THIRD EDITION

EVALUATING THE HEALTHCARE SYSTEM

EFFECTIVENESS, EFFICIENCY, AND EQUITY

Lu Ann Aday • Charles E. Begley
David R. Lairson • Rajesh Balkrishnan

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Health Administration Press, Chicago, Illinois
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MORE PRAISE for the third edition of
Evaluating the Healthcare System . . .

". . . As the leader of a large, multidisciplinary health services research unit, I use this text to introduce our new physician scientists and social scientists (faculty and post-doc) to the field of health services research. In one-on-one sessions, the chapters of this text provoke excellent discussions and debates, and it is a commonplace occurrence for me to see the faces of junior faculty and fellows light up with discovery and insight, provoked by the reading. The new edition is enhanced by the greater coverage of the population dimensions of health services research and a more in-depth treatment of policy analysis. In my view, there is no better health services research text available. The authors have made an incredibly useful and insightful contribution to our field."

—Carol M. Ashton, M.D., M.P.H., professor of medicine, Baylor College of Medicine, Houston, TX

"The book provides an excellent framework for understanding and evaluating healthcare systems and change. By focusing separately on the key system outcomes—effectiveness, efficiency, and equity—and carefully laying out relevant concepts before applying them to major policy issues, the authors provide an unusually thorough treatment of the field of health services research."

—Thomas Rice, Ph.D., professor of health services, University of California at Los Angeles School of Public Health

". . . The book is very well written. It makes complex issues very clear and understandable, and useful. I would highly recommend it to investigators, policy makers and graduate students who are interested in understanding how to develop health policies that are informed by the strongest empirical evidence and to evaluate the effectiveness of these policies for achieving their intended results."

—Morris Weinberger, Ph.D., Vergil N. Slee distinguished professor of healthcare quality management, department of health policy and administration, School of Public Health, University of North Carolina at Chapel Hill

"This third edition of *Evaluating the Healthcare System: Effectiveness, Efficiency, and Equity* . . . integrates the theories, issues, approaches, and methods of health services research better than any other health services research text in or out of print. It is comprehensive rather than distractingly encyclopedic. While the book serves as an excellent introduction to the field of health services research for people trained in primary disciplines who are new to the field, it is also excellent for the experienced researcher who wishes to deepen their comprehension of a particular issue. The conceptual framework provided in the text unifies theories and findings from many fields. The text reflects the wide-ranging yet deep expertise of the authors and provides invaluable insights to scholars of all levels..."

—Nelda P. Wray, M.D., M.P.H., professor of medicine,
Baylor College of Medicine, Houston, TX

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Foreword

MUCH HAS HAPPENED since publication of the second edition of *Evaluating the Healthcare System: Effectiveness, Efficiency, and Equity*. In addition to continuing issues of access and cost of care, the country faces significant variations in quality and outcomes of care across providers, documented disparities and inequalities in care among different ethnic groups, and a growing epidemic of obesity and chronic illness. Health services research has made major contributions to bringing these issues to light and has led to renewed efforts for transparency and accountability in our healthcare system.

At the same time, there appears to be growing interest in taking a broad ecological approach that recognizes that health is "produced" by a complex interaction among biological, environmental, and behavioral factors operating over a person's lifetime within the context of given populations and communities. This broad-based approach also underscores the permeability between the healthcare delivery system, the public health system, and broader societal forces as they influence human health. The third edition of *Evaluating the Healthcare System* recognizes this larger reality.

The book remains anchored in the three central dimensions of effectiveness, efficiency, and equity. In most respects, these envelop the six aims of any health system as outlined by the Institute of Medicine's *Crossing the Quality Chasm* report—that care should be safe, effective, efficient, timely, personalized, and equitable. New and updated evidence is provided on most of these dimensions, and an explicit *health-centered* public policy framework is advanced. Breast cancer screening

is used as a unifying example throughout the discussion of the various evaluation models and approaches. Of particular interest to many readers will be the application of various theories of justice—deliberative, distributive, and social—to measures of equity that embrace biological, behavioral, and environmental factors influencing health.

Because of its broader frame of reference, the utility of the third edition extends beyond the obvious audience of students and scholars in health services research and health management and policy to those interested in health and social behavior, social epidemiology, and community-based participatory research, among others. The authors are to be commended for broadening their framework of evaluation and, in doing so, have made an even greater contribution to increasing our understanding of how the healthcare system interacts with other factors to improve human health.

Stephen M. Shortell, Ph.D.
Blue Cross of California Distinguished Professor
of Health Policy and Management
and Dean, School of Public Health
and Professor of Organizational Behavior
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Preface

THIS BOOK DEFINES and illustrates the application of the effectiveness, efficiency, and equity criteria for evaluating healthcare system performance. It introduces and integrates the fundamental concepts and methods of health services research as a field of study and illustrates their application to policy analysis. Specific examples of the application of health services research in addressing contemporary health policy problems at the national, state, and local levels are presented.

The primary audiences for the book are practicing professionals and graduate students in public health, health administration, and the healthcare professions and federal, state, and local policymakers and program planners charged with the design and conduct of policy-relevant health services research. Professionals and students in medical sociology, the behavioral sciences, and public administration interested in conducting applied or policy-oriented health and healthcare research will also find the book of considerable interest. The authors developed and applied the perspective presented in the book in a course they have offered to master's and doctoral students in public health since 1986.

Revisions for the third edition of the book in particular draw on a growing body of research on the social and economic determinants of population health and explore the distinct and complementary roles of health services and public health research and policy in improving the health of individuals and communities. Breast cancer screening and related cancer morbidity and mortality, an important healthcare and public health problem area, is woven throughout the book in

demonstrating the effectiveness, efficiency, and equity concepts in the context of these issues.

In general, the revised edition of the book is intended to encompass a broader and more integrative look at the role of both population (or public) health and personal (or medical) care services in enhancing community and individual well-being; the design of health services research to assess the effectiveness, efficiency, and equity of public health and healthcare programs; and the normative and empirical application of the effectiveness, efficiency, and equity criteria in evaluating specific health policy alternatives.

Chapter 1 presents a framework for applying health services research to policy analysis. This framework is utilized to provide an integrative overview of the contributions of health services research to describing and evaluating the performance of the healthcare system with respect to the objectives of effectiveness, efficiency, and equity. This chapter defines the relationship between health services research and the major objectives and methods of policy analysis. The review of evidence and trends within the U.S. healthcare system, based on the health policy analysis framework, is highlighted.

Chapters 2, 4, and 6 introduce the concepts and methods of effectiveness, efficiency, and equity research, and Chapters 3, 5, and 7 review the policy strategies that have emerged to accomplish these objectives, as well as the criteria and evidence regarding their success in achieving them.

The effectiveness chapters (Chapters 2 and 3) introduce and apply a conceptual framework integrating methods for assessing the effectiveness of medical and nonmedical interventions from both the population and clinical perspectives.

The efficiency discussion (Chapters 4 and 5) examines the concepts of production and allocative efficiency and the major findings regarding the performance of the United States' and other countries' healthcare systems with respect to these objectives.

The equity chapters (Chapters 6 and 7) introduce a conceptual framework of equity, grounded in emerging and expanded theoretical dimensions of deliberative, distributive, and social justice, and apply it to assessing the progress of the U.S. healthcare system in achieving equity along each of these dimensions.

Chapter 8 analyzes the interrelationships between and among the objectives of effectiveness, efficiency, and equity and the role of health services research in conceptualizing and measuring the trade-offs among

them in formulating health policy. Chapter 9 discusses these trade-offs in the context of a policy analysis example evaluating mammography screening for older, Medicare-eligible women.

The unique contributions that this book makes are the following: (1) it presents and applies an organizing framework for defining health services research as a field of study, in the context of the major system performance dimensions of effectiveness, efficiency, and equity; (2) it reviews and integrates the conceptual, methodological, and empirical contributions of health services research to addressing these issues; (3) it illustrates how the perspectives and methods of effectiveness, efficiency, and equity research can be used to anticipate and pose relevant questions to inform both current and future healthcare policy debates; and (4) it provides a primer and point of reference at a time when both the support for and demands on health services research and policy analysis are increasing.

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Charles E. Begley
David R. Lairson
Rajesh Balkrishnan

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Special thanks go to Anne D. Wiltshire, who carefully compiled and edited the extensive references and data tables for the book and who served as the teaching assistant for the course on which the book is based.

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We want to especially acknowledge and recognize the role that our former colleague and coauthor for the first and second editions of the book, Carl H. Slater, M.D., played in establishing the foundation for the effectiveness chapters.

We are grateful for the flexible environment at the University of Texas School of Public Health, which supported the rewarding task of writing the book as a routine component of faculty roles and responsibilities.

We owe a special debt to the students in our course on health services delivery and performance throughout the years, who stimulated and challenged us to sharpen our mastery of the ideas put forth in the book.

Each of us feels that our understanding of the concepts of effectiveness, efficiency, and equity has been broadened and deepened in the process of writing the book. Our hope is that those who read it will be similarly rewarded.

Chapter 1

Introduction to Health Services Research and Policy Analysis

CHAPTER HIGHLIGHTS

1. *Health services research* produces knowledge about the performance of the healthcare system, and *policy analysis* applies this knowledge in defining problems and evaluating policy alternatives.
2. Effectiveness, efficiency, and equity are key criteria for evaluating healthcare systems and policies.
3. *Effectiveness* examines the extent to which healthcare improves the health of patients and populations, *efficiency* evaluates these improvements in relationship to the resources required to produce them, and *equity* is concerned with health disparities and the fairness and effectiveness of the procedures for addressing them.
4. Evidence suggests that the effectiveness of healthcare is either highly variable or limited, healthcare costs relative to benefits are substantial, and wide disparities in health and healthcare exist between groups in the United States and those in other countries.
5. This book presents and applies a framework for evaluating the effectiveness, efficiency, and equity of healthcare programs and policies at a variety of levels.

INTRODUCTION

"The goal of health services research is to provide information that will eventually lead to improvements in the health of the citizenry" (NICHSR 2002). This book provides guidance for applying the concepts and methods from health services research and policy analysis in assessing the effectiveness, efficiency, and equity of healthcare programs and policies in achieving this objective.

THE FIELDS OF HEALTH SERVICES RESEARCH AND POLICY ANALYSIS

Health services research produces knowledge about the performance of the healthcare system, and policy analysis applies this knowledge in defining problems and evaluating policy alternatives. This book delineates and defines the working partnership between health services research and policy analysis in assessing the performance of the U.S. healthcare system with respect to the objectives of effectiveness, efficiency, and equity, where

1. *effectiveness* examines the benefits of healthcare measured by improvements in health,
2. *efficiency* relates these health improvements to the resources required to produce them, and
3. *equity* is concerned with health disparities and the fairness and effectiveness of the procedures for addressing them.

Effectiveness focuses on the benefits produced by healthcare, as measured by improvements in people's health. Improvements in health include not only the sum of the individual benefits—that is, improved health-related life quality, reduced mortality rates, increased life expectancies, and decreased prevalence of disease—but also make reference to a distribution of disease and health in a way that maximizes overall economic productivity and well-being. The clinical perspective on effectiveness assesses the contribution of medical care to improving the health of individuals, while the population perspective assesses the contribution of medical and nonmedical (e.g., environmental and behavioral) factors to the health of communities as a whole.

A second major objective of the healthcare delivery system is the drive for *efficiency*. Where healthcare is viewed as an output, the focus is on production efficiency (producing services at least cost), and where healthcare is viewed as an input in the production of health improvements, the emphasis is on allocative efficiency (maximizing health given constrained resources).[1] Allocative efficiency depends on the relative cost and effectiveness of medical and nonmedical investments in improving health. Ultimately, maximization of health requires both production and allocative efficiency.

Equity is concerned with health disparities and the fairness and effectiveness of the procedures for addressing them. The ultimate test of the

equity of health policy is the extent to which disparities or inequalities in health persist among subgroups of the population. Substantive equity is reflected in minimizing subgroup disparities in health. Procedural equity refers to the extent to which the structure and process, or procedures, for achieving these outcomes may be judged to be fair. The normative relevance of variations in the structure and process of care ultimately, however, can be judged empirically by the contributions of these variations to predicting inequalities in health across groups and communities.

The effectiveness, efficiency, and equity criteria provide a broader perspective, grounded in relevant theory and research, for assessing the performance of health policies and programs in achieving the overall healthcare system goals of quality, cost containment, and access, respectively. The effectiveness, efficiency, and equity criteria are often complementary. Improving healthcare effectiveness while holding resources constant increases efficiency. Increases in efficiency create opportunities for improved effectiveness and equity. However, the objectives may also be in conflict. Maximizing effectiveness by allocating additional resources to improve health may conflict with efficiency if the cost of the resources is high relative to their effectiveness. Maximizing effectiveness and efficiency by distributing resources to persons who would gain the most may be deemed unfair in terms of procedural equity if the policy leads to a very uneven distribution of these resources.

Identifying trade-offs among the three objectives that often arise in complex policy choices is an important product of health services research. Assuming effectiveness, efficiency, and equity are important policy objectives, a key question for decision makers in comparing alternatives is the degree to which one objective must be sacrificed to achieve the others.

This book will explore the common ground for integrating and applying the effectiveness, efficiency, and equity criteria in evaluating the extent to which health policies contribute to improvements in the health and well-being of patients and populations. Health services research on effectiveness provides evidence on what medical and nonmedical interventions result in the greatest health improvements. Efficiency studies compare the costs and benefits of producing these improvements given constrained resources, and equity analyses provide guidance for assessing whether both the investments and outcomes in terms of health are distributed fairly.

The chapters that follow review the conceptual, methodological, and empirical foundations for the effectiveness, efficiency, and equity objectives; show how they are applied in policy analysis; and examine the health services research questions posed in analyzing the complementarity and trade-offs between these objectives in formulating and evaluating health policy.

In this chapter, the fields of health services research and policy analysis are compared and contrasted with other types of inquiry. We present a framework for classifying topics and issues in health services research and use this framework to provide a descriptive overview of the U.S. healthcare system. Historical contributions of health services research to the development of health policy are highlighted, and selected applications in terms of current U.S. policy debates are introduced.

Health Services Research

A 1979 Institute of Medicine (IOM) panel charged with defining and evaluating the field of health services research offered the following definition of the enterprise: "Health services research is inquiry to produce knowledge about the structure, processes, or effects of personal health services" (IOM 1979, 14). A study could be classified as health services research if it dealt primarily with "personal health services" and drew upon a conceptual framework other than that of applied biomedical science, which primarily focuses on the fundamental life processes of the human organism. Personal health services were defined as transactions between providers and clients for the purpose of promoting the health of the clients. These transactions largely fall within the domain of the medical care system, in contrast to public health, which focuses on interventions to promote the health and well-being of the community or the population as a whole rather than that of particular individuals within it.

A more recent IOM (1995, 17) report that addressed how best to plan for and develop the health services research workforce offered the following revised definition of the field: "Health services research is a multidisciplinary field of inquiry, both basic and applied, that examines the use, costs, quality, accessibility, and delivery, organization, financing, and outcomes of healthcare services to increase knowledge and understanding of the structure, processes, and effects of health services for individuals and populations." This definition differs from the for-

mer primarily in acknowledging that health services research serves to make contributions to basic, as well as applied, research in selected areas (e.g., the operation of medical care markets in health economics theory) and that it is concerned with studying a broader continuum of healthcare services, focusing on population-based as well as personal services.

A committee convened by the Academy for Health Services Research and Health Policy (now AcademyHealth) in 2000 defined health services research as "...the multidisciplinary field of scientific investigation that studies how social factors, financing systems, organizational structures and processes, health technologies, and personal behaviors affect access to healthcare, the quality and cost of healthcare, and ultimately our health and well-being. Its research domains are individuals, families, organizations, institutions, communities, and populations" (Lohr and Steinwachs 2002, 8).

