures, potential distributional inequities, effects of nuclear radiation if a nuclear energy source is used, and the opportunity costs. They also examine the efficacy, potential benefits and costs, and likelihood of saving lives by cardiac disease prevention programs.

Quality of life issues are addressed for both the short- and long-term effects. The authors draw on the experiences of those who have had heart and kidney transplants to illustrate the types of impacts on the patient and the family that can occur. The potential effects include personal, marital, family, physical, medical, and psychological problems that can occur after a person undergoes major surgery. The authors also discuss the added burdens/impacts that will result if a nuclear-powered energy source is used.

On the benefits side, although the authors briefly discuss the technological spinoffs of the artificial heart program, their primary focus is on two areas: the potential for patients returning to an active life, and the estimated years of life that may be gained. Morbidity, mortality, and added years of life are examined and estimated via a best case and worst case analysis if the artificial heart is implanted.

Luce, B., "Allocating Costs and Benefits in Disease Prevention Programs: An Application to Cervical Cancer Screening," in *The Implications of Cost-Effectiveness Analysis of Medical Technology/Background Paper #2: Case Studies of Medical Technologies*, prepared by OTA U.S. Congress (Washington, D. C.: in press, 1980).

This author believes that one important reason for the reluctance to fund disease prevention/health promotion programs is that benefits often do not accrue sufficiently to those who incur the costs. Also, the private sector is expected to fund such programs. Consequently, traditional CEAs which are performed only from the societal perspective may not be applicable for public policy.

This author performs a CEA of cervical cancer screening for a given risk group from different perspectives. Screening for cervical cancer is used to demonstrate the cost effectiveness of disease prevention programs. The disease process is modeled by the author using a Markov Chain technique to "age" a simulated population of 30- to 39-year-old women for 10 years (using disease transition probabilities reported in the literature). The cost effectiveness of screening is then calculated at different intervals—ranging from annual screening to no screening for the 10-year period. The effects are evaluated for: 1) different migration patterns, 2) different risk groups, 3) different modes of administering Pap tests, and 4) joint production considerations. The author also tests the sensitivity of the results to various discount rates and to the range of error rates for Pap tests.

The results indicate that a private party always has a financial incentive to postpone screening, whereas society finds it more cost effective to screen, but only at infrequent intervals. In addition, the author notes, the cost effectiveness of screening is markedly affected when a more efficient (i.e., less costly) delivery mode is simulated. Screening is significantly affected when joint production effects are considered. The cost effectiveness of screening, however, is not very sensitive to small changes in the discount rate, initially set at 10 percent, nor to varying assumptions regarding error rates.

The author concludes that if society wants the private sector to screen for cervical cancer at a socially determined optimal rate, then society must be willing to subsidize the cost of the program. The study also concludes that the cost effectiveness of cervical cancer screening is much more affected by the cost assigned to screening than by different assumptions of the precise error and discount rates.

The cost effectiveness of screening at each simulated interval was compared to no screening for a 10-year period. Efficacy information was addressed and different test error rates were used. The production of the Pap test was simulated, for cost purposes, at two levels: an expensive university hospital clinic using specialists, and an inexpensive health clinic using licensed nurses. Only lives and years of life saved were identified as benefits.

Costs were distinguished from charges, marginal costs were considered, and indirect costs are used. Discounting of costs and benefits was done (rates tested: 0, 5, 8 to 12 percent), and sensitivity analysis was performed; however,

issues of equity were not directly considered in the analysis.

Luft, H., "Benefit-Cost Analysis and Public Policy Implementation: From Normative to Positive Analysis," *Pub. Pol.* 24:437, Fall 1976.

The author argues that conventional CBA and CEA should be extended to include a predictive analysis of the implementation phase in order to determine whether and how the project will be done. The predictive analysis involves three steps: 1) a standard CBA to determine whether the project should be undertaken; 2) a CBA from the perspective of each decisionmaker or interest group capable of influencing the success of the project to determine the likelihood that the project will be undertaken; 3) a redesign of the project or the development of incentives to improve the likelihood of success for socially desirable projects.

In a case study of a surgicenter, it is noted that the resultant shifting of a revenue from one set of providers to another, though only a pecuniary externality in standard benefit-cost analysis, has a substantial impact on the likelihood that a surgicenter will actually be implemented. The importance of identifying decisionmakers and their respective power to influence the success of the project is discussed. The author points out that in the second step of the analysis (the "interest-group analysis"), transfer payments, taxes, and pecuniary externalities should be explicitly considered, so that the financial flows as perceived by the relevant interest groups are adequately represented. In addition, it may be appropriate to use substantially different discount rates for each interest group. The final step in the interest-group analysis is to estimate each group's utility function and the group's relative power to either promote or block implementation of the project.

Luft presents an application of predictive analysis to the use of a work evaluation unit for ascertaining functional work capacity following a myocardial infarction. The relevant interest groups in this analysis include the patient, family, physician, employer, insurer, and society. Luft estimates both the likely net effects on each interest group of using the work evaluation unit and each group's relative weight. The author concludes that this extended, positive form of benefit-cost analysis can improve the allocation of resources by helping to promote the implementation of desirable and feasible programs and "to prevent the adoption and implementation of proposals that appear promising in theory but are likely to be sabotaged in practice."

McNeil, B., et al., "Measures of Clinical Efficacy: Cost-Effectiveness Calculations in the Diagnosis and Treatment of Hypertensive Renovascular Disease," *N. Eng. J. Med.* 293:216, July 31, 1975.

The authors measure the value, in terms of sensitivity and specificity, of intravenous pyelography and radiohippuran renography as diagnostic screening methods for hypertension caused by renovascular disease. Costs associated with both diagnosis and subsequent surgical treatment are also calculated. Financial costs of the diagnostic procedures are based on the Massachusetts Relative Value Scale; hospital and operation costs are based on 1974

charges at Peter Bent Brigham Hospital. Three aspects of cost-effectiveness in the management of renovascular hypertension are examined: 1) the financial costs of case-finding in relation to the sensitivity and specificity of both diagnostic procedures; 2) the total dollar cost of screening the American hypertensive population, of making a definitive diagnosis, and of performing corrective operations; and 3) the life and dollar cost of each surgical cure. The cost of case-finding is found to be approximately \$2,000 per positive diagnosis when only one diagnostic examination is used for screening. This figure rises to \$2,600 to \$4,400 when both procedures are employed. The total costs of screening all patients with hypertension, performing arteriography on those with positive tests, and operating on all patients with renovascular disease amounts to \$10 billion to \$13 billion. The authors note that this estimate does not include the costs of initial identification of all hypertensive Americans. Thus, the authors estimate a cost of \$15,000 to \$20,000 per cure, and note that there are **15 deaths** for every 100 surgical cures. The cost-effectiveness calculations are not sensitive to varying the assumptions regarding the prevalence of renovascular disease in hypertension patients from 10 to 5 percent.

Neuhauser, D., "Elective Inguinal Herniorrhaphy Versus Truss in the Elderly," in *Cost, Risks, and Benefits of Surgery*, edited by J. P. Bunker, et al. (New York, N. Y.: Oxford University Press, 1977).