This definition highlights the following advances in the field:

1. Health services research has matured as a scientific field of study through its contributions to the development and testing of theories regarding the operation and impact of healthcare markets and systems.
2. The field has expanded to encompass the examination of an array of factors (including nonmedical social factors) in influencing the health and well-being of patients and the public.
3. The revised definition acknowledges the importance of research and interventions at a variety of levels, given the growing complexity of the U.S. healthcare system.

Figure 1.1 displays the continuum of programs and services that would be incorporated in a comprehensive integrated healthcare system. The continuum implies continuity and integration over time and between components in the context of promoting and protecting the health of individuals and populations through primary prevention to inhibit the onset of health problems, secondary prevention to restore a person who is already affected to maximum functioning, and tertiary prevention to minimize the deterioration of function for those with problems that are essentially not curable. The provision of ambulatory and acute institutional care within the conventional medical care system encompasses the treatment-oriented center of the continuum.

Community social and economic programs as well as public health programs and policies define the primary prevention-oriented beginning of the continuum; and long-term institutional, home, and community-based care extend the continuum to enhancing the quality of life and maximizing the functioning of the chronically ill or disabled. The prevention-oriented and long-term care poles encompass an array of nonmedical as well as medical programs and services directed toward promoting or protecting the health of the public and individuals.

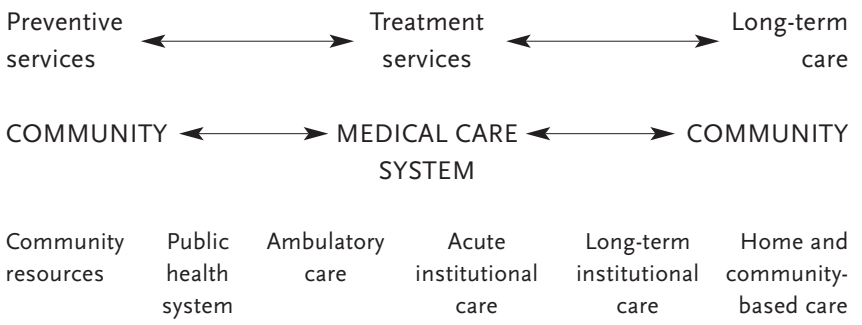
Health services research is inherently interdisciplinary in focus in that it draws on and applies theories and methods from an array of disciplines, including sociology, political science, epidemiology, demography, economics, law, and medicine, among others (Choi and Greenberg 1982; Ginzberg 1991; NICHSR 2002). Basic disciplinary research is primarily concerned with the development and testing of theories to explain social or biological phenomena, while health services research applies the theories and methods that have evolved within these disciplines to investigating problems related to the operation and performance of the healthcare delivery system. Further, whereas clinical research is principally concerned with medically related services and outcomes for individual patients, health services research more broadly acknowledges the array of nonmedical (i.e., social, economic, and organizational) factors that may help to promote health or prevent illness (see Figure 1.2).

Policy Analysis

Policy analysis is defined in terms of two principal objectives: (1) the production of information relevant to understanding social problems and identifying policy solutions and (2) the development of reasonable arguments translating the information into recommendations for governmental action (Dunn 2003). The distinction between health services research and policy analysis is that the first objective—the production of knowledge—defines the primary contributions of health services research, and the second—the application of knowledge—represents the primary contributions of health policy analysis to governmental decision making.

The first objective most directly mirrors the goal of health services research that is concerned with generating knowledge about the need for, implementation of, and effects of specific health services programs and policies. The principal questions and issues being addressed are factual or objective: to document the origins, scope, and causes of a

Figure 1.1 Continuum of Healthcare Services



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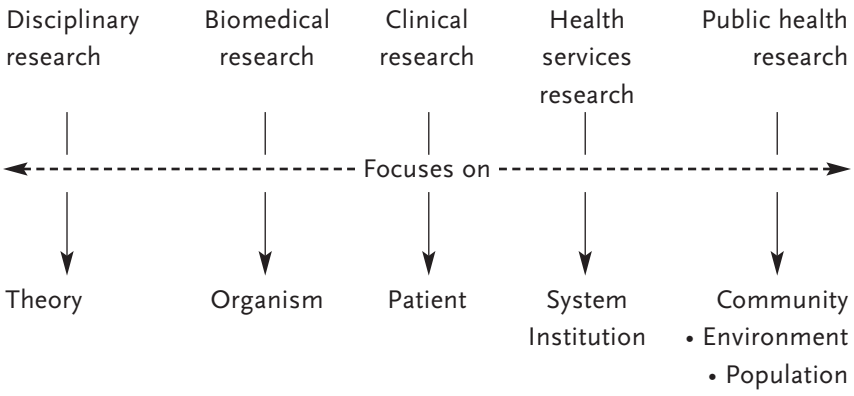
social condition of concern to policymakers (e.g., the proportion of the population and subgroups without insurance coverage) and project or evaluate the probable consequences of alternatives being considered for addressing it (e.g., cross-national comparisons of alternative systems of financing medical care).

The second objective extends somewhat beyond the role traditionally assumed by health services research. This objective involves synthesizing information to justify the relevance of particular types of research, to weigh existing evidence and compare trade-offs among competing objectives, and to construct a recommendation for policymakers regarding the nature and significance of a problem or the utility of a specific program or policy proposal. The primary emphases of this objective are normative and prescriptive: to provide a logical, well-documented rationale for choosing among competing views of the adequacy of existing policies (e.g., in providing insurance coverage) or between alternative strategies (health insurance reform) to achieve competing health policy goals (effectiveness, efficiency, and equity).

These objectives assume a rational, problem-solving process of policy development that is not realistic given the complex set of institutions and political processes that determine health policy in the United States. These elements of policymaking include the attitudes, concerns, and opinions of the public at large and of special interest groups; their respective and relative ability to influence the decision-making process;

Figure 1.2 Comparison of Focus of Health Services Research with Other Types of Research



the values of elected and nonelected officials that lead the institutions and processes making these decisions; and the nature and content of competing items on the policy agenda. Health services research and policy analysis may influence policymaking by providing information and analysis of problem conditions (e.g., the limited willingness of providers to see Medicaid patients), determining the consequences of possible solutions (increased provider fees, extended medical liability coverage, or Medicaid managed care), and evaluating the pros and cons of alternative policy proposals. The political will to apply this information to the policymaking process is not always forthcoming. Attempts at national healthcare reform in the 1990s provide evidence on the uses of information and analyses to enrich as well as distort policy debates (Rushefsky and Patel 1998).

At the end of the chapters presenting the policy strategies and the evidence regarding the effectiveness, efficiency, and equity of healthcare (Chapters 3, 5, and 7), criteria for assessing problems with existing policies and evaluating alternatives from each of these perspectives will be identified and illustrated in the context of breast cancer screening and treatment. The final chapter applies these criteria in a policy analysis of mammography screening policy for older Medicare-eligible women.

Comparisons with Other Types of Inquiry

Figure 1.3 contrasts health services research and policy analysis with other types of basic and applied scientific inquiry in terms of the primary research objectives of each. Disciplines (e.g., economics) provide useful theories (of demand and supply) to explain biological or social phenomena (the operation of consumer and provider behavior in the medical care marketplace). These theories underlie the ways in which health services research describes and assesses the performance of the healthcare system—in terms of efficiency, for example. Health program evaluation is concerned with assessing the effect of specific policies and programs (e.g., alternative health education or clinical screening strategies to prevent cancer) on a defined policy outcome of interest (e.g., survival or quality of life) and applies the concepts and methods of health services research in evaluating these alternatives. Evaluating the implementation and effect of healthcare programs such as community-based outreach, physician education, or financial incentives to encourage preventive behavior and service use has been a major activity of health services research (Grembowski 2001; Shi 1997). To the extent that such evaluations are directed toward assessing specific governmental policies or programs, they may provide direct input to related health policy analysis efforts. Policy analysis draws on the fund of knowledge generated by disciplinary and health services research to (1) define and analyze current problems (e.g., cost, access, or effectiveness of cervical cancer screening) and (2) compare and evaluate health policy alternatives (counseling safe sex practices).

Health services research has been criticized historically for not being sufficiently involved in the conduct of research that directly informs difficult health policy decisions (Anderson 1991; Choi and Greenberg 1982; Flook and Sanazaro 1973; Ginzberg 1991; IOM 1979, 1991, 1995; Lavis et al. 2002). Compilations of the contributions of health services research to health policy and management do clearly indicate, however, that the lines between health services research and policy analysis are more aptly characterized as diffuse, rather than distinct. Health services research has been directly used in evaluating a variety of policy options, such as the cost, quality, and access implications of alternative universal health insurance proposals and of enrolling Medicaid- and Medicare-eligible individuals in managed care (Altman and Reinhardt 1996; Brown 1991; DeFriese, Ricketts, and Stein 1989; Ginzberg 1991; Shi 1997; Shortell and Reinhardt 1992; White 1992).

Figure 1.3 Comparison of Objectives of Health Services Research with Other Types of Inquiry

Type of Inquiry	Objective
Disciplinary research	To explain biological or social phenomena $X \longrightarrow Y$
Health services research	To describe and assess the performance of the healthcare system Structure Process Outcome $X \longrightarrow Y$
Health program evaluation	To evaluate the effect of health policies and programs $x_0 \longrightarrow y_0$ $x_1 \longrightarrow y_1$ $x_2 \longrightarrow y_2$ $x_3 \longrightarrow y_3$
Health policy analysis	To analyze and compare alternative (1) problem definitions and (2) health policy solutions (1) Problem analysis: x_1 vs. x_2 vs. x_3 → X (2) Solution analysis: y_1 vs. y_2 vs. y_3 → Y

A Historical Overview of Policy-Related Health Services Research

Health services research is a relatively new field of inquiry, although its origins may be traced to the early 1900s in the United States. Selected historical contributions of health services research to the formulation of health policy are highlighted here. (For more detail, see Anderson 1991; Flook and Sanazaro 1973; IOM 1995; McCarthy and White 2000; and NICHSR 2002.)

The Flexner report, based on a comprehensive study of medical schools in the United States and Canada, was published in 1910. This report led to a major reorganization of medical education in the United States (Flexner 1910).

The Committee on the Costs of Medical Care (CCMC) was established in 1927. That prestigious 42-member committee played a major role in the design and conduct of research on the utilization and costs of care and on the inequities of access that existed among income groups. The committee published 28 reports, including a series of reports and recommendations that affected and continue to affect how medical care is organized and delivered in the United States (CCMC 1933).

In 1935–36, the Public Health Service conducted a national health survey and a business census of hospitals to provide basic data on the health and healthcare needs of the population and on the financial structure of U.S. hospitals. An outgrowth of this early research was the development of the concept of health service areas for general hospitals and health centers. In 1944, the American Hospital Association (AHA) established its Commission on Hospital Care, which provided the first complete inventory of the nation's hospitals. This and the earlier business census identified a need for more general hospital beds, especially in rural areas, which resulted in the passage of the Hill-Burton Act in 1946, authorizing a massive nationwide hospital survey and construction program.

The Commission on Chronic Illness, established in 1949 under the auspices of AHA, the American Medical Association, the American Public Health Association, and the American Public Welfare Association, carried out a number of studies dealing with the community prevalence and prevention of chronic illness, long-term care, and home care. The AHA Commission on Financing, established in 1951, attempted to address many of the issues related to the financing of hospital care (i.e., the factors affecting cost, prepayment, and financing of care for nonwage and low-income groups) that had not been dealt with directly by the 1944 AHA Commission on Hospital Care. The research carried out by these national commissions contributed to early deliberations concerning the appropriate role of the federal government in healthcare (as in President Truman's Commission on the Health Care Needs of the Nation), as well as to the development of survey research methodologies and statistical and economic analysis methodologies that were to provide the foundation for contemporary health services research.

The U.S. Department of Health, Education, and Welfare (DHEW) was established in 1953. The National Health Survey Act, which provided authorization for the major data-gathering efforts of the National Center for Health Statistics, was passed in 1956. The research conducted under the auspices of these agencies documented continuing inequities in health and healthcare for the poor and the elderly in particular—inequities identified more than 20 years earlier by the Committee on the Costs of Medical Care. The evidence of these persistent disparities provided an empirical foundation for passage of the Medicaid and Medicare legislation in 1965, which extended federally subsidized coverage to these groups.

The formalization of health services research at the federal level resulted from the creation of a National Institutes of Health Study Section on Health Services Research in 1960, formed from the merger of Public Health Research and Hospital Facilities Research Study Sections. The lead federal agency for support of formal health services research activities, the National Center for Health Services Research and Development, was established in 1968. During the intervening period, a number of other federal agencies (e.g., the Veterans Administration, Health Care Financing Administration [now Centers for Medicare & Medicaid Services], National Institute of Mental Health, and National Institute of Aging) as well as private foundations (e.g., the Robert Wood Johnson Foundation, the Commonwealth Fund, the Kaiser Family Foundation, and the Pew Foundation) assumed a greater role in supporting the design and conduct of health services research activities.

The first national meeting of the Association for Health Services Research and the Foundation for Health Services Research was held in Chicago in June 1984. In 1989, the National Center for Health Services Research received a substantial boost in funding for research on patient outcomes and medical effectiveness as a result of major outcomes research bills introduced by Congress; the agency itself was subsequently renamed the Agency for Health Care Policy and Research (AHCPR) to reflect its more policy-oriented focus. In 1999, AHCPR was reauthorized as the Agency for Healthcare Research and Quality, establishing it as the lead federal agency on quality-of-care research, with responsibility to coordinate all federal quality-improvement efforts and health services research (AHRQ 2003).

In the chapters that follow, the contributions of health services research in general as well as the contributions of specific studies in particular, such as the RAND Health Insurance Experiment and the

Medical Outcomes Study, in clarifying and evaluating health policies in terms of effectiveness, efficiency, and equity will be examined.

FRAMEWORK FOR APPLYING HEALTH SERVICES RESEARCH IN EVALUATING HEALTH POLICY

Description of the Framework

A framework for applying health services research in evaluating health policy is provided in Figure 1.4. The framework is based on adaptations of the framework for classifying topics and issues in health services research introduced in the first and second editions of this book (Aday et al. 1993, 1998).

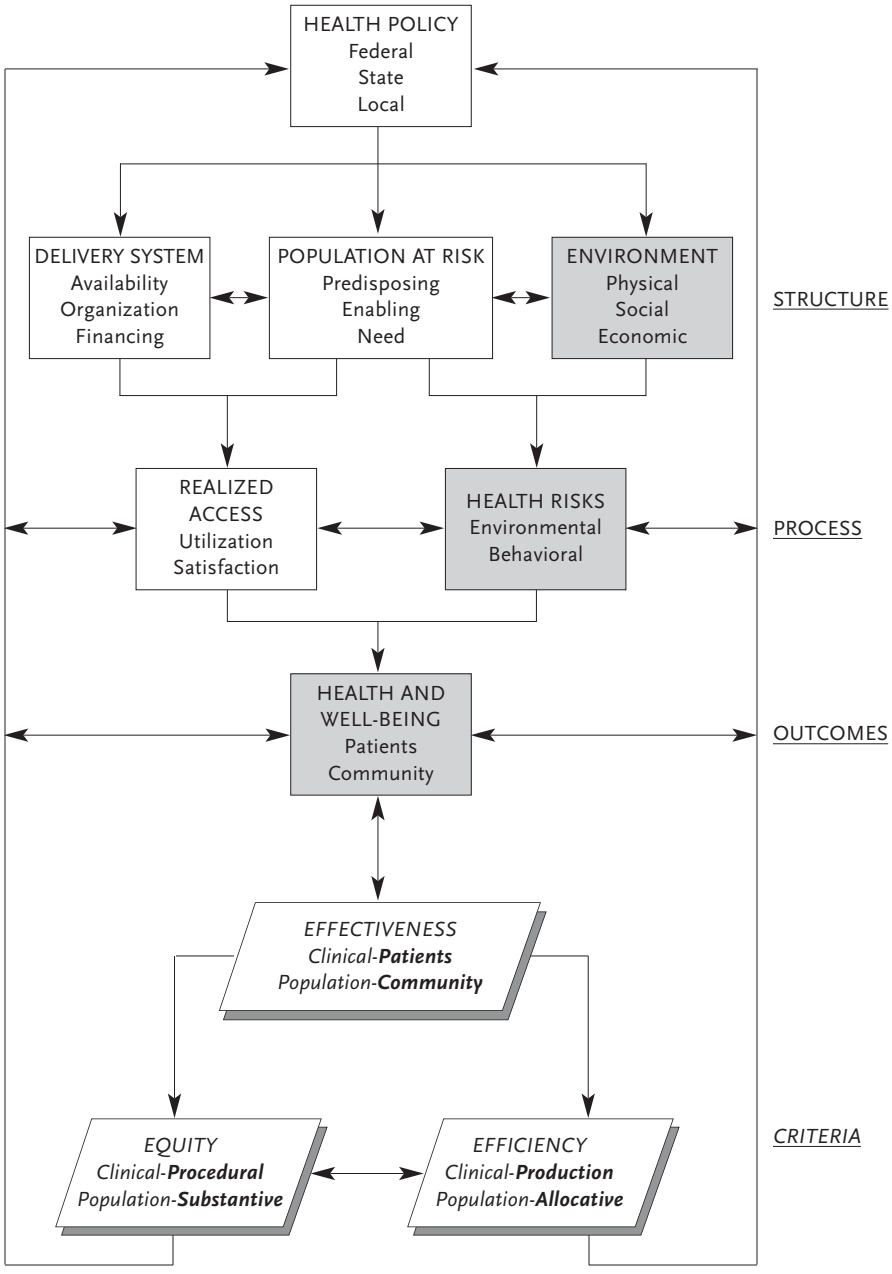
The design and conduct of health services research is often motivated by questions related to the formulation or evaluation of health policy. The access, cost, and quality dilemmas faced by governmental and private policymakers and institutions at the national, state, and local levels in providing and paying for healthcare serve as invitations to investigators to contribute to the knowledge base needed to make informed policy decisions. There is an increasing impetus as well, grounded in research on the fundamental social, economic, and environmental determinants of health, to increase investments in nonmedical programs and policies—for example, education, employment, community development—to ultimately improve population health and reduce health disparities.

The concepts and methods of health services research provide guidance for formulating and evaluating health policy by describing, analyzing, and evaluating the structure, process, and outcomes of the healthcare system associated with different policy alternatives.

Structure refers to the availability, organization, and financing of healthcare programs; the characteristics of the populations to be served by them; and the physical, social, and economic environments to which they are exposed. *Process* encompasses the transactions between patients and providers in the course of actual care delivery, as well as the environmental and behavioral transactions exacerbating health risks. The consequences of policies for the health and well-being of patients and the public may be viewed as the defining *outcomes* of health and health-related policies.

Health services research provides basic descriptive data on the organization and operation of the healthcare system, such as the number and

Figure 1.4 Framework for Applying Health Services Research in Evaluating Health Policy



distribution of providers, the percentage of population uninsured, and the rates of service utilization. It also analyzes likely relationships between and among components (reflected in the arrows in Figure 1.4), examining the impact of health policy on the delivery system; on the individuals and populations affected by these initiatives; and, ultimately and most importantly, on the health of the population the delivery system was intended to serve.

The shaded boxes in Figure 1.4 represent revisions or additions to the framework introduced in the first edition of this book (Aday et al. 1993), influenced by a conceptual framework focusing on the social and individual determinants of health developed by Evans, Barer, and Marmor (1994) and extended by Roos et al. (1996). The revised framework acknowledges the important role that physical, social, and economic environments—and their associated health risks—play in producing health.

The structure, process, and outcomes of healthcare can be studied at the macro level or micro level of analysis. The macro level refers to the population perspective on the determinants of the health of communities as a whole, and the micro level represents a clinical perspective on the factors that contribute to the health of patients at the system, institution, or individual level. Health may be measured using indicators developed for measuring progress toward health goals that have been set for identified populations or patients, such as the Healthy People 2010 objectives for the U.S. population or Health Plan Employer Data Information System benchmarks for patients in participating plans.

Effectiveness, efficiency, and equity may be viewed as specific criteria for evaluating policies and practices at both the clinical and population levels with respect to whether they actually result in significant health improvements (effectiveness), are the best use of scarce resources (efficiency), and distribute benefits and costs fairly across groups (equity). Table 1.1 summarizes the definitions of effectiveness, efficiency, and equity at both the clinical and population levels. The focus at the clinical (or micro) level is on *healthcare services delivery* and at the population (or macro) level on *population health improvements*.

At the micro level, clinical effectiveness addresses the impact of medical care on health improvements for individual patients, production efficiency is concerned with the combination of inputs required to produce these services at the lowest costs given that resources are limited, and procedural equity assesses the fairness of healthcare services delivery.

At the macro level, population effectiveness addresses the role of medical and nonmedical factors in influencing the health of populations as a whole, allocative efficiency analysis attempts to identify the mix of services that produce the greatest health improvements relative to the costs of producing them given resource constraints, and substantive equity is judged ultimately by the extent to which those health benefits are shared equally across groups in the community.

Effectiveness—or the production of health benefits—is placed before efficiency and equity in the framework (Figure 1.4) to indicate the central role it plays in assessing the cost of producing health benefits (i.e., efficiency) as well as the distribution of these benefits and costs across groups (i.e., equity). The double-headed arrow between HEALTH and EFFECTIVENESS indicates that patient and community health outcomes are direct inputs into evaluating effectiveness, and that effectiveness (at both the clinical and population levels) ultimately influences the health of patients and the community. Evidence on the effectiveness of clinical- or population-level interventions is needed to make informed judgments regarding the efficient allocation of scarce resources, as well as the types of services to which equitable access should be assured.

This framework has been adapted and applied in a variety of policy and program contexts, including the evaluation of the availability of community child health services; the health and healthcare needs of homeless populations; and the effectiveness, efficiency, and equity of behavioral healthcare services, among others (Aday and Awe 1997; Aday et al. 1999; Andersen 1995; DuPlessis, Inkelas, and Halfon 1998; Gelberg, Andersen, and Leake 2000; Halfon and Hochstein 2002; IOM 1993, 2002a).

As reflected in Table 1.1, the effectiveness, efficiency, and equity criteria may lead to differing conclusions regarding the best policy option based on these different criteria. Analyses of competing health policy alternatives would then optimally measure and evaluate each of these criteria and the trade-offs resulting when some are emphasized to the exclusion of others.

Macro Level Versus Micro Level of Analysis Based on the Framework

Kerr White and his colleagues identified a number of denominators for characterizing the distribution of demand for medical care in their foresightful 1961 article, "The Ecology of Medical Care" (White 1997;

Table 1.1 Definitions of Effectiveness, Efficiency, and Equity Criteria

	Level of Analysis	
Criteria	*Micro: Clinical*	*Macro: Population*
Effectiveness	*Clinical effectiveness:* Improving the health of individual patients through medical care services	*Population effectiveness:* Improving the health of populations and communities through medical and/or nonmedical services
Efficiency	*Production efficiency:* Combining inputs to produce services at the lowest cost	*Allocative efficiency:* Combining inputs to produce maximum health improvements given available resources
Equity	*Procedural equity:* Maximizing the fairness in the distribution of services across groups	*Substantive equity:* Minimizing the disparities in the distribution of health across groups

White, Williams, and Greenberg 1961). These successive denominators included general or geographically defined populations, populations of sick persons, those consulting physicians, those admitted to community hospitals, those referred to other physicians, and those referred to university medical centers.

These respective denominators can be broadly adapted and applied in characterizing the impacts of health policy at either a macro level or micro level of analysis (Table 1.2). The community level encompasses the population in a defined area and the physical, social, and economic environment in which they reside. *System level* refers to the healthcare system, including resources such as money, people, physical infrastructure, and technology and "the organizations and systems or networks of organizations that transform these resources into health services and distribute them to consumers" either within a specific region or

for the country as a whole (Longest 2002, 54). *Institution level* refers to a specific organizational entity such as a hospital, clinic, or health maintenance organization. *Patient level* refers to the microcosm of clinical decision making and treatment.

Information from each of these levels is required to fully understand and interpret the effects of health policies and programs. Commitments to developing medical technologies or procedures to optimize individual patient outcomes may fail to consider whether, in the light of limited resources, these are the best investments to enhance the health and well-being of the population as a whole. Treatments that have been demonstrated to be efficacious at the individual patient level may not be applied similarly across institutions, or even within the same institution. System-level outcomes may be influenced by organizational and financial incentives that influence the patterns of healthcare provision. Community-level outcome studies allow exploration of the variations in care that may result from differential access to healthcare and from different styles of practice not detectable by outcomes research at the institutional or system levels alone. A focus on the role that personal lifestyle practices (e.g., smoking) and attitudes (e.g., toward regular physical activity) play in affecting individuals' health status may not fully reveal the array of social structural and environmental factors (e.g., poverty, lead paint, toxic wastes) that may have consequential impacts on the health of populations residing in an area.

The discussion in the chapters that follow offers additional insights on the performance of the healthcare system that may be uniquely illuminated, as well as omitted, by a singular focus on any one point of view.

International Implications of the Framework

The following discussion highlights the issues in applying the framework (Figure 1.4) and related effectiveness, efficiency, and equity criteria in evaluating health programs and policies in the international context.

Effectiveness

Effectiveness focuses on the benefits produced by healthcare, as measured by improvements in people's health. However, these benefits and the related organization and delivery of care could vary considerably across regions and countries. Factors that influence this variation may

Table 1.2 Levels of Analysis in Health Services Research

	Level of Analysis[1]				
	Community				
Data Sources	*Environment*	*Population*	*System*	*Institution*	*Patient*
Census	X	X			
Public health surveillance systems	X	X			
Vital statistics		X			
Area resource files			X		
Market-area inventories			X		
Surveys					
Population		X			
Organizations			X	X	
Providers			X	X	
Patients			X	X	X
Records					
Enrollment		X	X	X	X
Encounters			X	X	X
Claims			X	X	X
Medical records			X	X	X
Qualitative studies					
(Non)participant observation	X	X	X	X	X
Case studies			X	X	
Focus groups		X			X
Ethnographic interviews		X			X

[1] The denominator for population-level analyses is individuals residing in a designated geographic area. The denominator for patient-level analyses is individuals who have utilized healthcare services. Data collected at one level can be aggregated to other levels of analysis in which these units are nested (e.g., patient-level data can be aggregated to the institution or system level). Patient-level data, and estimates based on aggregating them to the system or institution level, may be used as the numerator, but not the denominator, for population-level analyses.

include socioeconomics, human development, and governmental regulations and policies. Applying the example of breast cancer screening to be utilized throughout the book, a series of articles by the International Breast Cancer Screening Network (IBSN) chronicles the variations in structure, process, and outcomes of breast cancer screening programs across 23 different countries, all of which are developed nations (Ballard-Barbash et al. 1999; Klabunde et al. 2001a, 2001b). This group of studies documents that the effectiveness of these programs is shaped in part by the approach to screening delivery within a country.

In many countries there is strong organization of screening mammography without strict regulation of quality, whereas in the United States, screening occurs largely outside of an organized context but with legally based and federally imposed quality requirements. Most IBSN-participating countries have established mechanisms for ensuring the quality and effectiveness of screening mammography in population-based programs, although these mechanisms vary across countries.

Little evidence about the adoption and effectiveness of such programs in developing nations exists. The increasing incidence of breast cancer and the late stage at diagnosis of most disease in developing countries supports efforts to encourage introduction of breast cancer screening programs. However, the considerable economic burden imposed by mammography in terms of technology and skilled personnel make it difficult to apply in most developing countries at present, prompting researchers to suggest promotion of clinical breast exams as a more cost-effective alternative in these circumstances (Albert and Schulz 2003).

Therefore, while the clinical perspective of effectiveness gains more importance in developed countries (where the emphasis is on improving the monitoring of process and outcomes indicators for selected clinical conditions), the need for a population perspective of effectiveness (where issues such as health-needs assessment and provision of the continuum of healthcare services are foremost) is highlighted in developing countries.

Efficiency

Although most acute in the United States, efficiency concerns are universal, from the wealthiest to the poorest nations. Developed countries appear most concerned with macro cost control, whereas developing

nations strive to allocate to areas that will achieve the greatest health benefit from their extremely limited resources (European Observatory on Health Care Systems 2002). All systems could benefit from more efficient methods of producing and delivering healthcare services. Even highly market-minimized systems in Sweden and the United Kingdom have integrated aspects of market competition. These countries have been relatively successful in controlling health spending as a proportion of gross domestic product (GDP). Their focus is, therefore, more on making their systems more responsive to consumers, but they are very cautious about the threat of market strategies to the equity of their systems. Market-maximized countries such as the United States have been less successful in achieving cost control, and they are pursuing competitive efficiency strategies to control spending as well as to rationalize the allocation of resources.

There is concern that these market-driven policies will fail to control cost and that equity will be further undermined by these policies (Cutler 2002; Reinhardt 1997). Especially in the poorest countries, the public sector may be so limited that private health sector development may be the only way to develop the capital necessary for public health sector improvement. Thus, some knowledge of the strengths and limitations of markets and market competition in healthcare may inform policies in a wide range of countries.

The limitations of healthcare markets in achieving both efficiency and equity provide an opportunity for government entities to make improvements in the allocation of healthcare resources and production of health services. Without competitive market price signals, alternative methods and information are needed to make efficient resource allocation decisions. The techniques of economic evaluation and cost-effectiveness, cost-benefit, and cost-utility analysis (described in Chapter 4 and illustrated in Chapter 9) can help guide public and private decision makers.

While these techniques can require extensive information on costs, health consequences, and how people value resources and health outcomes, they can be applied with the best available information to even the least developed countries (Marseille, Kahn, and Saba 1998). Using the economic evaluation frameworks to think through resource allocation issues, strategies can be identified that are likely to be more efficient and highlight the areas of uncertainty and where information is needed for a more complete assessment.

Equity

Concerns with the equity of health and healthcare serve as important drivers for the formulation and evaluation of health policies and programs in many countries. Equity of healthcare is essentially concerned with the operation and performance of the healthcare system and focuses on maximizing fairness in the distribution of services (*procedural equity*). Equity of health encompasses a look at the medical and nonmedical determinants of health and focuses on minimizing disparities in health across groups within a population (*substantive equity*). Both types of equity criteria are encompassed in the framework presented in this chapter (Figure 1.4) and are applied specifically to assessing health policies in terms of equity in Chapters 6 and 7.

Although both equity of healthcare and equity of health are universal goals of healthcare systems, their relative emphasis in designing and evaluating systems and policies differs across countries. In developed countries with large and complex healthcare systems, the bulk of the expenditures and the focus in terms of evaluating equity are often in terms of the operation and performance of the system itself. A particular equity concern, for example, is the universality of insurance coverage. As documented in this and subsequent chapters, the lack of public or private third-party coverage can have a major impact on the rates of utilization of needed preventive and treatment services. The healthcare reform debates in the United States and other countries have typically centered on methods for ensuring more universal insurance coverage. Wide variations exist across countries in the availability and means of financing care. The heart of the debate regarding healthcare reform is often related to whether more market-maximized versus market-minimized methods for the financing and delivery of services would be most effective in achieving the equity of healthcare objective (Blendon et al. 2002; Hacker 1996; Skocpol 1996).