This CEA reviews available data in order to see what effect the choice of elective herniorrhaphy v. truss has on the life expectancy of a 65-year-old person. The analysis includes a calculation of the average effects of 1) having an immediate elective herniorrhaphy (with its low mortality, but the risk that the hernia will recur and require additional elective operations); and 2) using a truss (with its attendant risk of obstruction, followed by an emergency operation with a high mortality rate). Using data obtained from the relevant literature, the author estimates: 1) the mortality rates associated with (a) elective and (b) emergency surgery, 2) the probability of recurrence of the hernia after an operation, 3) the yearly probability of strangulation, and 4) the life expectancy of the patient. Two sets of numbers are used in the analysis. The first set of numbers serves as a conservative test of the hypothesis that the truss prolongs life, because the values in this set are those which systematically place the benefit of the doubt in a direction favorable to the elective operation. The numbers in the second set are based on what seem to be the most reasonable and reliable data. (The author notes that there are insufficient data to consider a "do nothing" third alternative.)

The model takes the form of a decision tree, which is designed so that the "payoffs" equal the expected value of the average number of life-years lost. The results of the conservative test (used because it makes the strongest **case** for the elective operation, which is standard of surgical practice uniformly proposed by current surgical literature) indicate that the elective operation has a higher loss of life associated with it for the 65-year-old than the truss does. The test using the "most reasonable" estimates indicates that the elective operation has a mortality rate 5.5 times

greater than the truss. This large relative difference, however, translates into an absolute difference of only 14.29 days. The author notes that in view of this small absolute difference in mortality, the issue of quality of life becomes important. The article continues with a discussion of this type of adjustment, but no quality-of-life adjustments on the analysis data are attempted. The magnitude of the costs involved in the elective operation is noted, but a detailed analysis is not presented. On the basis of this study, the author observes that medicare funds expended on elective herniorrhaphy serve, if anything, not to increase life expectancy, but rather to improve the quality of life. He asks, therefore, if these funds might better serve to improve the quality of life for the elderly in some other way (such as in reducing subway fares for the elderly).

Neuhauser, D., and Lewicki, A., "What Do We Gain From the Sixth Stool Guaiac?" *N. Eng. J. Med.* 293: 226, July 31, 1975.

This article examines the costs and effects of the sixth sequential stool guaiac for screening asymptomatic colonic cancer. An analysis of the expenditures concludes that costs rise exponentially, so the marginal cost of the sixth test may be 20,000 times the average cost. In addition, data indicate that there is little gain in the true positive rate from testing beyond the second guaiac examination. Thus, the cost per true positive becomes gigantic. The marginal cost is decreased with lower test sensitivity and increased with lower prevalence of colonic cancer. The authors conclude that defining a high-risk group, which would serve to lower marginal cost, is essential to justify such screening programs in a world of constrained resources.

Neutra, R., "Indications for the Surgical Treatment of Suspected Acute Appendicitis: A Cost-Effectiveness Approach," in *Costs, Risks, and Benefits of Surgery*, edited by J. P. Bunker, et al. (New York, N. Y.: Oxford University Press, 1977).

This article presents a quantitative approach to the costs and benefits associated with the "interventionist" and "noninterventionist" management of suspected appendicitis. The assessment considers lives, postoperative disability, and economic costs. Since the author relied on the rather scanty data from the available literature and on many simplifying assumptions, however, he cautions that the analysis should be viewed as "paradigmatic rather than definitive." The analysis addresses the question of when to operate, not alternative strategies, such as a dietary prevention program or antibiotic therapy.

On the basis of two symptoms (location and severity of pain) and two signs (presence of right lower quadrant rebound tenderness and rectal tenderness), an "appendicitis risk score" was developed. Twenty-four symptom combinations were developed and the probability of appendicitis for each combination was determined and ranked.

For example, the highest rank (24) corresponds to the combination of right lower quadrant, severe pain with rebound, and rectal tenderness. Assumptions are presented regarding: 1) the distribution of cases and noncases of appendicitis along the risk scale, 2) the prevalence of cases

and noncases, and 3) the net costs of the false negatives and false positives in terms of mortality, convalescence, and direct hospital costs. Two analyses are performed, one assuming that 100 percent of the appendicitis patients on whom surgery is not performed will perforate, the other assuming that 30 percent will perforate. The results indicate that a surgeon can ensure an acceptable mortality rate by taking an "interventionist" approach, but only at the cost of increasing convalescent days and hospital costs. Relaxing the indications for surgery to include patients who lack the most obvious symptoms saves lives, but at an ever diminishing rate. The few lives saved by operating on patients with minimal symptoms are purchased at great costs in convalescence and dollars associated with the removal of large number of normal appendices. The author suggests a solution to this dilemma—namely, increasing discrimination by using very complete diagnostic information and careful clinical interpretations. Increased discrimination can reduce the removal rate of normal appendices without an increase (and possibly with a decrease) in the rate of perforation. The author estimates the possible savings in lives, convalescence, and money that may result from an increase in discrimination.

Office of Technology Assessment, U.S. Congress, A Review of Selected Federal Vaccine and Immunization Policies, GPO stock No. 052-003 -00701-1 (Washington, D. C.: U.S. Government Printing Office, September 1979).

This study includes an examination of the cost effectiveness (CE) of applying a primary preventive technology—vaccination against pneumococcal pneumonia—to different age groups. Medical care costs and health effects associated with a preventive program are explored from the perspectives of society and of a third-party payer such as Medicare.

A CEA was used to calculate the expected change in health effects and medical costs from vaccination against pneumococcal pneumonia—an alternative compared to continuing the present situation in which pneumococcal pneumonia is treated if it occurs. In the analysis, costs were limited to expenditures and savings within the medical care sector, and changes in health status were expressed in years of healthy life. Thus, the cost-effectiveness ratio represented the net medical cost per year of healthy life that would be gained by a vaccinated person. The calculations were based on a single hypothetical vaccination program conducted in June 1978. The analysis used a simulation model to estimate the costs and effects that would result from 1978 to 2050 for two closed populations, one vaccinated and the other unvaccinated. Costs and effects were discounted at 5 percent per year. Separate cost-effectiveness ratios were calculated for five different ages: 2 to 4 years, 5 to 24 years, 25 to 44 years, 45 to 64 years, and 65 years and older. The analysis employed a sensitivity analysis to test the effect on the results of varying the values of several uncertain parameters over reasonable ranges.

Net health effects were expressed in quality-adjusted life years (QALYs). Mortality rates for pneumonia as an un-

derlying cause of death provided by the National Center for Health Statistics (NCHS) formed the basis for estimating 1978 pneumonia mortality among the unvaccinated. Unpublished age-specific data from the Health Interview Survey conducted by NCHS was used to estimate the days of pneumonia morbidity among the unvaccinated.

Medical care costs, expressed in dollars, included additional expenditures for vaccinations and for treatment of vaccine side effects; reduced expenditures for treating pneumococcal pneumonia that would be expected to occur without vaccination; and additional expenditures for other illness in the extended years of life gained by vaccinees who avoid death from pneumococcal pneumonia. Unpublished age-specific data from the Hospital Discharge Survey and the National Ambulatory Care Survey of NCHS was used to construct estimates of the costs of treating pneumonia.