In developing countries, the type of prevalent health problems, such as environmentally related risks, infectious diseases, and maternal and child health needs, as well as the lack of public or private resources for supporting a complex healthcare infrastructure, makes equity-of-health considerations assume great importance. Correspondingly, fundamental public health and primary care investments are of central concern in developing countries. The World Health Organization (WHO) has, through a variety of national and international programs, attempted to better ensure "Health for All" and facilitated the development of

indicators and data systems for monitoring and evaluating progress toward this goal across countries. The WHO and U.S. Healthy People 2010 objectives, for example, represent efforts to assess the extent to which the equity of health goal has been achieved (Murray and Evans 2003). The discussion that follows points out the utility of the framework introduced here (Figure 1.4) for identifying and addressing issues in the operation and performance of healthcare systems across countries.

Policy Analysis

The World Health Organization has identified five common problems that policymakers in both developed and developing countries face in making choices to improve their health systems:

1. Confusion over the goals of health systems
2. Relatively weak and often conflicting evidence on strategies to improve health system performance
3. The lack of public or private institutions and individuals who are accountable for system outcomes
4. A societal focus on the development of new technologies with less attention on the delivery of technology
5. The increasingly technical nature of health system debates (Murray and Evans 2003, 3–5)

By fostering a common framework and set of measurement methods for health policy evaluation, the effectiveness, efficiency, and equity perspectives address many of these problems.

Policy debates in developed countries over the past two decades have remained focused on the short-run goal of cost containment, with dialog over market-based versus nonmarket-financing strategies to increase efficiency in service delivery (Begley et. al. 2002). The focus in developing countries has been on expanding public infrastructure, reducing waiting time, and introducing user fees (Murray and Evans 2003). Often lost in these debates is the connection of these intermediate goals to the ultimate goal of maintaining and improving population health. The effectiveness, efficiency, and equity perspectives offer a useful hierarchical framework for linking population health as an end goal to intermediate structure and process goals as the means for achieving it. (A hierarchical framework refers to the formal logic for identifying possible causes of a problem situation [Dunn 2003].) Thus, when applied

in policy debate, the framework can add clarity to goal identification by distinguishing intermediate goals and showing their linkage with the ultimate goal of improved health.

One reason for the relatively weak evidence of what works in the design or reform of health systems is the absence of a common framework for evaluating these systems. When studies use different definitions and measures of system structures, processes, outputs, and outcomes, it is difficult to build a consistent database. The efficiency, effectiveness, and equity perspectives provide a common framework and a similar set of measurement methods that may be used as a basis for developing an international database on what works in health systems performance over time.

The application of the combined perspectives of effectiveness, efficiency, and equity in policy evaluation requires policymakers to consider all health-related activities (healthcare, nonpersonal health services, and intersectoral programs such as water and sanitation programs) the primary purpose of which is to promote, restore, or maintain health. Thus, this framework offers a coherent set of tools that can help provide a broad and integrated context for policy accountability.

Finally, the specialized language and complex empirical methods used in health systems analysis often limits participation of nonexperts in national and international policy debates. The integrative framework reflected in the effectiveness, efficiency, and equity perspectives reflects a commitment to making health policy research and analysis accessible to a broad population of students and practitioners in a variety of policy contexts.

EVIDENCE AND TRENDS BASED ON THE HEALTH POLICY ANALYSIS FRAMEWORK

As indicated by the framework (Figure 1.4), health policy has been directed at a variety of factors that may ultimately determine the health of individuals and populations. The discussion that follows provides an overview of historical and current trends with respect to each of these dimensions and the role of health policy in influencing them.

Health Policy

The diversity and complexity of contemporary health policy has its roots in the evolution of the role played by different levels of government —federal, state, and local—as well as in the variant investments at these

respective levels in public health–oriented versus medical care–oriented programs and services (Lee and Benjamin 2002).

The U.S. Constitution provides a broad foundation for the evolution of federal involvement in health through the assignment of governmental powers to promote and provide for the general welfare and to regulate commerce. The constitutional basis for state health and healthcare policy is lodged in the so-called "police powers" that permit state and local governments to limit the actions of individuals to control and abate health nuisances or risks related to communicable diseases and environmental hazards from wastes, water, and food. This translated initially into public health–oriented interventions to prevent the importation of epidemics and to assist states and localities with their periodic needs for disease control. One of the earliest federal health initiatives, the Marine Hospital Service, was established in 1798 to serve merchant seamen and to prevent the spread of epidemic diseases; it grew over time into what is now the U.S. Public Health Service. Local health boards and departments in industrialized seaport cities developed public health policies to control communicable disease and improve sanitation. State health departments were formed throughout the late 1800s and early 1900s to carry out disease control activities and to run state mental institutions and state-owned university hospitals.

With the passage of the 16th Amendment to the U.S. Constitution in 1916, which authorized the federal tax on income, the federal government in the early twentieth century was in an enhanced position to assume a larger role in both the regulation and provision of healthcare. Through federal grants in aid to the states, major investments in broader public health–oriented and medical care–oriented programs and services evolved.

The bulk of federal resources came to be devoted to the expansion of coverage for medical care and, to a lesser extent, public health–oriented programs and services. The 1935 Social Security Act established a significant federal role in funding health programs with the creation of the social security "safety net" programs for the elderly, disabled, and families with dependent children. Federal grants to states were initiated in maternal and child health, public health, and healthcare for the aged and poor. The power and influence of the federal government grew rapidly from the 1930s to the 1960s with the support of biomedical research; a nationwide program of hospital planning and construction; direct federal aid to professional schools of medical education;

and programs to protect the public in food and drug safety, environmental protection, and occupational health and safety, among others. After 1965, the federal government's role was expanded further as a major purchaser of healthcare with the creation of the Medicaid and Medicare programs.

States, then, had served as the initial locus for programs and policies oriented to the health of the public until the federal government began to use its vast resource potential to meet changing public expectations after the Depression. State actions were soon driven by the conditions established for federal grant programs in public health and, eventually and centrally, for personal medical care services funding programs for vulnerable populations. These developments in categorical funding to address health problems have led to a patchwork of government-sponsored programs addressing specific diseases and populations. Health policy has become identified with the establishment, financing, and rule making for specific service programs at the federal level and the creation of administrative and service-delivery capacity to implement these programs at the state and local level. There has been a burgeoning commitment of public, particularly federal, resources to medical care provision and coverage and a significant, albeit substantially lesser, investment in categorical programs aimed at population or public health.

The 1980s and early 1990s featured broad-based efforts at health policy reform focused on controlling costs, increasing access and coverage, and improving healthcare performance. The comprehensive reform effort began in many states and provided the basis for the national reform proposal of the Clinton administration. With the demise of the Clinton plan in 1992–93, the comprehensive reform effort ended and more incremental strategies for reform gained center stage at both the state and national level (Lee and Benjamin 2002).

The tides of political change at the federal level in the latter part of the 1990s continued to shift more responsibility to the states for providing and paying for publicly supported medical care services—a shift that has catalyzed a corresponding reexamination of the importance and interface of public health and medical care service provision at both the federal and local levels. Furthering this reexamination has been the realization of the limited capacity of state and local public health infrastructure, originally highlighted by the IOM report of 1988

but receiving much greater attention since the terrorist attacks of September 11, 2001, in the United States.

A key assumption underlying the framework and the associated approaches to measuring effectiveness, efficiency, and equity presented here (see Figure 1.4) is the importance of highlighting improvements in the health of individuals and communities as the essential and desired endpoint of health policy. The framework also assigns a greater importance to the "health" descriptor in "health policy" and to nonmedical as well as medical factors in producing this valued policy outcome.

Health and Health Risks

The Healthy People 2010 process, guided by the Office of Disease Prevention and Health Promotion, U.S. Department of Health and Human Services, established specific objectives designed to achieve the following two overarching health policy goals:

1. Increase quality and years of healthy life for all Americans.
2. Eliminate health disparities in the United States.

To help meet these goals, nearly 28 separate priority areas were identified, 10 categories of leading health indicators delineated, and quantifiable targets set for improvements in health status, risk reduction, and service delivery. These objectives encompassed combating chronic disease, improving preventive services and the quality of medical care, and enhancing health education and communication. The 2010 Healthy People agenda provides a specific set of targeted goals for individuals, organizations, and communities for promoting health and preventing disease (NCHS 2003a; Office of Disease Prevention and Health Promotion 2003).

The Healthy People 2010 goals and objectives are used in this section to document the health of the community and associated environmental and behavioral health risks outlined in our framework (Figure 1.4). The health of the community is examined in terms of the objectives deigned to improve access and the quality of medical care delivered to communities. Environmental health risks are addressed by a series of health protection objectives, and behavioral risks are addressed by the preventive health objectives. As a means to benchmark progress in achieving the objectives of Healthy People 2010, evidence for 22 health indicators covered under the 10 broad categories of health indicators

will be reviewed. A similar comparison is made using breast and cervical cancer as health indicators (see Appendix 1.1).

Quality and Years of Healthy Life for All Americans

When indicators of access to healthcare are examined, 16 percent of persons under the age of 65 had no health insurance in the United States. Eighty-eight percent of the U.S. population had a specific source of ongoing primary care, and 17 percent of pregnant women received no prenatal care in the first trimester. These estimates fall short of the 2010 Healthy People objectives, which aim to provide healthcare insurance to every individual as well as increase the rates of having a specific source of ongoing primary care and receiving prenatal care in the first trimester of pregnancy to 96 percent and 90 percent, respectively.

Examining an indicator of environmental quality, we find that nearly 41 percent of the U.S. population was exposed to ozone above the Environmental Protection Agency (EPA) standards, far short of the 2010 target that no U.S. resident be exposed to ozone above the EPA standards. When indicators of responsible sexual behavior are examined, we find that only 23 percent of sexually active unmarried women age 18 to 44 years reported condom use by partners. This is substantially lower than the target of 50 percent for the year 2010. In contrast, 86 percent of adolescents in grades 9 through 12 were either not sexually active or were sexually active and used condoms in the year 2001, a figure closer to the 2010 planned target of 95 percent.

Examination of indicators related to immunization follows a similar pattern. While the percentage of children who received all necessary immunizations in 2001 is close to the estimated target rates for 2010 (74 percent versus 80 percent), substantial progress needs to be made in improving pneumococcal vaccination rates in noninstitutionalized adults age 65 years and older (54 percent in 2001 versus the target goal of 90 percent for 2010). A substantial burden due to injury and violence still exists in the United States, as evidenced by a death rate of 6.1 and 14.9 per 100,000 standard population for homicides and vehicular accident injuries in 2001, respectively. Healthy People 2010 goals are to reduce these figures to 3.0 and 9.2 per 100,000 standard population, respectively.

Mental health access is an area that remains sorely neglected, with only 23 percent of adults age 18 years and older with recognized depres-

sion receiving treatment. The goal is to increase treatment of recognized depression to cover 50 percent of the population that needs it by the year 2010. The nation is facing an alarmingly growing obesity epidemic, with nearly 15 percent of children and adolescents and 31 percent of adults who were either overweight or obese. Goals set by Healthy People 2010 expect to decrease these rates to 5 percent and 15 percent, respectively. Only 65 percent of adolescents and 32 percent of adults engaged in moderate or vigorous physical activity in the year 2001, much below the target rates of 85 percent and 50 percent set by Healthy People 2010. Nearly 21 percent of adolescents and 6 percent of adults reported recent illicit drug use. The goals here are to reduce those rates to 11 percent and 2 percent, respectively, by the year 2010. Finally, tobacco use remains rampant in spite of the widespread public health campaign against its use. Healthy People 2010 targets a 50 percent reduction in the tobacco use rates of 28 percent and 23 percent by adolescents and adults in the United States, respectively.

When indicators for breast and cervical cancer are examined, a similar pattern is observed. Some areas have seen current delivery rates fall well below target rates for Healthy People 2010. For example, only 37 percent of primary care providers counseled patients about mammograms, much below the target rate of 85 percent. Only 55 percent of primary care providers counseled patients about Pap tests, again less than the Healthy People 2010 target rate of 85 percent. In some cases, good progress toward Healthy People 2010 target rates has been made. For example, 70 percent of women age 40 years and older reported receiving a mammogram within the preceding two years in 2000 (Healthy People 2010 target rate: 70 percent), and 81 percent of women age 18 years and older received a Pap test within the preceding three years in 2000 (Healthy People 2010 target rate: 90 percent). Death rates due to breast cancer (2001 estimate: 26.0 per 100,000) and cervical cancer (2001 estimate: 2.7 per 100,000) must decrease by 14 percent and 26 percent, respectively, by the year 2010 if the goals of Healthy People 2010 are to be attained.

Health Disparities in the United States

Disparities across a variety of conditions and services exist for cross-sections of the U.S. population, including by race, gender, and age subgroups. Some of these disparities were highlighted in the previous

section, including extremely low rates of treatment for patients with diagnosed depression (23 percent) and low rates of pneumococcal vaccination for non-institutionalized older adults (54 percent), a sharp contrast with vaccination rates for children (74 percent). The major indicator of health disparities in the United States, however, continues to be race. For example, while 88 percent of whites and 81 percent of blacks or African Americans had health insurance in the year 2001, only 65 percent of Hispanics or Latinos had health insurance. Similarly, while only 36 percent of whites were exposed to ozone levels above the EPA standard, nearly 61 percent of the Hispanics or Latinos were exposed to these levels. While 58 percent of white, older, noninstitutionalized adults received the pneumococcal vaccine, only around one-third of the black or Hispanic, older, noninstitutionalized adults received the vaccine. Similarly, the death rate per 100,000 due to homicide was only 2.9 in the white population, compared to 20.9 and 7.4 per 100,000 in the black and Hispanic populations, respectively.

Childhood obesity rates for the black and Hispanic populations (22 percent and 24 percent, respectively) are also nearly double the rates in the white population (12 percent). However, several indicators are comparable across racial categories. These include responsible sexual behavior among adolescents and vehicular injury-related deaths.

We also find racial disparities in some indicators for breast and cervical cancer screening. Rates of death due to breast and cervical cancer are much higher for blacks compared to other races. The death rate per 100,000 was 35.0 in blacks compared to 26.0 and 16.2 in whites and Hispanics, respectively, for breast cancer and 4.9 in blacks compared to 2.3 and 3.4 in whites and Hispanics, respectively, for cervical cancer. While around 30 percent of the breast cancers in whites were diagnosed at a late stage, the rates were much higher for blacks and Hispanics (around 38 percent for both populations). While 72 percent of white women age 40 years and older had received a mammogram in the preceding two years, the rates were lower for black and Hispanic women (68 percent and 62 percent, respectively). The percentage of women 18 years and older who had ever had a Pap test or had one in the preceding three years was lower for Hispanic or Latino women compared to white or black women. Around half of black women were diagnosed with late-stage cervical cancer compared to around 42 to 43 percent of white and Hispanic women.

In summary, although progress has been made toward achieving a number of the health goals for the nation, most have not yet been accomplished. The discussion that follows describes the dimensions and trends of health disparities with respect to the U.S. healthcare delivery system, and subsequent chapters introduce approaches for evaluating system performance with respect to effectiveness, efficiency, and equity criteria and, ultimately, in contributing to improvements in the health of the U.S. population and minimizing disparities between groups.

Population at Risk

The population at risk may be characterized in terms of predisposing (e.g., demographics, attitudes), enabling (e.g., personal and family resources), and need (e.g., perceived and evaluated health status) characteristics. Healthcare access and health status differ according to characteristics such as race, gender, and socioeconomic status, and the differences are often substantial (see NCHS 2003b and Appendix 1.1).