The study found that, given the range of factors involved, vaccinations would entail positive medical expenditures for every age group and would be most cost effective for those 65 years or older. The cost-effectiveness ratio was about \$4,800 per QALY gained for all ages and \$1,000 per QALY for ages 65 and older. The analysis found that vaccination of 21.5 percent of the population 65 years and older would result in a net cost to society of about \$23 million and would yield about 22,000 QALYs over the lifetimes of those vaccinated. The study also concluded that vaccination for all age groups in the population would have a net cost of about \$150 million for a gain of 31,000 QALYs.

The study also examines policy implications of these findings, including a possible change in the Medicare law to permit Federal payment for pneumococcal vaccine for the elderly.

Pauker, S., and Kassirer, J., "Therapeutic Decision-Making: A Cost-Benefit Analysis," N. Eng. J. Med. 293:229, July 31, 1975.

In this article, a mathematical relationship is derived between the benefits and costs of a treatment in a given disease and the threshold level of clinical suspicion of the disease. When the probability of a patient's illness exceeds this threshold level, the better choice is to administer treatment; when the probability is below the threshold, the better choice is to withhold treatment. The benefit equals the net benefit of appropriate treatment and is calculated as the difference between the utility of administering treatment and the utility of withholding treatment from patients who could benefit from it. The cost is the net cost of unnecessary therapy and is calculated as the difference between the utility of avoiding treatment and the utility of administering treatment to those who do not have the disease. Using probabilities, the authors develop equations expressing the expected values of treatment and no treatment. The point of indifference as to course of action is where the expected value of treatment equals the expected value of no treatment. The probability value at the indifference point is the threshold level. Using this concept in a clinical setting requires assessing the probability of the disease in a given patient and determining whether it is above or below the threshold level. A unique threshold value must be calcu-

lated for each disease and its treatment in a given cohort of patients (defined as having common risk characteristics). Sensitivity analysis may be employed when significant uncertainty surrounds the probabilities and utilities involved in the calculations. In addition, if the clinical status of the patient or if the circumstances of administration of the therapy differ notably from the typical case, the benefits and/or costs must be adjusted appropriately.

Rettig, R. A., "Formal Analysis, Policy Formulation, and End-Stage Renal Disease," in *The Implications of Cost-Effectiveness Analysis of Medical Technology/Background Paper #2: Case Studies of Medical Technologies*, prepared by OTA, U.S. Congress (Washington, D. C.: in press, 1980).

This paper examines the role of formal analysis in Federal decisionmaking related to end-stage renal disease rather than that of CEA per se. The study places special emphasis on institutional factors encouraging or inhibiting the use of formal analyses. These are defined as "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." CEA and CBA fit this definition, as do risk-benefit and cost analyses.

The case study presents information on patients with end-stage renal disease. The author notes that the proportion of men in the total patient population on dialysis declined between 1970 and 1976. The average age of dialysis patients increased, and the proportion of home dialysis patients declined from 40 percent in 1972 to 24 percent in 1976. The number of dialysis patients in the Medicare program has risen from 14,000 in 1973 to 50,000 in 1978.

The paper deals primarily with the impact of two formal analyses of end-stage renal disease issued in 1967: 1) the "Gottschalk report," prepared by an expert advisory committee for the Bureau of the Budget; and 2) the "Burton report," prepared by a Public Health Service task force for the U.S. Surgeon General. (The paper mentions several other formal analyses but focuses on these two.) The author describes policy-related and institutional/bureaucratic factors that led to the conduct of these formal analyses and that affected the form the analyses took along with many of their methodological assumptions. The author also describes and summarizes the results of the CEA in the Gottschalk report and of the "costs and benefits" analysis in the Burton report.

The author then addresses the effects of both reports. The Gottschalk report, for example, led the Bureau of the Budget to fund a Veterans Administration (VA) administered hemodialysis program that included a substantial portion of the VA dialysis patients. The Burton report, according to the author, had no direct program effects.

On the whole, this study suggests that formal analysis "did not affect the fact that the policy choice was a basic political choice." Yet the paper also notes that the analyses may have raised the consciousness of high level policymakers as to cost implications. The paper also mentions some of the factors that limit the effect of analysis such as inadequate data, lack of access of analysts to decisionmak-

ers, and difficulties in making assumptions that frame the problem.

Saxe, L., *The Implications of Cost-Effectiveness Analysis of Medical Technology/Background Paper #3: The Efficacy and Cost-Effectiveness of Psychotherapy*, prepared by OTA, U.S. Congress (Washington, D. C.: in press, 1980).

This case study prepared by Leonard Saxe, was based on a document prepared for OTA by Brian Yates and Frederick Newman. It describes a variety of methodological and substantive problems that arise in assessing the effects of mental health treatments. The report both summarizes the existing literature and attempts to present the divergent perspectives within the research-policy community concerned with psychotherapy. As described below, it deals with four issues that are centrally related to the evaluation of psychotherapy.

Definition. Psychotherapy is not a simple intervention, and part of the confusion about its effectiveness has to do with reviewers' use of different definitions. The present report uses a relatively broad definition of psychotherapy in order to best represent current therapy practice. This definition includes treatments based on Freudian ideas about psychodynamics, as well as newer therapies based on theories of learning and cognition. The report also notes that psychotherapies are not distinguishable only by their theoretical bases. In addition, patient variables (e.g., intelligence), therapist variables (e.g., empathy), and the nature of the treatment setting affect the nature of psychotherapy. Although the inclusion of such factors makes the analysis of psychotherapy more difficult, there seems to be ample evidence as to the importance of these factors on the outcome.

Assessability. Although psychotherapy itself is complex and there is no clearly agreed upon way of viewing it, the methods for assessing psychotherapy seem better established. The report describes the variety of experimental and quasi-experimental designs that have been used in assessing psychotherapy, along with an analysis of what types of information can be obtained by application of these techniques. The report also describes and analyzes various methodological strategies for measuring the outcomes of psychotherapeutic treatment and the ways in which the reliability and validity of measures are established. Unfortunately, research practice does not always meet these standards. Some explanations offered in the report include the difficulties of withholding treatment and the problems of assessing effects over time. The report also considers the recent development of systematic procedures for synthesizing the findings of multiple investigations. The problems of such techniques, as well as their promise for detecting valid trends in the research literature, are analyzed.

Efficacy. The report describes some of the plethora of research which has been conducted on psychotherapy. The focus of the report's efficacy analysis is a discussion of six important earlier reviews of the psychotherapy literature. In addition, many of the evaluative studies themselves were reviewed. Despite some fundamental differences,

both in the criteria they develop for assessing psychotherapy and the studies they include for review, the reviews all seem to support the findings that (under specified conditions) there is evidence as to psychotherapy's effectiveness. In fact, with the exception of reviews that focus on psychoanalytically oriented therapies, there seems to be little negative evidence as to efficacy of such treatments. Although it is difficult to make global statements, the evidence seems more supportive of psychotherapy than of any alternative hypothesis (spontaneous remission, placebo effects). However, there is a great need for well-conducted research which evaluates psychotherapy for specific disorders under specified treatment conditions. This research would need to be carried out in actual delivery settings.