The challenges presented in addressing these differences are related to (1) understanding and defining health and healthcare "disparities" and (2) determining the macro- and micro-level pathways through which these differences emerge.

Differences or variations in healthcare use or outcomes between groups are not all necessarily judged to be disparities (Carter-Pokras and Baquet 2002). The IOM (2003a, 3–4) report *Unequal Treatment: Confronting Racial and Ethnic Disparities in Health Care*, for example, defines "disparities in healthcare as racial or ethnic differences in the quality of healthcare that are not due to access-related factors or clinical needs, preferences, and appropriateness of intervention."

Unequal Treatment focuses on two levels in accounting for these disparities: (1) operation of healthcare systems and the legal and regulatory environment in which they function and (2) discrimination at the individual, patient-provider level. Disparities, in contrast to differences or variations, imply normative judgments regarding the equity or fairness of these differences. Chapters 6 and 7 explore criteria that could be implied in making these judgments.

A related extension and application of research on the social determinants of health is to identify the individual and societal pathways through which social factors influence health and well-being. This research focuses on two principal lines of inquiry: (1) the development

and application of ecosocial theories of disease distribution and (2) multilevel and contextual analyses of the social determinants of health. Nancy Krieger (2002, 9) has, for example, defined the concept of "embodiment" as "how we literally incorporate, biologically, the material and social world in which we live, from utero to death." Related ecosocial theory–driven research attempts to empirically specify the pathways of exposure, susceptibility, and resistance to illness leading to the embodiment of socially defined risks and opportunities associated with gender, race, and/or socioeconomic status, for example. Fruitful methodological developments that have facilitated and extended this line of research include the application of multilevel statistical modeling of different levels of impact (individual, neighborhood, regional or political jurisdiction, etc.) (Diez-Roux 1998) and the use of community and participatory action research models to identify and implement programs that affected (in particular, socially disadvantaged) populations deem most relevant to their needs (Chambers 1997; Minkler and Wallerstein 2002).

In summary, health and healthcare disparities between groups remain substantial and show little evidence of narrowing. In addition, environmental and behavioral risks persist, and the attendant health impacts for some groups in particular are significant.

Environment

The physical, social, and economic environments in which individuals live and work significantly influence exposures to health-related risk factors. The physical environment directly affects the prevalence and distribution of health risks resulting from exposures to toxic hazards transmitted through the soil, water, and air (Goldfarb 2001; McKinney and Schoch 2003). Such risks, for example, have been variously blamed for the prevalence of childhood lead-paint poisoning; rising rates of childhood asthma, particularly among minority children; the high incidence of birth defects among residents along the U.S.-Mexican border; and excessive cancer prevalence and death rates along the Mississippi River valley (known as "cancer alley"), in which there is a high concentration of pollution-producing industries. Further, research on environmental justice has documented that such risks, and associated adverse health consequences, are disproportionately inflicted on low socioeconomic and minority neighborhoods because high-risk industries or toxic waste sites are often found in such areas (Brown 1995).

A large body of public health and health services research has substantiated the importance of socioeconomic factors in influencing the differential distribution of health and health risks (Aday 2001; Adler et al. 1994; Evans, Barer, and Marmor 1994; Feinstein 1993; Moss 2000; Subramanian, Belli, and Kawachi 2002; Syme and Berkman 1976). Evans, Barer, and Marmor (1994) provide a compelling synthesis and argument regarding the impact of social and economic hierarchies (e.g., occupation, education, income, race, and gender), and of individuals' location within them, on health. Research based on animal models, as well as human populations, has consistently documented the poorer health status of those at lower, compared to higher, positions in such hierarchies. The dynamic that appears to be operating is that individuals in the lower ranks are more likely to be exposed to greater risks and associated stresses in their social and economic environment that can lead to both physiological and behavioral responses (e.g., biochemical changes and adoption of high-risk, addictive practices). Ultimately, these responses give rise to health disparities by social position. Whiteis (1997, 2000) and others (Abraham 1993; Cohen et al. 2003; Wilson 1980, 1987, 1989) have convincingly documented the role of public and corporate disinvestment in the poor and minority neighborhoods of large urban centers and the pervasive effect on the economic, social, and physical health and well-being of the people residing within them.

Detailed evidence on the impact of environmental risk factors, and particularly on the disproportionate distribution of health outcomes as a consequence, is reviewed in Chapter 7.

Delivery System

The discussion that follows highlights the availability, organization, and financing of the U.S. healthcare system, focusing in particular on the major changes that have taken place over the past three decades. Much of the information is based on an annual publication of the National Center for Health Statistics, *Health: United States* (NCHS 2003b).

Availability

In the 1960s, a worsening physician shortage was perceived to exist in the United States. In response, federal and state governments greatly expanded investment in medical schools, which resulted in a corresponding increase in the number of medical graduates. These trends,

along with the growth in managed care organizations, raised subsequent concerns in the 1980s and 1990s about a burgeoning physician surplus (NCHS 1997, 239; NCHS 2003b, 294–95; Politzer et al. 1996; Reinhardt 1991; Weiner 1994).

Contrary to these concerns, later reports suggest that there was no surplus of physicians in the United States (Salsberg and Forte 2002). The medical market has continued to absorb the growing number of physicians, both primary and specialty care. The aging population, increasing complexity and intensity of treatment, physicians' work effort, and backlash against managed care led to demand keeping pace with the increasing supply of physicians in the 1990s. The policy to increase the number of primary care physicians in the 1990s may well result in subsequent shortages in several medical specialties such as geriatrics (Cooper et al. 2002).

A critical shortage of hospital nurses and nursing school faculty exists in many areas of the country. Rural areas in particular suffer from a shortage of providers in general and dentists in particular (Escarce et al. 2000; Knapp and Hardwick 2000; Seago et al. 2001).

Organization

Managed care systems. Managed care encompasses various forms of health maintenance organizations (HMOs), point-of-service plans (POSs), and preferred provider organizations (PPOs). HMOs are organizations that guarantee delivery of a comprehensive prepaid benefit package to a voluntarily enrolled population through an organized system of care. POSs represent HMOs that offer partial reimbursement for services that an enrollee chooses to obtain outside of the HMO network. PPOs contract to provide services at a discounted rate under conditions of utilization review that offer providers a wider network of enrolled populations, and enrolled populations a wider choice of providers, while restricting the scope or increasing the out-of-pocket costs of the benefits provided (AMA 1996; Reinhardt 1996).

HMO plans and enrollment have grown since the early 1970s. HMOs have also become vigorous competitors of traditional health insurance plans in several metropolitan areas, enrolling about 26.4 percent of the U.S. population (or 76.1 million persons) in 2002 (NCHS 2003b, 339). Trends also include growth in for-profit managed care plans, such as PPOs and nontraditional HMOs that allow enrollees to select a non-HMO

provider in exchange for a financial penalty. In 2001, 93 percent of Americans who received health insurance through an employer were enrolled in managed care compared to 73 percent in 1995 (Gabel et al. 2001). The most rapid growth in the 1990s was in enrollment in PPOs and POSs. Their combined enrollment of covered workers increased from 42 percent in 1996 to 70 percent in 2001. HMO enrollment declined from 31 percent to 23 percent during this same period, representing the shift to a less restrictive form of managed care.

As growth in the commercial market slowed in the early to mid-1990s, managed care plans began to compete vigorously to enroll public beneficiaries. Medicare enrollment in managed care plans was 18 percent of beneficiaries in 1999. Strong growth was projected to continue, reaching one-third of beneficiaries by 2007 (Lamphere et al. 1997), but managed care enrollment share had declined to 15 percent of the Medicare population by 2002 (MedPAC 2003). Managed care growth has remained strong in the Medicaid program, representing more than half (23.1 million) of all Medicaid beneficiaries in 2002 (CMS 2003).

Physician organizations. Of the 668,939 physicians in patient care in 2001, 76.8 percent were in office-based practice, 14 percent were in training, and 9.2 percent were full-time hospital staff. Almost half of physicians in office-based practice were in primary care specialties (AMA 2003a).

Thirty-three percent of physicians in non-institutional settings were solo practitioners in 2001 compared to about 90 percent in 1965. The average number of physicians per medical practice was 20.4 with a range from 7.8 in obstetrics/gynecology to 41.5 in radiology. Nearly all medical practices had one or more managed care contracts, and around one-third had contracts with capitation payment (AMA 1996, 2003b).

Hospitals. The hospital industry has also undergone tremendous changes during the past 40 years. These include

- the rapid advancement in medical technology,
- an expansion in outpatient services,
- a growth in multihospital systems,
- the emergence of increased competition among hospitals and between hospitals and other providers,
- increasing mergers and conversion of community nonprofit hospitals to for-profit status, and

- a fundamental change in the Medicare payment system that supplies about half of the hospital revenue in the United States.

The shift, described in more detail later in this chapter, has been from a retrospective reimbursement system to a prospective payment system (PPS) based on diagnosis-related groups (DRGs) (NCHS 2003b, 299, 301–02).

The total number of community hospitals declined from 5,875 in 1975 to 4,908 in 2001, with total beds declining from 942,000 to 826,000 over the same time period (AHA 2003). The decline was accompanied by a shift toward investor-owned (for-profit) community hospitals and away from state and local government community hospitals. The former represented 13.2 percent of the community hospitals in 1975 compared to 15.4 percent of the hospitals in 2001. Not-for-profit community hospitals continue to represent the majority of hospitals and hospital beds, however (AHA 2003).

Another reaction to managed care and other cost-containment strategies has been the development of strategic alliances between hospitals. In the proprietary sector, large hospital corporations began purchasing hospitals in different markets and instituting centralized and standardized management practices to achieve greater efficiency and profits. Merger activity was especially strong in the mid-1990s with 235 deals affecting 768 hospitals. Mergers had declined to 142 in 1999 (Bellandi 2000). Not-for-profit hospitals also began affiliations with hospitals in their region of the country to establish referral patterns and share services and possibly to protect against the expansion of the proprietary chains (Luke, Begun, and Pointer 1989). This move to horizontal integration was followed by efforts to achieve vertical integration. Hospital systems and physician groups began forming organized systems of care (Shortell and Hull 1996). However, the trend toward vertical integration and tightly managed care failed to yield the expected efficiencies and was largely abandoned by hospitals, physician groups, and health plans across the nation (Lesser, Ginsburg, and Devers 2003; Robinson 2001).

Public health. The Centers for Medicare & Medicaid Services estimated that expenditures for public health activities by all levels of government in the United States were around 3 percent of total national health expenditures, or $46.4 billion, in 2001 (NCHS 2003b, 309–10).

A 1999–2000 survey of local public health agencies (LPHAs) conducted by the National Association of County and City Health Officials (NACCHO 2001) documented that the majority (60 percent) of local public health agencies were county based. The most common programs and services provided included adult and child immunizations, communicable disease control, community assessment, community outreach and education, environmental health services, epidemiology and food surveillance, food safety, health education, restaurant inspections, and tuberculosis testing.

The occupations LPHAs usually employed include public health nurses and environmental scientists, as well as administrative/clerical staff. The average LPHA staff size in full-time equivalents (FTEs) was 67, with a median of 13 FTEs. The median annual LPHA expenditure in constant 1999 dollars was $621,100. The largest proportion of LPHA budgets came from local sources (county or city), followed by state sources. Funding streams varied by metropolitan and nonmetropolitan area location and the size of the population served. Local public health officials consistently indicated that workforce and partnerships with their local communities were their agencies' greatest strengths, while funding was consistently mentioned as the biggest challenge.

Health departments face major additional challenges today. One is the financial vulnerability of their primary care clinics in this time of movement to managed care. Another is concern about the provision of preventive services as clients traditionally served by these public health clinics are moved into private sector medical care. The September 11, 2001, terrorist attacks in the United States subsequently placed even greater expectations and burdens on local and state health departments to develop expanded emergency response systems. A growing body of research on the social determinants of health has also served to broaden the public health mandate to develop intersectoral programs and policies to address the fundamental determinants of population health. These and other challenges are compelling health departments to reconsider their mission and the ways in which it can be accomplished.

A series of IOM reports have assayed the strengths and limitations of the U.S. public health system and suggest fruitful new directions for better achieving U.S. public health policy objectives (IOM 1988, 2002b, 2003b). The 1988 IOM report set out an assessment and vision for the future of public health in terms of the core public health functions of

assessment, policy development, and assurance and ten related essential public health services. The more recent IOM reports importantly argue for grounding innovations in the design and implementation of public health policies and programs in an ecological model of population health, based on research on the multifactorial determinants of health, and broader intersectoral collaboration to ultimately improve the health of populations and reduce persistent health disparities.

Financing

Payment Arrangements

Until the 1980s, physicians in the United States controlled their means of payment and the amount they could charge through fee-for-service reimbursement. This led to high physician incomes relative to the average full-time employee as well as to other professionals and to healthcare delivery practices that were both inefficient and inequitable. The fee-for-service system resulted in overpayments for procedural care at the expense of visits and consultations, physicians providing identical services yet receiving very different fees, and systems of charges and reimbursement that were both difficult to understand and complex to administer (Simon and Born 1996).

A new physician payment system under Medicare, the resource-based relative value scale, was developed in the early 1980s in response to these problems (Physician Payment Review Commission [PPRC] 1991). The relative value was the sum of physician work, practice expense, and malpractice costs adjusted for geographic cost differences and converted to dollars using a conversion factor. The attempt was to develop a physician payment system that would (1) rationalize fee-for-service payments under Medicare, (2) reduce the rate of growth in physician expenditures, (3) protect access to care for Medicare enrollees, and (4) support quality care (Epstein and Blumenthal 1993).

The implementation of Medicare's prospective payment system in 1984 was the cornerstone for a corresponding movement to contain hospital costs. Under PPS, hospitals are paid a prospectively determined amount per discharge, rather than on a retrospective reasonable-cost basis. Payment varies by DRG category and is updated annually to reflect changes in a hospital input price index (McClellan 1997).

Hospital payment was sharply affected by the growth in managed care and competition in the private sector in the 1990s. Resulting devel-

opments include hospitals increasingly engaging in cost cutting and mergers, forging closer relations with physicians and other providers, assuming of insurance functions, and contracting directly with employers. Driven mainly by an increase in outpatient services, spending on hospitals began increasing in 1997 and reached 12 percent growth in 2001, reclaiming the position of primary driver of healthcare spending growth (Strunk, Ginsburg, and Gabel 2002).

Expenditures and Costs

National healthcare expenditures for the complex and highly technological U.S. medical care enterprise were $1,424.5 billion in 2001 compared to $26.7 billion in 1960. For the same period, healthcare expenditures grew from $143 to $5,035 per capita and from 5.1 to 14.1 percent of the GDP (NCHS 2003b, 306).

While all national healthcare expenditures have grown, the 40-year shifts in the distribution of spending for services were mainly toward nursing home and home care. Hospitals still represent the largest sector, followed by expenditures for physician services. Although the absolute levels of expenditures increased, the relative share for drugs declined but increased again during the 1990s (NCHS 2003b, 310).

The growth in personal healthcare expenditures (i.e., in spending for the direct provision of care) increased sharply after the passage of Medicare and Medicaid in 1965 and continued a strong upward trend in the 1970s, a period of high general inflation. Growth declined initially in the 1980s in response to cost-containment measures and the decline in general inflation. However, average annual cost increases continued between 9 to 10 percent during the late 1980s and early 1990s. Growth in personal healthcare expenditures slowed in the mid-1990s but began to climb again in early 2000 (Levit, Lazenby, and Sivarajan 1996, 132; NCHS 2003b, 308). The major factors affecting growth in personal health expenditures have been economywide inflation, medical price inflation in excess of general inflation, and the increased use and intensity of services per capita (Heffler et al. 2003).