Cost effectiveness. The application of CEA/CBA to psychotherapy is much more recent, and hence less developed than efficacy research. Nevertheless, a number of models are available for conducting such analyses. In general, the models are based on those used in other applications of CEA/CBA, and the problems engendered by their use are similar. A particular concern with such psychotherapy assessments is whether costs and benefits can be comprehensively measured. Thus, for example, although the costs of psychotherapy treatment are relatively easy to measure, it is more difficult to determine and quantify what type of benefit has been achieved. Much of the CEA/CBA research to date has involved a comparison of psychotherapy treatments. Although such research indicates the potential use of CEA/CBA to improve the functioning of clinical settings where psychotherapy is given, its use for policymaking is less clear. Such work seems possible, however, and may potentially be incorporated as part of large-scale efficacy assessments.

Schachter, K., and Neuhauser, D., "Surgery for Breast Cancer," in *The Implications of Cost-Effectiveness Analysis of Medical Technology\Background Paper #2: Case Studies of Medical Technologies*, prepared by OTA, U.S. Congress (Washington, D. C.: in press, 1980).

This study is an examination of the scientific and technical issues that are part of the debate over the appropriate approach to detecting and treating breast cancer. The major focus of the analysis is devoted to the review, discussion, and evaluation of the various types of surgical and nonsurgical procedures used to treat breast cancer. Cost-effectiveness considerations, however, are not totally ignored. The authors note that the resolution of the detection and treatment issues will have major cost and benefit implications. The authors also perform a hospital cost analysis of two different treatment strategies—inpatient versus outpatient tissue biopsy.

The background of the study is established by a brief overview of the extent and effects of breast cancer in America. A history of cancer of the breast is presented, as is a description of the development and popularization of the Halsted method of performing radical mastectomy procedures to treat breast cancer. Developed in the late 1880's, the Halsted method remained the generally accepted "treatment of choice" for over 80 years—in 1970, 80 percent of

breast cancer patients in the United States received radical mastectomies.

Variations of the Halsted method and completely new approaches to treating breast cancer (both surgically and nonsurgically or a combination of both techniques) over the last two decades have challenged the traditional Halsted technique. In this paper, the authors examine the evidence regarding the efficacy, safety, mortality, and morbidity of these new techniques, as well as that for the Halsted method.

The six treatment procedures they examine are: 1) radical mastectomy, 2) extended radical mastectomy, 3) modified radical mastectomy, 4) simple or total mastectomy, 5) partial mastectomy, and 6) local excision, lumpectomy (or tylectomy). Special emphasis is placed on reviewing the status of the nontraditional methods of treating breast cancer, i.e., those procedures that run contrary to the Halsted approach (radical mastectomy). Also discussed are the roles of three American surgeons—Dr. Leslie Wise, Dr. George Crile, Jr., and Dr. Oliver Cope—who have long advocated and practiced a more limited surgical approach to treating breast cancer. Their investigations and results regarding the success of using non-Halsted procedures to treat patients are examined.

The authors summarize the debate by discussing the results of the National Cancer Institute's consensus panel meeting on the topic of breast cancer treatment held June 5, 1979, at the National Institutes of Health. In essence, the conclusion was that much work is left to be done in evaluating the various techniques. The conference recognized the potential of the nontraditional procedures and the value of the total mastectomy as used in place of the Halsted radical procedure for certain women. More information is needed regarding the efficacy and safety of the alternative procedures; segmental mastectomy, primary radiotherapy, etc. Over the last few years, the modified radical procedure has become more popular than the Halsted radical technique, but there is still no general consensus on what procedure(s) should be the treatment of choice.

According to the authors, there is good evidence that survival rates are no better for the radical procedures than for the less severe techniques available. Why then is there still adherence to the more drastic approach? The authors set out a number of micro and macro issues that may help explain the continued reliance on the Halsted method: cultural and traditional reasons, economic incentives, individual personalities and reputations, existing logic of cancer treatment, structure of the medical specialties, burden of proof requirements on innovators and traditionalists, medical conservatism, and the scaling of evidence.

The authors' cost analysis, as mentioned above, is a comparison of the cost differences of inpatient versus outpatient tissue biopsy. The authors consider these alternative strategies in light of the number of cases of breast surgery at Massachusetts General Hospital in 1976 and the total number of procedures for the United States in 1975. Their calculations and extrapolations determined that \$185 million (excluding radiation therapy) or a 45-percent reduction in total costs would result per year if outpatient biopsies were used uniformly and radical surgery were re-

placed with more limited surgery. However, as the authors note, the reader must realize the very approximate nature of cost analysis. Nevertheless, the authors feel that the magnitude of the cost differences warrant a more complete investigation.

Scheffler, R. M., and Delaney, M., "Assessing Selected Respiratory Therapy Modalities: Trends and Relative Costs in the Washington, D.C. Area," in *The Implications of Cost-Effectiveness Analysis of Medical Technology/Background Paper #2: Case Studies of Medical Technologies*, prepared by OTA, U.S. Congress (Washington, D. C.: in press, 1980).

This study is basically a cost analysis of alternative methods to deliver respiratory therapy. The authors describe the technology of respiratory therapy, the indications for the use of each type of therapy, and the substitutability of different modalities. The authors also review the literature on effectiveness and conclude that respiratory therapy's efficacy and effectiveness has not been adequately proven and is still in dispute.

The paper describes an empirical survey which the authors undertook in the metropolitan Washington, D. C., area. Using data from that survey, the authors chart the utilization of respiratory therapy techniques by type of hospital and by number of beds. They also chart the trends in use from 1976 to 1979, noting a shift from the more expensive high-technology oriented therapy (IPPB) to the less expensive simpler aerosols and spirometers.

In their cost analysis, the authors compare each type of therapy with another. Cost savings of the shift in technology are estimated. By focusing on a cost comparison analysis, the authors implicitly assumed that efficacy and effectiveness across therapies are constant. The costs of one therapy are compared with those of the others.

The adequacy of efficacy and effectiveness information is addressed (and found to be inadequate). Specific benefits and effectiveness are not identified, measured, or valued. Costs are distinguished from charges, and "avoidable," or incremental costs are identified. The indirect costs (lost production) are not identified. Discounting is not used (costs are incurred in the present, future benefits are not projected). Sensitivity analysis is not used, and issues of equity are not addressed. Public policy considerations are discussed.

Schoenbaum, S., et al., "Benefit-Cost Analysis of Rubella Vaccination Policy," *N. Eng. J. Med.* 294: 306, Feb. 5, 1976.

The authors estimate the costs and benefits of various rubella vaccination strategies, each at 100- and 80-percent compliance. Benefits are the savings that result from the prevention of both acute rubella and congenital rubella. The direct costs of rubella (and hence the direct cost savings from prevention) are the costs of medical care, medication or special devices, and special education or rehabilitation. Indirect costs result from temporary disability during acute illness and complications, in addition to deaths from purpura or encephalitis, and from permanent disability that results from congenital rubella syndrome. The

costs of rubella vaccination were estimated on the basis of the cost of measles vaccination. Vaccination at ages 10 to 12 appears preferable to vaccination at ages 1 to 3 for two reasons: 1) because the gap between vaccinating and realizing benefits from prevention of congenital rubella is shorter the closer vaccination is to childbearing; and 2) because the net benefits of preventing congenital rubella are greater than those associated with preventing acute rubella infection. The latter reason was demonstrated by employing conservative assumptions: Only the most obvious abnormalities associated with congenital rubella were included in the analysis, and the number of clinical cases of acute rubella was probably overestimated.