Government and private insurers have increased their roles in financing healthcare services in the United States. Government programs covered 45 percent of the cost in 2001, almost double the proportion covered in 1960 (Levit et al. 1991, 50; Levit, Lazenby, and Sivarajan 1996, 141; Levit et al. 2003, 162). Around 17 percent of personal health expenditures

were paid for out-of-pocket in 2001, compared to 55 percent in 1960. Private insurance, primarily including Blue Cross and Blue Shield plans, employer self-insurance, independent plans, and commercial insurance companies, covered 35 percent of the cost in 2001, compared to 21 percent in 1960. Despite the growth in government and private insurance, there were 43.6 million uninsured persons in 2002, and an equal or greater number without adequate insurance coverage (NCHS 2003b, 11; U.S. Census Bureau 2003). (Additional evidence on the uninsured will be presented in Chapter 7.)

Realized Access

Health services research has documented substantial variations by geography in the levels of medical care resources, in the rates of administering various medical diagnostic procedures, and in the rates of performing surgical operations. These variations have, however, not been correspondingly associated with variations in health outcomes. The discussion that follows focuses on this variations evidence. Descriptive information on widely used indicators of the utilization of and satisfaction with healthcare will be highlighted in Chapter 7.

Glover (1938) is credited with first reporting the phenomenon of variations in the rate of surgical procedures, specifically for tonsillectomy rates in England. Since then, a host of studies have reported findings of variations in rates for common surgical procedures within a state of the United States (Lewis 1969; Wennberg and Gittelsohn 1973), within a Canadian province (Roos 1984), within a country (McPherson et al. 1981; Wennberg, Bunker, and Barnes 1980), and between countries (Bunker 1970; McPherson et al. 1981, 1982; Vayda 1973; Wennberg, Bunker, and Barnes 1980). All of these studies have found that the rates for common surgical procedures being done can vary as much as five- and sixfold from one geographic area of a state to another and as much as two- and threefold between countries. In addition, the same has been found for the rates of various diagnostic and medical procedures within the United States (Chassin et al. 1986; Wennberg 1990). Wennberg (1990), using data from 16 university hospital or large community hospital market areas, found that the ratios of high to low varied from 2.0 for inguinal hernia repair to 3.6 for coronary artery bypass graft surgery and 19.4 for carotid endarterectomy.

Studies have also demonstrated variations in screening and treatment of breast cancer. For example, a survey study by Lucci et al.

(2001) found a nearly fivefold difference between surgical oncologists in the western and central United States with regards to adopting a newer surgical technique for breast cancer (sentinel lymph node dissection [SLND]), although there were no variations in the procedures used to perform the SLND. A study by Goel, Iron, and Williams (1997) documented substantial variations by region in mammography screening rates in Ontario, Canada. The authors attributed this variability to physician referral patterns, patient uptake, and access to mammography screening.

In summary, dramatic changes are under way in the U.S. healthcare system as managed care comes to increasingly dominate the provision of and payment for medical care services. Trends in healthcare expenditures suggest that these changes may offer some promising constraints on the continued increases in healthcare costs. Nonetheless, wide variations in the patterns of providing medical care prevail across regions and delivery settings.

The chapters that follow introduce the concepts and methods for operationalizing and applying the effectiveness, efficiency, and equity criteria. In the final chapter, the relationship of health services research and policy analysis is described and illustrated in evaluating breast cancer screening policy for older women in terms of these criteria.

NOTE

1. More generally, we are concerned about allocating resources among all possible goods and services to achieve maximum social welfare (or well-being).

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Appendix 1.1 Healthy People 2010 Leading Health Indicators

2010 Leading Health Indicators	*Baseline Year*	*2010 Target*	*2001 Value (baseline value)*			
			All Races	*White, Not Hispanic or Latino*	*Black or African American, Not Hispanic or Latino*	*Hispanic or Latino*
Access to healthcare						
% persons with health insurance, under 65 years of age, age adjusted (Obj. 01-01)	1997	100	84 (83)	88 (86)	81 (80)	65 (66)
% with a specific source of ongoing primary care (Obj.01-04a)	1998	96	88 (87)	90 (89)	88 (86)	77 (79)
% pregnant women who received prenatal care in the first trimester (Obj. 16-06a)	1998	90	83 (83)	89 (88)	74 (73)	76 (74)
Environmental quality						
% population exposed to ozone above EPA standard (Obj. 08-01a)	1997	0	41 (43)	36 (37)	45 (47)	61 (61)
% nonsmokers age 4 and over exposed to environmental tobacco smoke (age adjusted) (Obj. 27-10)[1]	1988–94	45	53.9 (88.1)	52.0 (87.8)	72.0 (93.9)	44.1[2] (83.4)[2]

Responsible sexual behavior						
% adolescents in grades 9–12 who are not sexually active or who are sexually active and used condoms (Obj. 25-11)	1999	95	86 (85)	87 (85)	85 (84)	84 (84)
% sexually active unmarried women age 18 to 44 years who reported condom use by partners (Obj. 13-06a)[3]	1995	50	23	24	22	17
Immunization						
% children age 19–35 months who received all DtaP, polio, MMR, Hib, and Hep B vaccines (Obj. 14-24a)	1998	80	74 (73)	75 (76)	67 (67)	74 (69)
% noninstitutionalized adults age 65 and older who received influenza vaccine in the past 12 months (age adjusted) (Obj 14-29a)	1998	90	63 (64)	65 (66)	48 (47)	52 (51)
% noninstitutionalized adults age 65 and older who ever received pneumococcal vaccine in the past 12 months (age adjusted) (Obj 14-29b)	1998	90	54 (46)	58 (50)	34 (26)	33 (23)
Injury and violence						
Death rate from motor vehicle traffic-related injuries per 100,000 standard population (age adjusted) (Obj. 15-15a)	1999	9.2	14.9 (14.7)	14.9 (14.8)	15.5 (15.9)	14.6 (13.9)

Appendix 1.1 Healthy People 2010 Leading Health Indicators *(continued)*

2010 Leading Health Indicators	*Baseline Year*	*2010 Target*	*2001 Value (baseline value)*			
			All Races	*White, Not Hispanic or Latino*	*Black or African American, Not Hispanic or Latino*	*Hispanic or Latino*
Death rate for homicide per 100,000 standard population (age adjusted) (Obj. 15-32)	1999	3.0	6.1 (6.0)	2.9 (2.9)	20.9 (20.7)	7.4 (7.6)
Mental health						
% adults age 18 years and over with recognized depression who received treatment (Obj. 18-09b)[4]	1997	50	23	24[5]	16[6]	20
Overweight and obesity						
% children and adolescents, age 6–19 years, who are at or above the sex- and age-specific 95th percentile of body mass index based on CDC growth charts (Obj. 19-03c)[1]	1988–94	5	15 (11)	12 (10)	22 (14)	24[2] (15)[2]
% adults age 20 and over with body mass index of 30kg/m^2 or more (age adjusted) (Obj. 19-02)[1]	1988–94	15	31 (23)	29 (22)	40 (30)	34[2] (29)[2]

Physical activity						
% adolescents in grades 9–12 who engaged in 20 minutes or more of vigorous activity 3 or more days per week (Obj. 22-07)	1999	85	65 (65)	67 (67)	60 (56)	61 (61)
% adults age 18 years and over who engaged in moderate or vigorous physical activity (age adjusted) (Obj. 22-02)	1997	50	32 (32)	35 (34)	25 (24)	21 (23)
Substance abuse						
% adolescents age 12–17 years who reported no use of alcohol or illicit drugs in the past 30 days (Obj. 26-10a)[7]	1998	89	79	77	82	79
% adults age 18 years and over who reported illicit drug use in the past 30 days (Obj. 26-10c)[7]	1998	2	5.8	5.7	8.0	5.5
% adults age 18 years and over who reported binge drinking in the past 30 days (Obj. 26-11c)[7]	1998	6	16.6	17.3	12.7	17.2
Tobacco use						
% adolescents in grades 9–12 who smoked cigarettes in the past month (Obj. 27-02b)	1999	16	28 (35)	32 (39)	15 (20)	27 (33)
% adults age 18 years and over who smoked more than 100 cigarettes in their lifetime and now report smoking on some days or every day (age adjusted) (Obj. 27-01a)	1998	12	23 (24)	24 (25)	22 (25)	16 (19)

Appendix 1.1 Healthy People 2010 Leading Health Indicators *(continued)*

2010 Leading Health Indicators	*Baseline Year*	*2010 Target*	*2001 Value (baseline value)*			
			All Races	*White, Not Hispanic or Latino*	*Black or African American, Not Hispanic or Latino*	*Hispanic or Latino*
Other 2010 Indicators: Breast and Cervical Cancer[8]						
Female breast cancer						
Female breast cancer deaths per 100,000 (age adjusted) (Obj. 03-03)	1999	22.3	26.0 (26.6)	26.0 (26.6)	35.0 (35.7)	16.2 (16.4)
% primary care providers who counsel about mammograms (Obj. 03-10f)[9]	1988	85	37	—	—	—
% women age 40 years and over who received a mammogram within the preceding 2 years (age adjusted) (Obj. 03-13)[10]	1998	70	70 (67)	72 (68)	68 (65)	62 (60)
Cervical cancer						
Cervical cancer deaths per 100,000 (age adjusted) (Obj. 03-04)	1999	2.0	2.7 (2.8)	2.3 (2.5)	4.9 (5.4)	3.4 (3.6)
% primary care providers who counsel about Pap tests (Obj. 03-10g)[9]	1988	85	55	—	—	—

% women age 18 years and over who ever received a Pap test (age adjusted) (Obj. 03-11a)[10]	1998	97	93 (92)	95 (94)	95 (94)	87 (85)
% women age 18 years and over who received a Pap test within the preceding 3 years (age adjusted) (Obj. 03-11b)[10]	1998	90	81 (79)	83 (80)	84 (83)	77 (74)
Other Indicators for Breast and Cervical Cancer[11]						
% female breast cancers diagnosed at late stage[10]		N/A	29.5	28.6	38.0	37.8
% cervical cancers diagnosed at late stage[10]		N/A	44.7	43.1	50.5	42.1

— Data are not available.
[1] 1999–2000 data.
[2] Mexican American.
[3] 1995 data.
[4] 1997 data.
[5] White, may include Hispanic or Latino.
[6] Black or African American, may include Hispanic or Latino.
[7] 1998 data.
[8] These indicators are from Healthy People 2010, but are not leading indicators. They are included here as indicators of access to; utilization of; and, potentially, quality of health services for women.
[9] 1988 data.
[10] 2000 data.
[11] These indicators are not from Healthy People 2010. Instead, they are included here as additional indicators of access to; utilization of; and, potentially, quality of health services for women.

Appendix 1.1 Healthy People 2010 Leading Health Indicators *(continued)*

Note: The first line of information for an indicator includes the year 2010 Healthy People target value for the indicator, the actual year 2001 data for the U.S. population (all races), followed by the year 2001 data for whites, blacks, and Hispanics. On the second line of information for an indicator is the baseline year for which data on the indicator are available, the baseline year data (in parentheses) for the U.S. population (all races), followed by the baseline year data (in parentheses) for the respective racial/ethnic groups. If no data are available subsequent to the baseline year, then only the baseline year data are reported. Footnotes are also provided to further document the year for which data are reported for a given indicator.
Obj. = Objective number for Healthy People 2010.

Sources:
Healthy People 2010 Indicators: NCHS (2003a).

Estimates of female breast cancers and cervical cancers diagnosed at late stage: computed from the National Cancer Institute SEER*Stat Databases, SEER 12 Regs Public-Use, Nov 2002 Sub for Expanded Races (1992–2000) (NCI 2003).

Chapter 2

Effectiveness: Concepts and Methods

CHAPTER HIGHLIGHTS

1. *Effectiveness* is the degree to which improvements in health now attainable are, in fact, attained. Effectiveness concerns the results achieved in the actual practice of healthcare with typical patients and providers, in contrast to *efficacy*, which is assessed by the benefits achieved under ideal conditions.
2. Effectiveness research reflects two seemingly competing, but in fact complementary, definitions of effectiveness. One represents a *population perspective*, and the other represents a *clinical perspective*. The four levels of measuring effectiveness include the community level, associated with the population perspective, and the system, institution, and patient levels, associated with the clinical perspective.
3. *Structure, process,* and *outcomes* are linked conceptually in a research paradigm that assumes structural elements of healthcare as having an influence on what is and is not done in the process as well as how well it is done; this process in turn influences the health outcomes people experience as a result of their encounters with the care delivery process.
4. Outcome measures fit into the general categories of mortality, morbidity, and health status, although specific indicators of each of these broad measures may be used at different levels.
5. Nonexperimental observational designs, in which investigators do not directly intervene but instead develop methods for describing events that occur naturally and their effect on study subjects, characterize much of effectiveness research. The majority of effectiveness

research studies rely on medical records and related sources such as claims data collected for billing purposes and hospital discharge abstract data collected principally for quality assurance purposes.

6. *Risk adjustment* of patient outcomes is necessary in effectiveness research to account for the differing risks patients bring to the clinical setting (e.g., based on patient demographics or conditions unrelated to their presenting illness). Most effectiveness research is conducted under nonexperimental conditions, and these varying risks and their potential for confounding evaluations of the performance of a given intervention must be adjusted for in the analysis.

OVERVIEW

The fundamental questions posed in this chapter are (1) What is effectiveness? and (2) How should the effectiveness of healthcare be assessed? Chapter 3 considers (3) To what extent has effectiveness been achieved? and (4) What policy strategies contribute to enhancing effectiveness?

A widely accepted definition of effectiveness is the degree to which improvements in health now attainable are, in fact, attained (Donabedian 2003, 6). Questions of effectiveness have assumed great importance in recent years because of the continually escalating costs of medical care; evidence of wide and unexplained variations in the rates of utilization of medical care across states and regions; community-level evidence suggesting the limited effectiveness of medical care as contrasted to nonmedical factors in improving the health of populations; clinical evidence of the potential for improvement in the provision of medical care; and healthcare reform, both at the federal and state levels in the United States.

While the health of the population overall in the United States has improved substantially over the past century, the health of certain vulnerable groups has declined. Specifically, trends make evident the significant reduction of acute infectious disease mortality, declines in mortality from major chronic diseases, and resulting increases in life expectancy. But, as pointed out in Chapter 1, health status continues to differ by race as well as by other demographic variables; the differences are substantial (see Appendix 1.1). Rates of death due to breast and cervical cancer are much higher for blacks compared to other races. In 2001, the death rate per 100,000 was 35.0 in blacks compared to 26.0 and 16.2 in whites and Hispanics, respectively, for breast cancer and 4.9 in blacks compared to 2.3 and 3.4 in whites and Hispanics, respec-

tively, for cervical cancer. In addition, substantial geographic variations exist in the levels of medical care resources as well as in the rates for various medical and surgical procedures. These findings raise the questions of whether health improvements are in fact attributable to medical care or to some other factor or set of factors, whether the continuing disparities for selected groups are a result of failures in medical care, and whether geographic variations are associated with varying outcomes for patients across areas. These and related questions are addressed by effectiveness research.

In 1996, the Institute of Medicine (IOM) launched a major initiative focused on assessing and improving the nation's quality of healthcare, known as the Health Care Quality Initiative (IOM 2003). The first phase of the initiative built on an intensive review of the literature conducted by the RAND Corporation and a framework that defined the nature of the quality problem as one of overuse, misuse, and underuse of healthcare services. The report emanating from phase one of the initiative, *Ensuring Quality Cancer Care*, documented the wide gulf that exists between ideal cancer care and the reality that many Americans with cancer experience (IOM 1999a). During the second phase, spanning 1999 through 2001, the report *To Err Is Human: Building a Safer Health System* documented how tens of thousands of Americans died each year from medical errors (IOM 1999b). A second report from phase two, *Crossing the Quality Chasm: A New Health System for the 21st Century*, defined six aims—care should be safe, effective, patient centered, timely, efficient, and equitable—and related rules for care-delivery redesign to reduce the magnitude of errors (IOM 2001). Phase three of IOM's Health Care Quality Initiative focused on operationalizing the vision of a future health system described in the *Quality Chasm* report, emphasizing reform at three different overlapping levels of the system: the environmental level, the level of the healthcare organization, and the interface between clinicians and patients.

This chapter presents and discusses a conceptual framework for effectiveness research as a foundation for evaluating the success of the U.S. healthcare system in improving the quality of healthcare *and* ultimately the health of patients and populations. Key methods of effectiveness research from both the clinical and population perspectives are presented and illustrated using breast cancer screening effectiveness studies as examples. Chapter 3 categorizes the various policy strategies

for enhancing population health, reviews the evidence on the effectiveness of each strategy, and develops a set of criteria for assessing policy alternatives in terms of effectiveness in the context of breast cancer prevention and treatment.

CONCEPTUAL FRAMEWORK AND DEFINITIONS

Two Perspectives

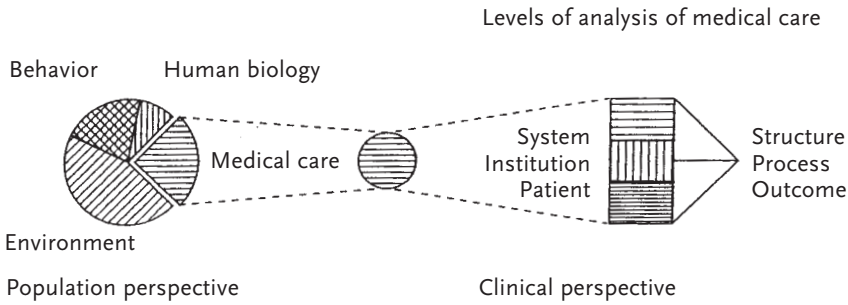
Effectiveness research reflects two seemingly competing, but in fact complementary, definitions of effectiveness (see Figure 2.1). One represents a population perspective, or macro level view, that considers the role of physical, social, and economic environments on the health of the population. This macro definition is represented in the earlier conceptual work of Milio (1983) and the later work of Evans, Barer, and Marmor (1994). It can be characterized as the *epidemiology of health*. It includes in its purview both patients who have received medical care and individuals in the population as a whole who have not.

The second is a clinical perspective that represents the micro-level view and focuses on the interactions of patients and providers in the medical care system and institutions and the resulting clinical improvement or health benefits achieved by patients. Research conducted from this point of view examines the impact of the structures and processes associated with delivering medical care in achieving improvements in the health of patients. It is represented in the work of Donabedian (2003); Kerr White and his colleagues, who introduced the concept of the "ecology of medical care" (White 1997; White, Williams, and Greenberg 1961); Wennberg (1990), who has labeled this area "clinical evaluation science"; and Brook and Lohr (1985), who called for an *epidemiology of medical care*.

Health services research related to each of these views mirrors their differing perspectives.

The epidemiology of health focuses on the benefits from both medical and nonmedical determinants of the health of the population, including environment, behavior, human biology, and medical care. The epidemiology of medical care, or clinical evaluation science, delineates the benefits from medical care for patients. The following example illustrates these differing views.

Figure 2.1 Factors Contributing to Population Health



Sources: Blum (1976); Lalonde (1975); Milio (1983).

The National Health and Nutrition Examination Survey, conducted by the National Center for Health Statistics, periodically collects both interview and physical examination data on a representative sample of the U.S. population. Based on the 1999–2000 survey, Hajjar and Kotchen (2003) determined that of 1,000 people in the population, 287 had elevated blood pressure (hypertension). Of these 287 people, 168 were under medical care for their hypertension, and 89 had their blood pressure effectively controlled by this treatment. Translating this into the perspectives discussed, from a *clinical perspective*, 89 of 168, or 53 percent, of hypertensive *patients* had their blood pressure effectively controlled, but from a *population perspective*, 89 of 287, or 31 percent, of the hypertensive *individuals* in the population had their hypertension effectively controlled. The difference between the two views leads to widely varying empirical estimates of primary care effectiveness: 53 percent versus 31 percent.

The differing perspectives also explore different factors to account for the respective rates: biological or clinical factors, or patient adherence, versus social or behavioral factors, or medical care access. A point worth noting, however, is that while this description of two perspectives is instructive, a middle ground may exist between the two perspectives involving health promotion and disease prevention services that encompasses both medical and nonmedical interventions. This middle ground might be labeled *health-* (in contrast to *medical*) care and represents the transition to a broader set of policy alternatives for improving the health of the population (see Figure 1.1).

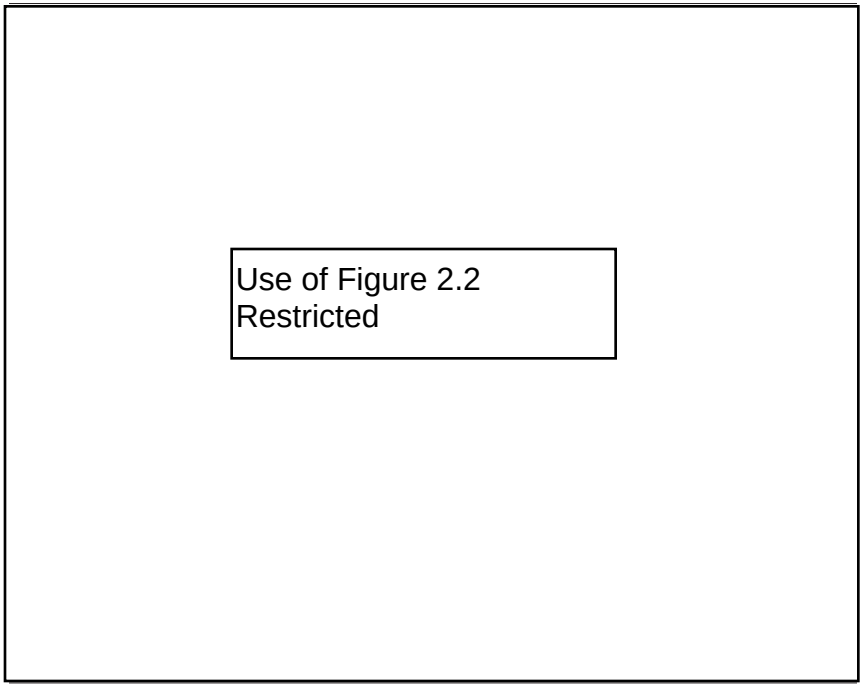
Conceptual Frameworks

The major conceptual frameworks that guide effectiveness research are derived in the population perspective from Evans, Barer, and Marmor (1994) and in the clinical perspective from the work of White, Williams, and Greenberg (1961); Kane (1997); and Donabedian (2003). The Evans, Barer, and Marmor framework, represented in the population perspective displayed in Figure 2.1, defines the determinants of health as the physicial and social environment, human biology, individual behavior, and medical care services. Kerr White and his colleagues are responsible for describing what they termed the ecology of medical care, represented within the clinical perspective displayed in Figure 2.1 at the system, institution, and patient levels, but also including the community level as represented by the medical care component of the population perspective displayed in this figure (White 1997; White, Williams, and Greenberg 1961). From the clinical perspective, Donabedian (2003) first offered the categorization of medical care in terms of structure, process, and outcomes for the purpose of determining those aspects that might be indicators of quality. This is shown in Figure 2.1 as the components that can be examined at the patient, institution, and system levels.

Two conceptual models are presented that serve to further clarify and delineate the central determinants of health from the population and clinical perspective, respectively: Kindig and Stoddart's (2003) concept of the field of population health, and Donabedian's (2003) and Kane's (1997) conceptual models for quality and outcomes research.

Kindig and Stoddart (2003, 380) have defined population health as "the health outcomes of a group of individuals, including the distribution of such outcomes within the group." Figure 2.2 displays a schematic definition of the field of population health from the work by Kindig and Stoddart. This framework centrally incorporates both nonmedical and medical determinants of health over the life course and importantly points out the role of policies and interventions at both the individual and social levels to influence these determinants and, ultimately, the level and distribution of health in the population.

A conceptual framework to analyze the effectiveness of clinical prevention and treatment (e.g., for breast cancer) can be divided into the three classic compartments of structure, process, and outcomes (Donabedian 2003; Kane 1997). This framework for assessing effectiveness in the context of the clinical perspective is illustrated in Figure

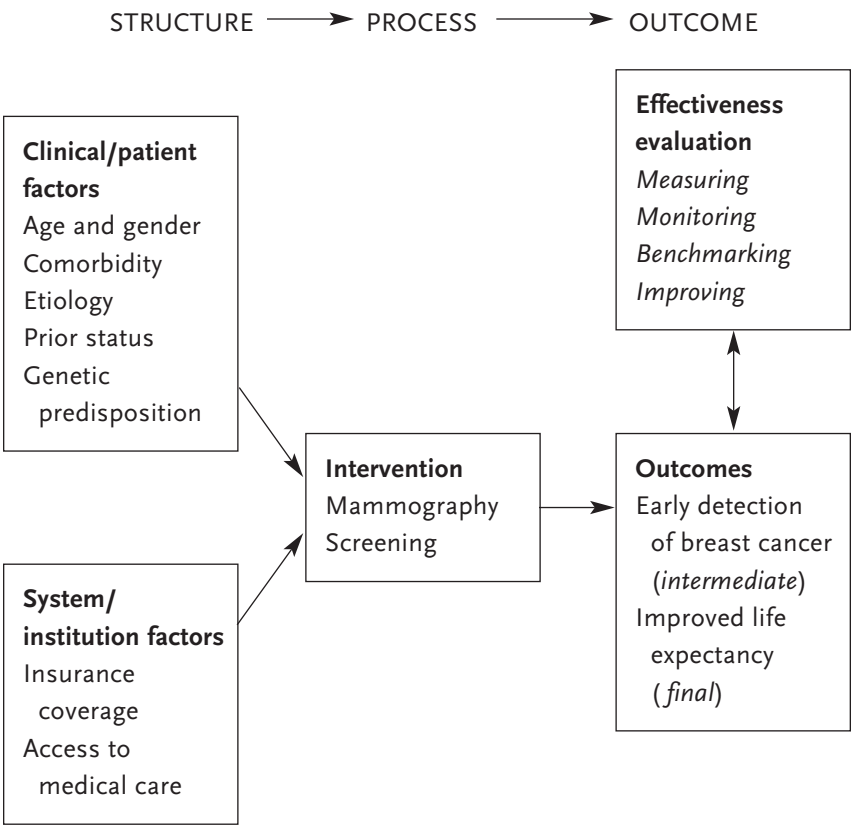


2.3. In this case, structure refers to elements of medical care that are associated with the receipt of services. These may include factors such as availability of insurance coverage and access to facilities. Process refers to the intervention being tested for effectiveness, such as mammography screening. Finally, structure and process together result in outcomes. In the breast cancer example, the intermediate outcome is the early detection of breast cancer, and the final outcome is the improved life expectancy as a result of early detection.

All components of medical care need to be constantly evaluated in the following ways for effectiveness:

1. *Measuring,* for example, the intensity of the intervention (in terms of rates of women obtaining screening), improvement in outcomes (earlier diagnosis of breast cancer), or changes in the structure (increased insurance coverage for screening);
2. *Monitoring* the types of populations receiving the screening, the adoption rates of screening, and improvements in outcomes;
3. *Benchmarking* these aspects of effectiveness against extant standards of quality (such as Healthy People 2010 goals and objectives or

Figure 2.3 Conceptual Model of Health Determinants from the Clinical Perspective



Sources: Adapted from Donabedian (2003, 46–47), Kane (1997, Figure 1-1, 13).

published estimates of maximum effectiveness of interventions); and

4. Ultimately, *improving* the outcomes of care.

The four levels of measuring effectiveness outlined in the integrative framework in Figure 2.1 include the community level, associated with the population perspective, and the system, institution, and patient levels, associated with the clinical perspective. *Community* includes the population as a whole and the environments in which its members

reside. *System* refers to the healthcare system, including resources such as money, people, physical infrastructure, and technology and "the organizations and systems or networks of organizations that transform these resources into health services and distribute them to consumers," either within a specific region or for the country as a whole (Longest 2002, 54). It includes all of the elements within the system nationally or in a specific region. *Institution* refers to a specific organizational entity such as a hospital, clinic, or managed care organization. *Patient* refers to the recipient of services at the clinical level where the focus is on prevention, treatment, or follow-up and includes an encounter between a patient and a provider. As illustrated in Figure 2.1, each of the three levels within the clinical perspective, as well as the medical care system as a component of the population perspective, can be further elaborated in terms of structure, process, and outcome.

Structure refers to

> . . . the conditions under which care is provided. These include: (1) material resources, such as facilities and equipment; (2) human resources, such as the number, variety, and qualifications of professional and support personnel; (3) organizational characteristics, such as the organization of the medical and nursing staffs, the presence of teaching and research functions, kinds of supervision and performance review, methods of paying for care, and so on. (Donabedian 2003, 46)

Delivery system characteristics as defined in the seminal Medical Outcomes Study (Tarlov et al. 1989) include the organization, specialty mix, workload, and access and convenience of care; provider characteristics of specialty training, preferences, and job satisfaction; and patient characteristics of diagnosis and condition, severity, comorbidity, and health habits. The population perspective would view this component relative to the denominator of a community's population as a whole, while the clinical perspective would lodge it in relationship to the enrollees or patients to be served by a given system or institution.

Process refers to "...activities that constitute health care—including diagnosis, treatment, rehabilitation, prevention, and patient education —usually carried out by professional personnel, but also including other contributions to care, particularly by patients and their families"

(Donabedian 2003, 46). Examples of process variables within the clinical perspective include technical aspects such as visits, medications, referrals, test ordering, and hospitalizations and interpersonal characteristics such as interpersonal manner, counseling, and communication level on the part of patients or enrollees. A population perspective would consider utilization rates or de facto (realized) access for the target population in an area.

Outcomes are "...taken to mean changes (desirable or undesirable) in individuals and populations that can be attributed to health care. Outcomes include: (1) changes in health status; (2) changes in knowledge acquired by patients and family members that may influence future care; (3) changes in the behavior of patients or family members that may influence future health; (4) satisfaction of patients and their family members with the care received and its outcomes" (Donabedian 2003, 46–47).

Examples of clinical outcomes include endpoints such as symptoms and signs of problems, laboratory values, disability, and death and health-related quality of life including the physical, mental, social, and role dimensions. The population perspective would focus on overall population mortality, morbidity rates, or health status.

Structure, process, and outcome are linked conceptually in a research paradigm that assumes structural elements of healthcare as having an influence on what is and is not done in the process as well as how well it is done; this process in turn influences the health outcome people experience as a result of their encounters with the process. This categorization and the implied linkage among these components has become the basic conceptualization in studying the effectiveness of medical care and its determinants. These relationships are captured in the summary framework shown in Table 2.1.

Definitions

Table 2.1 provides several illustrative definitions for the major components of the structure, process, and outcomes of care; the discussion that follows summarizes the key idea of each. *Quantity* refers to the number of physicians, nurses, and other providers as well as the quantity of monetary resources. *Efficacy* is concerned with the benefits achievable from a therapy or an intervention under ideal conditions, such as those found in a randomized clinical trial (Cochrane 1971; Donabedian 2003; Lohr, Eleazer, and Mauskopf 1998; Sackett 1980; Williamson 1978).