The results indicate that the economic benefits of a rubella vaccination program, assuming 100-percent compliance, are greater if offered once to females at age 12 rather than to children of both sexes at age 6 or younger. If compliance is 80-percent instead, the least number of babies with congenital rubella will be born when vaccination is offered twice, once to children of both sexes at the age of 2 and again to females at the age of 12. Finally, the analysis indicates that if the vaccine is to be offered to children at or before age 2, it is more effective to use combined measles and rubella vaccine.

A 6-percent discount rate is used throughout the analysis, with no sensitivity testing done. It is assumed that complications of rubella vaccination in the age groups under consideration are negligible. The frequency of rubella infection was estimated on the basis of two serologic surveys.

Schoenbaum, S., et al., "The Swine-Influenza Decision," *N. Eng. J. Med.* 295: 759, Sept. 30, 1976.

This CBA examines alternative strategies for a swine influenza vaccination program. The benefits of a vaccination program are described as the product of the direct and indirect costs that would be incurred in the event of an epidemic, the probability of an epidemic, and vaccine efficacy. The costs involved in the program include those associated with vaccine production and administration, resultant complications, and intangibles. Both private and public sector programs are examined. The Delphi method is used to obtain information regarding the probability of an epidemic, age-specific morbidity and mortality rates for both total and high-risk populations, vaccine efficacy and side effects, and vaccine acceptance rates. The net benefits for three strategies, which vary by age and risk of the target population, are calculated. The probability of an epidemic, vaccine efficacy, and vaccine acceptance rates are subjected to sensitivity analysis. The three strategies under consideration were found to be sensitive to acceptance rates. The results of the analysis indicate that expected net benefits are not maximized by the vaccination of everyone over 5 years of age. A policy of orienting the program toward the general adult population can be justified with low vaccine-administration costs, high vaccine efficacy, and high acceptance rates (59 percent), assuming further that the flu strain represents a potential pandemic. Otherwise, only high-risk group vaccination is warranted.

A major feature of this study—both in its design and achievement—is demonstration that a sound, useful analysis can be initiated and completed in a matter of weeks.

Schweitzer, S. O., and Scalzi, C., “The Cost-Effectiveness of Bone Marrow Transplant Therapy and Its Policy Implications,” in *The Implications of Cost-Effectiveness Analysis of Medical Technology/Background Paper #2: Case Studies of Medical Technologies*, prepared by OTA, U.S. Congress (Washington, D. C.: in press, 1980).

The study is a CEA of a highly technical and very costly emerging medical technology. The cost and effectiveness (lives and years of life saved) data the authors use were empirically derived from the Bone Marrow Transplant (BMT) Program at the University of California at Los Angeles. Much of the effectiveness data had been previously published. Quality of life data was collected by a single observer, a BMT Program nurse.

Patients with aplastic anemia and leukemia were studied. Since there were insufficient resources to allow all eligible patients into the BMT Program, patients who received transplants were compared to those who were judged eligible but not selected. The sample sizes were very small and survival data was limited to 3 years as a result of the newness of the technology.

Bone marrow transplant procedures are compared to conventional therapy, as opposed to no treatment, even though there is no indication that conventional treatment is efficacious. The cost of transplant procedures is considered to be the incremental—or avoidable—cost above that what would have been spent anyway.

Efficacy data is empirically derived from the study of patients admitted to the program, extrapolated to normal life expectancy for “successful” transplants (defined as those patients still living after 3 years), and compared to the group of nonselected patients. The production process described and used in the analysis is the one currently in place (this is an emerging technology).

A wide range of benefits is identified, and an attempt is made by the authors to value and combine quality of life with projected increase in life. Hospital charges are used for costs, and incremental costs are identified and included in the analysis. Indirect costs are also calculated. Discounting is not used for future benefit (years of life saved) valuations. All costs were assumed to occur in the present. Sensitivity analysis is not used. Bone marrow transplantation is still being employed in a research mode, so equity issues are mainly relevant to the patient selection process; such issues are not directly addressed in this study.

The results of the analysis are expressed as a cost-effectiveness ratio (cost per year of life saved). The authors do not qualify these results by discussing the confidence which the reader can place in them. An extensive discussion on the relevance this study has to public policy is presented. The cost-effectiveness ratios developed for bone marrow transplant procedures are compared to the cost-effectiveness ratios for other life-saving programs.

Showstack, J. A., and Schroeder, S. A., “The Costs and Effectiveness of Upper Gastrointestinal Endoscopy,” in *The Implications of Cost-Effectiveness Analysis of Medical Technology/Background Paper #2: Case Studies of Medical Technologies*, prepared by OTA, U.S. Congress (Washington, D. C.: in press, 1980).

This report examines the use of the fiberoptic endoscope to visualize the upper gastrointestinal (UGI) tract from the esophagus to the upper portion of the small intestine. The study covers the effectiveness and economic costs of this common form of endoscopy. Issues related to evaluating endoscopy's benefits and costs are discussed, though no formal comparison of costs and benefits is undertaken.

The authors describe the technique of endoscopy and the device used—the fiberoptic endoscope. They briefly touch on training in the technique and identify the common medical indications for endoscopy's use.

The report discusses the clinical effectiveness of UGI endoscopy, which is used to diagnose conditions of the UGI tract and to obtain specimens of tissue. The medical indications for use are quite broad and inclusive. Studies of the diagnostic value of the technique suggest that endoscopy significantly contributes to the amount of diagnostic information. Very often, however, the medical condition being diagnosed is such that the information gained does not improve morbidity or mortality for the patient(s).

The authors state that the most common dangers associated with endoscopy are perforation (esophagus or stomach), bleeding, cardiopulmonary effects, and infection. These complications are relatively rare, yet not insignificant given the large number of endoscopies performed nationally (at least .500,000 each year).

The authors distinguish between the cost of performing the procedure and the charges for it. Using data from California, they provide a median charge of \$240, and by extrapolation, a total national expenditure of \$122 million. Using a hypothetical cost analysis, they then estimate that the average cost to a physician for performing a routine procedure ranges from \$41 to \$83.

The study addresses issues in evaluating benefits and costs of endoscopies. The authors point out the difficulties of adequately estimating the value of a diagnostic procedure such as endoscopy. They cite the difficulties of conducting a clinical trial ethically when conditions such as gastric cancer are involved. They also cite other difficulties, such as problems in extrapolating from the results of clinical trials in the event that such trials were conducted. The authors maintain that cost-effectiveness studies would be limited in their usefulness because of these difficulties in assessing benefits. Though theoretically possible, measurements of costs and benefits are unlikely since such measures cannot realistically be made sensitive enough to provide an accurate and useful assessment for decisionmakers.

The authors also discuss the use of endoscopy and policy considerations, such as incentives leading to its use and the regulatory issues involved. Finally, the need for increased investigation of more narrowly defined indications for use of endoscopy is discussed.

Stange, P., and Sumner, A., "Predicting Treatment Costs and Life Expectancy for End-Stage Renal Disease," *N. Eng. J. Med.* 298: 372, Feb. 16, 1978.

The objectives of this analysis are to predict the future medical care costs and life expectancy of patient cohorts in facility dialysis, home dialysis, and cadaveric transplantation over the next decade and to estimate the cumulative effect on costs and life expectancy of successive 1,000-patient cohorts, changing methods of treatment in each of the 10 years. Three treatment transition options are evaluated: 1) facility dialysis to home dialysis, 2) facility dialysis to cadaveric transplantation, and 3) home dialysis to cadaveric transplantation. Both costs and life expectancy are discounted at a rate of 7 percent, which is not subjected to sensitivity analysis. The 10-year survival and cost estimates are obtained through linear extrapolation of recent data trends. The experience of the cadaveric-transplantation cohort is predicted for two survival-rate assumptions. The low assumption is based on rates reported in 1976, and the high assumption is an estimate of the average survival rates that will be experienced nationally over the next 10 years. The results of the first phase of the analysis indicate that, over the next decade, each of the dialysis cohorts is predicted to have more added years of life than the transplantation group. Though the predicted number of life-years for both forms of dialysis is approximately equal over the 10-year period, treatment for the home-dialysis cohort will cost **about \$43 million less than that for the facility-dialysis cohort. Transplantation is less costly than both forms of dialysis.**

The second phase of the analysis indicates that undergoing home dialysis instead of facility dialysis (the first option) provides approximately the same life expectancy, but at 34 percent lower costs. The second option, moving from facility dialysis to transplantation, also results in a substantial reduction in costs, but there is an accompanying reduction in life expectancy as well. The third option, moving from home dialysis to transplantation, has results similar to those of the second option. The authors conclude that while it is clear that there are potential savings to society from public policies that encourage patients who are able and willing to shift from facility to home dialysis, an evaluation of the two dialysis-to-transplant options is ambiguous. Transplantation is less costly than dialysis over the 10-year period, but attention must also be paid to the impact of the shift in life expectancy. No cost-effectiveness ratios are presented. The authors caution that the intent of their analysis is not to promote any specific form of treatment, but rather to provide information, such as the relative magnitude of the "tradeoffs" between cost reduction and life expectancy in each of the treatment options.

Stason, W. B., and Fortess, E., "Cardiac Radionuclide Imaging and Cost-Effectiveness," in *The Implications of Cost-Effectiveness Analysis of Medical Technology/Background Paper #2: Case Studies of Medical Technologies*, prepared by OTA, U.S. Congress (Washington, D. C.: in press, 1980).

The authors of this analysis examine a considerable range of issues that deal with the recent growth and ex-

panded use of cardiac radionuclide imaging technology in the health care field. The areas they address are the present and potential future characteristics of the technology; the market for and industry involvement in cardiac imaging innovations; the uses and users of these procedures; the clinical efficacy and risks associated with the techniques; the costs and charges of imaging technology use; and the cost effectiveness of these procedures in different service delivery situations.

The authors point out that much of the rapid diffusion and use in this area is taking place without a well-grounded understanding of the benefits and limitations of the various scanning techniques. To date, only very selected patient populations have been evaluated out of a much broader spectrum of uses and techniques available and in use. Adding to the uncertainty are the rapid technological changes that are occurring and the poorly defined target population for cardiac scans.

Using the various suggested clinical indications and uses as a backdrop, the authors estimate that the potential target population for cardiac imaging could be 134 million people per year if all asymptomatic people 20 years old and over were scanned, 70.8 million people per year if routine screening were limited to those 40 and over, and 11.7 million people per year if scans were restricted to people with suspected or established coronary heart disease.

The study looked at these direct nonlabor costs (equipment, maintenance, radionuclides, etc.), direct labor costs (personnel needs, training, support staff), and indirect costs (overhead) to estimate the financial costs of cardiac scanning services. The authors estimate the annual fixed costs of a model radionuclide laboratory to be \$112,300 for the complete service, with the costs of the various individual procedures ranging from \$258 to \$72 (there are nine different types of procedures and two different types of radionuclide testing materials involved in the range of procedures available). Significant variations exist across the country regarding the charges for the various procedures. Nomenclature and billing procedures/listings are not comparable from hospital to hospital. As a result, it is extremely difficult to determine if there is a relative standard or range of charges for these techniques. The authors developed a set of suggested fee schedules for these procedures that range from **\$405 to \$155** per scan.

The medical literature is examined to determine if there is a proper role for scanning techniques. The authors examine extant studies to determine what types of sample populations have been used, the reference or control groups used, the technical and medical standards against which radionuclide procedures were judged, and the clinical settings in which the studies were conducted. In addition, the authors examine the risks associated with these procedures—both to the health care professionals and the patients—and assess the value of the diagnostic information that the scans provide to the diagnosis or the understanding of the extent of the disease and its response to treatment.

The authors fit the many variables into a cost-effectiveness framework to conduct a limited analysis of cardiac imaging procedures. No discount rate is employed (the benefit, costs, and risks occur in the present), nor is a sensi-

tivity analysis performed. The conclusion is that "decision strategies based on threshold cutoff probabilities of a given disease(s) are cost effective compared to blanket testing . . . and that use of cardiac imaging appears to identify additional surgical candidates at reasonable cost when compared to exercise tolerance testing." The reasonableness of these additional costs will depend, to a large extent, on the incremental health benefits achieved by coronary artery surgery.

The authors identify many of the policy issues raised by this emerging technology. A few of the areas they discuss are issues of reimbursement, safety and efficacy determination, disposal of the radionuclide wastes, clinical standards and indications for use, allocation of resources, and responsibility for regulation and diffusion of these procedures throughout the medical community.

Stason, W., and Weinstein, M., "Allocation of Resources To Manage Hypertension," *N. Eng. J. Med.* 296: 732, Mar. 31, 1977.

CEA is applied to the management of essential hypertension to "determine how resources can be used most efficiently within programs to treat hypertension and to provide a yardstick for comparison with alternative health-related uses of the resources." Costs of treatment consist of the lifetime costs of hypertension treatment, costs of treating diseases that occur during additional years of life gained by antihypertensive treatment, minus the costs that would have been incurred for the treatment of cardiovascular morbid events if treatment had not been given. Effectiveness is calculated in terms of increased years of life expectancy from blood-pressure control, adjusted for changes in the quality of life due to the prevention of morbid events and to the side effects of medication. The analysis is performed under three alternative assumptions concerning the proportional reduction of risk of cardiovascular events and death associated with the reduction of blood pressure due to treatment: 1) full benefit, 2) half benefit, and 3) age-varying partial benefit.

One year of life with side effects is taken to be the equivalent of 0.99 quality-adjusted life years. A 5-percent discount rate is used throughout the analysis. Sensitivity analysis is performed on several critical variables, including the discount rate, medical treatment costs, and the quality-of-life adjustment. In addition, the effects of incomplete adherence to the treatment regimen are examined.

The results of the analysis indicate that in no case does treatment pay for itself. At best, only 22 percent of gross treatment costs, on average, can be recovered from savings in the treatment of strokes and heart attacks. However, the analysis also indicates that, in terms of effectiveness, funds spent to improve adherence may be a better use of resources than efforts to screen a maximum number of subjects.

Steiner, K., and Smith, H., "Application of Cost Benefit Analysis to a PKU Screening Program," *Inquiry* 10:34, December 1973.

The authors compare and contrast the techniques of CBA and CEA, stating that although equally sound deci-

sions may be reached by either method, one of the two is usually better suited for a particular problem. The authors believe that CBA is the best approach for screening programs, and it is this technique that they subsequently use in evaluating a PKU screening program in Mississippi. (PKU is a hereditary condition which causes mental retardation if not detected and treated with a dietary regimen early in life.)

The costs associated with PKU are classified as direct and indirect. Direct costs are defined in this study as the actual expenditures for medical and other services attributable to the disease. Indirect costs are defined as a loss of economic productivity attributable to the disease. These costs serve to measure the benefits of a successful prevention program. The analysis is performed from both a retrospective and a prospective point of view. The retrospective approach measures the costs of the current population with PKU and estimates what the costs of screening, detecting, and treatment would have been. For this study, the direct costs associated with PKU are estimated using data from three mental institutions in Mississippi. Indirect costs are measured by the loss of income, under the assumptions that the PKU victim remains incapacitated for life. Detection costs are based on estimates of the incidence of PKU. The retrospective analysis indicates that the total costs of institutionalization and lost earnings associated with the current Mississippi population with PKU (25 patients) amount to \$2,314,595. The costs of detecting and treating the 25 patients are estimated at \$1,392,668, yielding a cost-to-benefit ratio of 1 to 1.66.

The prospective method calculates the cost of screening all live births in a given year to treating those found to be suffering from PKU. In this study, these calculations are based on the 1967 live births in Mississippi. Testing the 46,714 live births that year would have detected an average of 1.76 PKU cases. The costs associated with these cases amount to \$135,062, if the minimum expected length of institutionalization (30 years) is assumed, or \$256,418, if institutionalization is assumed to cover the normal life expectancy of a 1-year-old child born in 1967 (70.8 years). Program costs are estimated at \$98,518, yielding cost-benefit ratios of 1 to 1.37 and 1 to 2.6, respectively. The authors state that in all calculations, the detection costs are high and the total illness costs (i.e., possible benefit) are low in order to produce conservative results. A discount rate of 4 percent is applied to the lost earnings data, but not to direct or detection costs. Other than varying the length of institutionalization in calculating the prospective cost-benefit ratios, the authors do not perform sensitivity analysis.

Van Pelt, A., and Levy, H., "Cost-Benefit Analysis of Newborn Screening for Metabolic Disorders," *N. Eng. J. Med.* 291:1414, Dec. 26, 1974.

This article examines the costs and benefits of a Massachusetts program designed to detect inborn errors of metabolism and transport in newborn infants. The costs, based on a survey of all hospitals with obstetric and newborn units in Massachusetts, include those for routine specimen collection, laboratory analysis, the collection of additional specimens, confirmatory testing, and followup care

and therapy. For fiscal year 1972-73, these costs amounted to \$460,638. Benefits are calculated as the estimated savings from the prevention of mental retardation and other complications. For 1972-73, estimated total savings amounted to \$825,300, yielding a net benefit of \$364,662 or a benefit-cost ratio of nearly 1.8. Indirect costs of metabolic disorders (such as reduced economic productivity due to disability and premature mortality), which would also be averted as a result of a screening program, are not included in the calculation of benefits. Presumably, the inclusion of the present value of such benefits, when considered along with a similar future stream of the other costs and benefits (also discounted to present value), would result in even higher net benefits.

Wagner, J. L., "The Feasibility of Economic Evaluation of Diagnostic Procedures: The Case of CT Scanning," in *The Implications of Cost-Effectiveness Analysis of Medical Technology/Background Paper #2: Case Studies of Medical Technologies*, prepared by OTA, U.S. Congress (Washington, D. C.: in press 1980).

This study examines the appropriate methodology of CEA/CBA for diagnostic procedures. Following the development of a framework for analysis, the author reviews the literature of the cost effectiveness of CT scanning, critically evaluating it in terms of the evaluation model.

The author describes a theoretical "ideal" evaluative model in which the analysis compares alternative diagnostic pathways, each of which begins with the presentations of signs and symptoms and ends with patient outcomes. The purpose of the evaluation is not to examine the technology per se, but rather to evaluate its appropriate use. The author describes the need for an appropriate means to 1) identify homogeneous patient groups, 2) specify diagnostic pathways, 3) measure diagnostic accuracy, 4) measure diagnostic and therapeutic costs, and 5) specify outcomes of the diagnostic and therapeutic process.

In a review of the literature on the economic impact of CT scanning, only one study that attempted to specify diagnostic pathways was identified. Most of the other studies examined the impact CT has on diagnostic costs or examined the cost of case finding.

Efficacy information is addressed both for diagnostic studies in general and for CT scanners in particular. Comments regarding the potential benefits associated with negative findings are also included.

Costs are distinguished from charges; marginal, or avoidable, costs are recommended; the difficulty of capturing true costs is discussed extensively. Indirect costs are not considered. Discounting was not specifically discussed, except within the context of the reviewed case studies; where, in one, future benefits were discounted. Equity issues were not addressed.

Despite major limitations in applying principles of economic evaluation to diagnostic procedures, such evaluations are feasible. For CT scanning, when sufficient demand exists to operate a scanner at full capacity, some specific uses appear to be cost effective.

Weinstein, M., "Economic Evaluation of Medical Procedures and Technologies: Progress, Problems, and Prospects," in *Medical Technology*, proceedings of the Urban Institute Conference, National Center for Health Services Research, West Palm Beach, Fla., Dec. 10-12, 1978.

This paper presents a state-of-the-art assessment of CBA and CEA of medical procedures. CBA/CEAs are defined and distinguished from each other. The author advocates the use of a multiattribute accounting framework, in conjunction with CEA and CBA, in which unquantifiable concerns, such as equity and ethical issues, are considered along with the traditional, measurable impacts. The basic methodological principles are reviewed, including estimation of event rates, sensitivity analysis, choosing a discount rate, measurement of costs, and measurement of benefits. The controversy surrounding the assignment of monetary value to life saving and health improvement in CBA is discussed.

A review of selected applications, classified as treatment, secondary prevention, screening, and immunization, is presented. The author states that diagnostic procedures other than screening have not received much attention, in part because of methodologic obstacles. He predicts that technology evaluation will be the area where the next major advances in CEA and CBA will develop. He then discusses current methodologic problems, classified as 1) the valuation of multiattributed outcomes, 2) the evaluation of diagnostic tests, 3) the evaluation of multifaceted technologies, and 4) uncertainty concerning efficacy, costs, and ultimate uses of evolving technology.

The paper concludes with a generally optimistic assessment of the prospect for CEA and CBA in medical care and for overcoming the current methodological problems. The author recommends a multidisciplinary approach to analysis, including the expertise of physicians, engineers, and economists. He notes that the value of formal economic analysis lies not so much in the actual results, but rather in the ability of such analysis to highlight uncertainty and the most important value tradeoffs involved in alternative policies.

Weinstein, M. C., and Pearlman, L. A., "Case Study on Cost-Effectiveness of Automated Multichannel Chemistry Analyzers," in *The Implications of Cost-Effectiveness Analysis of Medical Technology/Background Paper #2: Case Studies of Medical Technologies*, prepared by OTA, U.S. Congress (Washington, D. C.: in press, 1980).

This study illustrates the possible techniques for evaluating the cost effectiveness of automated multichannel chemistry analyzers. The authors also examine and discuss the limitations due to data deficiencies, areas for future research, and influences of clinical practice on the evaluation of such analyzers.

The case study briefly reviews the history of the multichannel clinical chemistry technology and presents an analytical framework for evaluating the cost effectiveness of the multichannel analyzer. The authors review the available data concerning the costs of multichannel chemistry

analyzers and examine the evidence concerning the cost effectiveness of using the cardiac enzymes and isoenzymes in the diagnosis of myocardial infarction.

The paper discusses several important issues related to the clinical efficacy and cost effectiveness of clinical laboratory chemical tests. A prominent example of such an issue is the potential influences on physicians' test-ordering behavior that may be induced by the availability of multichannel analyzers.

Various types of automated multichannel chemistry analyzers could be compared to one another under specified circumstances. Continuous flow models, for example, could be compared to discrete sample models and even single channel models to find the most cost-effective method of running specific cardiac enzyme tests. The study also discusses the cost effectiveness of using automated multichannel chemistry analyzers to obtain laboratory values as compared to not obtaining that laboratory value at all. Further, the authors advocate comparing the efficiency of using the automated multichannel chemistry analyzers under varying workloads (i. e., the number of tests performed per unit of time).

The study addresses the adequacy—or inadequacy—of efficacy information. As usual, such information is not plentiful; studies designed to produce efficacy data are underway. The authors discuss alternative ways to use the technology in various forms to produce the greatest degree of efficiency. The authors also discuss the variability of benefits resulting from use of automated multichannel chemistry analyzers. Potential benefits are described from a societal perspective and include potential reduced costs from reduced incidence of unnecessary hospitalization resulting from more accurate diagnostic and monitoring testing. Health benefits would be measured in quality-adjusted life years.

Costs are distinguished from charges, and several direct costs are identified, including those for nonlabor (e. g., equipment, service and maintenance, reagents, and consumables) and labor. Fixed, variable, and induced costs are all addressed. The authors state that indirect costs have not been adequately studied and may not be extensively affected by automated analyzers. Discounting would be included in analyses described in this case study, as would the use of sensitivity analysis. The extent to which sensitivity analysis would be used is not explained explicitly.

Data results are not derived from this case study; however, the many different ways the CEA could be conducted are discussed. Each different approach would yield results of a different meaning; hence several caveats would be needed for each approach and set of results.

The authors discuss the potential public policy implications of this analysis which could affect reimbursement policies regarding laboratory tests, the use of automated analyzers by hospitals and physicians, and the design of equipment by manufacturers. No conclusions regarding the cost effectiveness of automated multichannel chemistry analyzers can be drawn from this study. The study was not designed to be an actual assessment; rather, it was intended to illustrate how a CEA of automated analyzers could be performed.

Weinstein, M. C., and Stason, W., "Foundations of Cost-Effectiveness Analysis for Health and Medical Practices," *N. Eng. J. Med.* 296: 716, Mar. 31, 1977

This article presents principles of CEA as applied to the allocation of health care resources. The authors caution that in conducting an analysis, the objectives of the actual decisionmaker may be more relevant than the societal point of view. Whenever possible, measures of effectiveness should be expressed in outcome-oriented terms, such as length of life and quality of life. Tradeoffs between present and future health benefits and costs, and hence the use of discounting, are discussed. Net health care costs are expressed as the sum of costs associated with treatment, side effects, and increased longevity less the savings from decreased morbidity. Net health effectiveness is expressed as the expected number of quality-adjusted life years gained, calculated as the expected number of unadjusted life years, adjusted for improvements in the quality of life due to the alleviation or prevention of morbidity and side effects of treatment. Sensitivity analysis is described, and its use is recommended whenever uncertainty is involved in the estimation of key variables (e. g., discount rates, clinical efficacy, prevalence, etc.). The article ends with a discussion of the value and application of CEA in health care and concludes that its principle value is that it forces one to be explicit about the beliefs and values that underlie allocation decisions.

Weisbrod, B., "Costs and Benefits of Medical Research: A Case Study of Poliomyelitis," *J. Polit. Econ.* 79: 527, May-June 1971.

This CBA of poliomyelitis research uses and expands on the benefit calculations first presented in Weisbrod's *The Economics of Public Health* (578). These calculations comprise savings from avoided premature mortality, morbidity, and treatment and rehabilitation costs. The analysis requires an estimation of 1) the time stream of research expenditures directed toward poliomyelitis, 2) the time streams of a number of forms of benefits resulting from (or predicted to result from) the application of the knowledge generated by the research, and 3) the cost of applying that knowledge.

Using this information, Weisbrod calculates internal rates of return on research expenditure. Savings per case prevented, application costs, the time horizon, and research expenditures are all subjected to sensitivity analysis. The internal rates of return were found to be sensitive to application costs, varying from 4 to 14 percent. In approximating present value of expenditures and benefits, Weisbrod uses a discount rate of 10 percent. No sensitivity analysis is performed on this variable. The difficulties encountered in trying to associate specific medical research expenditures with a particular disease are discussed. These include the fact that basic research is often not directed at a specific disease problem and even disease-specific research frequently yields knowledge relevant to the prevention or treatment of other diseases. The data used here are estimates of awards for poliomyelitis research from 1930 to "1956. Weisbrod stresses the need to include the costs in-

volved in the application of new medical knowledge, as well as the costs of generating it, when attempting to comprehensively analyze a medical research program.

The article concludes with an interesting discussion of the impact on private market allocative efficiency **when a** collective consumption good (e. g., medical research) requires for its application a procedure such as vaccination which is provided individually and from which nonpayers may be excluded. Weisbrod also discusses the effects of externalities on the provision of medical research and its application for contagious diseases. The author concludes that when collective consumption goods require use of individual consumption goods for their application, and where these individual goods produce real external economies, neither the nature nor the extent of private market inefficiency is clear.

Witte, J., et al., "The Benefits From 10 Years of Measles Immunization in the United States, " *Public Health Rep.* 90: 205, May-June 1975.

For the 10-year period 1963-72, the authors estimate the costs the Nation would have sustained without measles im-

munization (i. e., the benefits of measles immunization) and the actual costs of measles during that period in terms of illness and associated resources consumed. The research costs of developing and testing the measles vaccine are not included because of the difficulty in identifying them and in determining the share applicable to the United States in the period under consideration.

The benefits associated with the measles immunization program considered in this analysis include 1) savings in medical care costs for services of physicians and for *long-term* institutional care for those who would have become retarded, and 2) avoidance of production losses due to morbidity and premature mortality. Program costs are those incurred in vaccine production, distribution, administration, and promotion. The analysis concludes that the net benefits achieved through immunization in the United States totaled \$1.3 billion for the period 1963-72. A single discount rate of 4 percent is used. The authors assume that the national immunization effort had no significant effect on the demand for medical care *or* on the size and composition of the labor force.