Variations in use relate to the quantity, or what is more commonly referred to as utilization, of healthcare services and procedures (Lohr, Eleazer, and Mauskopf 1998). It also includes the frequency or volume of procedures done. *Quality* is an attribute of the healthcare process having to do both with whether the right thing is done and whether it is done well (Brook and Lohr 1985; Donabedian 1973, 1980, 1982, 2003; Lohr, Eleazer, and Mauskopf 1998). The Institute of Medicine (2003) Health Care Quality Initiative has defined quality as "the degree to which health services for individuals and populations increase the likelihood of desired health outcomes and are consistent with current professional knowledge." Quality assessment thus deals with evaluating the process of healthcare in the service of ultimately improving health outcomes. *Appropriateness* is the subset of quality that concerns determining whether the right thing was done for the patient.

Effectiveness concerns the results achieved in the actual practice of healthcare with typical patients and providers, in contrast to efficacy, which is assessed by the benefits achieved under ideal conditions (Cochrane 1971; Donabedian 2003; Lohr, Eleazer, and Mauskopf 1998; Sackett 1980; Williamson 1978). Quality is that part of the gap between efficacy, or what is achievable, and effectiveness, or what is achieved, that can reasonably be attributed to healthcare itself. Evidence-based medical care focuses on the use of the best available efficacy and effectiveness evidence to inform decisions about patient care and guide healthcare policy (Lohr, Eleazer, and Mauskopf 1998).

In conclusion, the argument has been made that both the population and clinical perspectives are important in examining the effectiveness of healthcare. The population perspective argues that nonmedical as well as medical investments are required to improve the health of individuals and communities, while the clinical perspective illuminates how enhancing the precision of medical care can contribute to this improvement.

KEY METHODS OF ASSESSING EFFECTIVENESS

Two basic questions related to effectiveness were presented at the beginning of this chapter: (1) What is effectiveness? and (2) How should the effectiveness of healthcare be assessed? Table 2.2 introduces a framework for effectiveness research that attempts to integrate the two perspectives—population and clinical—and the four levels—community, system, institution, and patient—in empirically addressing these questions. The

Table 2.1 Dimensions of Effectiveness

Structure	*Process*	*Outcomes*
Quantity **Efficacy**	**Variations in Use** **—Quantity** **—Quality** **—Appropriateness**	**Effectiveness** **—Mortality** **—Morbidity** **—Health status**
QUANTITY Refers to the number of physicians, nurses, and other providers as well as the quantity of monetary resources.	VARIATIONS IN USE Refers to different observed levels of per capita consumption of a service, especially hospital care, office visits, drugs, and specific procedures.	EFFECTIVENESS Refers to *actual* achieved benefit. *Does it work*? Does the maneuver, procedure, or service do more good than harm to those people to whom it is offered?

EFFICACY

Refers to maximum *achievable* benefit.

Can it work?
Does the health maneuver, procedure, or service do more good than harm to people who fully comply with the associated recommendations or treatment?

This question refers to the ability of a particular medical action in altering the natural history of a particular disease for the better, under ideal conditions.

Sources: Cochrane (1971); Donabedian (2003); Lohr, Eleazer, and Mauskopf (1998); Sackett (1980); Williamson (1978).

QUALITY

Refers to a judgment concerning the process of care, based on the extent to which that care contributes to valued outcomes.

Quality of medical care is that component of the difference between efficacy and effectiveness that can be attributed to care providers, taking account of the environment in which they work.

APPROPRIATENESS
Refers to the extent to which available knowledge and techniques are used or misused in the management of illness and health.

Sources: Brook and Lohr (1985); Donabedian (1973, 1980, 1982, 2003); Institute of Medicine (2003); Lohr, Eleazer, and Mauskopf (1998).

This question refers to the ability of a particular medical action in altering the natural history of a particular disease for the better, under actual conditions of practice and use.

Sources: Cochrane (1971); Donabedian (2003); Lohr, Eleazer, and Mauskopf (1998); Sackett (1980); Williamson (1978).

population perspective focuses on addressing these questions in the context of a community-level analysis, while the clinical perspective can seek to address them at the system, institution, or patient level of analysis or at a combination of these levels.

The outcome measures, risk-adjustment procedures, study designs, and data sources that might be drawn on at each level, as well as examples that illustrate the application of these methods at each of the levels, are highlighted in Table 2.2 and are discussed in the sections that follow.

Outcome Measures

Outcome measures fit into the general categories of mortality, morbidity, and health status, although specific indicators of each of these broad outcome measures may be used at different levels. The usefulness of a measure depends in part on the degree to which it meets the criteria of reliability, validity, feasibility, and sensitivity (McDowell and Newell 1996). *Reliability* concerns the reproducibility of the measure under various conditions of administration. *Validity* relates to the accuracy of the measure, in the sense that it measures what it is intended to measure. *Feasibility* refers to the ease with which the scale can be used in various populations. *Sensitivity* refers to the ability of the measure to detect changes—improvement or deterioration—in the condition of the person as a result of healthcare. Sensitivity to healthcare variation is particularly important for outcome measures being used to assess the effectiveness of care.

Community-level outcome measures include population death, morbidity, and disability rates as well as disease prevalence and incidence rates and perceived health status. One problem with these measures is how to combine them into a positive index of the community's or population's health, as opposed to negative indexes such as death rates, to yield a representation of health-adjusted life expectancy (Kindig 1997). The disability-adjusted life year (DALY) represents one attempt to combine these community-level measures in a way that reflects the burden of disease on a population (Murray and Lopez 1996). Specifically, DALY expresses years of life that are lost to premature death and years lived with a disability of specified severity and duration. One DALY is thus equivalent to one lost year of healthy life. In a comprehensive review, the proponents of this approach have examined the global burden of disease using the DALY measure. They find that the leading causes of disease burden were the following: childhood and maternal underweight

(138 million DALYs, 9.5 percent), unsafe sex (92 million DALYs, 6.3 percent), high blood pressure (64 million DALYs, 4.4 percent), tobacco (59 million DALYs, 4.1 percent), and alcohol (58 million DALYs, 4.0 percent) (Ezzati et al. 2002). Another attempt to index a population's health was based on the years of healthy life derived from a combination of the responses to the activity limitation and self-perceived health status questions from the National Health Interview Survey (NCHS 1995).

Population-based data, as exemplified by mortality rates, while relatively high in reliability, validity, and feasibility, have been shown repeatedly to be insensitive to medical care variation. This suggests that these data are useful for addressing the question of medical care's contribution to the health of the population, but they have limited applicability in assessing the clinical effectiveness of medical care.

For examining mortality, morbidity, and health-status outcomes across institution and system levels, outcomes may be aggregated from the patient level within these categories. Patient-level outcome measures focus on individual deaths, on morbidity as reflected both in adverse outcomes and disability limitations, and on the health status outcomes of clinical endpoints, such as blood sugar and blood pressure and subjective health-status measures. At the institution and system levels, these patient-level measures are aggregated to produce case fatality, complication, disability, and diagnosis rates as well as averaged subjective health status for groups of patients.

Subjective Health-Status Measures

Subjective health-status (SHS) measures, based on individuals' self-reports, are singled out here for a more detailed discussion because they have undergone extensive development and have achieved widespread use in the past two to three decades (Ware 2003). They may be of a generic type, applicable across all disease conditions, or of a disease-specific type. Both types are needed in the assessment of the medical care outcomes—the generic indicators for comparisons across disease conditions, and the disease-specific ones to more sensitively identify the effects of diseases on people and the effects of treatments on particular disease conditions. A range of possible SHS measures has been collected in *Measuring Health: A Guide to Rating Scales and Questionnaires* (McDowell and Newell 1996). For each of over 100 instruments, this book presents a description, copies of the actual questionnaire, information on the reliability and validity of the instrument, and a

Table 2.2 Framework for Effectiveness Research

	Level of Analysis			
	Population Perspective	***Clinical Perspective***		
	Community	***System***	***Institution***	***Patient***
Outcome measures	Mortality	Mortality	Mortality	Mortality
	Population death rates	Case fatality rates	Case fatality rates	Individual deaths
	Morbidity	Morbidity	Morbidity	Morbidity
	Population morbidity rates	Complication rates	Complication rates	Adverse events
	Disability rates	Disability rates	Disability rates	Disability limitation
	Health status	Health status	Health status	Health status
	Disease incidence and prevalence rates	Diagnosis rates	Diagnosis rates	Clinical endpoints
	Perceived health status	Averaged HRQOL	Averaged HRQOL	Health-related QOL
Risk adjustment	Demographic characteristics	Demographic characteristics	Demographic characteristics	Patient profiles
		Comorbidity rates	Comorbidity rates	Comorbidities
		Risk-adjustment systems	Risk-adju	agnoses
Study designs	Observational-epidemiological	Observational-interorganizational	Observat intrao	servational Case reports/series perimental—RCT nthetic Meta-analysis Decision analysis

Data sources	Records Population health information system Vital statistics Disease surveillance Surveys	Records Medical records Discharge data Claims data Surveys	Records Medical records Discharge data Claims data Surveys	Records Medical records Discharge data Claims data Surveys
Example	Chinese American community screening (Tu et al. 2003)	European national screening program (de Koning 2000)	Public hospital clinic screening program (Thompson et al. 2000)	Patient screening in response to intervention (Ell et al. 2002)

Typical Effectiveness Research Questions by Level of Analysis

Community	What is the contribution of medical care to the health of the population?
System	What is the impact of system-level variables (e.g., provider specialty mix, organizational form, payment mechanism) on the processes and outcomes of medical care?
Institution	What is the impact of the quality of care on the outcomes of medical care?

Note: HRQOL = health related quality of life; QOL = quality of life; RCT = randomized clinical trial.

complete listing of references. McHorney (1999) also provides a brief summary and review of the ten most widely used SHS measures.

One example of generic SHS measures is the Medical Outcomes Study Short Form (SF-36), with its simpler companion, the SF-12. This questionnaire contains an array of questions related to the effect of disease on physical health, mental health, and social health, as well as on health perceptions. The scores on each of eight dimensions were originally kept separate and presented as a profile. They have subsequently been grouped into two categories to yield summary scores for physical and mental health, respectively (QualityMetric 2003; Ware, Kosinski, and Keller 1996).

In addition to the generic measures are disease-specific subjective health status measures, such as the European Organization for Research and Treatment of Cancer Core (Aaronson et al. 1993) and the Functional Assessment of Cancer Therapy—General (Cella et al. 1993), which have been developed specifically for cancer patients, and the Dermatology Life Quality Index (Finlay and Khan 1994) and the Skindex (Chren et al. 1997), which have been developed for patients with skin disease. Such questionnaires focus on unique aspects of the disease for which they were developed. All the instruments described above are reliable; valid; feasible; and, above all, sensitive to the changes following medical treatment.

The optimum strategy for outcomes assessment, given the different levels of sensitivity, may be to use a generic instrument, such as the SF-36 or the SF-12, supplemented with disease-specific questions or a disease-specific questionnaire (e.g., see Ren et al. 1998). The generic instrument would allow comparisons to be made across diseases, while the disease-specific instrument would be more likely to provide sufficient sensitivity to detect small changes in patients' conditions.

Other Subjective Outcome Measures

In addition to the above measures, the use of other patient-centered outcome measures to evaluate aspects of medical care quality such as satisfaction (which evaluates perceptions of discrete past healthcare transactions) and trust (a forward-looking assessment of an overall relationship) with medical care is becoming commonplace in many health services research studies. Several measures of trust of and satisfaction with physicians, insurers, and the medical profession have been devel-

oped and validated and are being used in assessments of overall healthcare quality (Balkrishnan et al. 2003). Consumer assessments of healthcare provide important information about how well health plans and clinicians meet the needs of the people they serve. The Agency for Healthcare Research and Quality has sponsored development of the Consumer Assessment of Health Plans Survey (CAHPS), an integrated set of tested and standardized questionnaires and reporting formats to collect and report information about the experiences of consumers enrolled in health plans since 1995 (Hays et al. 1999). Studies have reported that CAHPS ratings could affect consumer selection of health plans and ultimately contain costs (Spranca et al. 2000).

Risk Adjustment

Ultimately, the value of all effectiveness research depends on the ability to validly adjust for differences in risks associated with final outcomes. Risk adjustment of patient outcomes is necessary in effectiveness research to account for the differing risks patients bring to the clinical setting. Clearly, patients who differ at admission to a hospital in their risks and who receive similarly effective treatments will experience different outcomes. When randomized clinical trials are possible, these differences can be minimized by the random allocation of subjects to experimental and control groups. But under nonexperimental conditions, under which most effectiveness research is done, these differences and their potential confounding should be adjusted for in the analysis. These differing risks that require adjustments include differences in patient demographic characteristics such as age, gender, and race; comorbidities, or conditions unrelated to the primary illness that can negatively affect treatment outcomes; and diagnoses that may differ in the initial severity of illness.

At the patient level, two general approaches may be taken in this adjustment. A subjective approach, relying on the informed judgment of experienced clinicians in rating the severity of the patient's illness at entry, may provide a valid assessment of a patient's status (Charlson et al. 1986), but such an expensive procedure, in terms of the physician's time, is rarely possible. In its place, an objective approach, utilizing clearly identified data related to the patient's risk, clinical state, and probable outcome, applies an algorithm or formula to generate a score characterizing the patient's risk. These data may include characteristics

of patients, their comorbid conditions, and their diagnoses, which at the institution and system levels may be incorporated into risk-adjustment systems such as the Acute Physiological and Chronic Health Evaluation (APACHE) scale or the Medical Illness Severity Grouping System (MedisGroups) described later. Also at the institution and system levels, where some of the detailed patient data such as discharge and claims data may be lacking, demographic characteristics or comorbidity rates may be used as proxies for actual severity measures. At the community level, demographic characteristics such as age and gender are used to adjust for differing risk of illness.

The risk-adjustment methods in common use assume the objective approach described above; 11 types of them, all at the patient level, are described and thoroughly analyzed in *Risk Adjustment for Measuring Health Care Outcomes* (Iezzoni 2003). This resource also provides information on the dimensions of risk, data sources, and performance of these measures. Iezzoni specifies several issues important to the assessment and measurement of risk: level of analysis, time frame for observation, timing of data collection, feasibility, reliability, and validity. The level or unit of analysis determines both the data that are available and the dimensions important to consider. Health-related quality of life (HRQOL), for example, is not likely to be found when entire systems are being analyzed, because HRQOL is not routinely collected on all patients in all settings. The time frame for observation of outcomes—for example, whether consideration is to be given only to hospital inpatient events or to things occurring within six months postdischarge—determines which dimensions are important. The timing of data collection is also important; if the severity of illness at admission is to be the basis for the risk adjustment, it is not appropriate to use risk data gathered from the entire stay in the hospital because the results may be confounded by the treatments subsequent to admission.

The 11 different systems for risk adjustment that Iezzoni (2003) describes represent a mix of dimensions as well as disease-specific and generic measures. All are proprietary to some extent, and are therefore less available for the kind of critical analyses done of outcome measures. Two of these, APACHE and MedisGroups, will be described now in greater detail. Both are grounded in the clinical perspective on effectiveness. APACHE, one of the first risk-adjustment systems developed,

continues to be updated and widely applied. MedisGroups has been mandated in several states as the system to be used by hospitals. (See Cardinal Health [2003] and Cerner [2003] for more information on the vendors for these systems.)

The APACHE scale was developed for the evaluation of patients in hospital critical care units and uses a dozen physiologic values generated from physical findings and laboratory data in the first 24 hours after admission (Knaus et al. 1985). Scale scores for each of these values are added and are combined with adjustments for age and chronic health conditions to yield an overall score. The APACHE score is generic in that it can be applied across diseases for comparisons of severity. Subsequent versions of APACHE, such as APACHE III, added several more variables to the risk-adjustment scale such as age and additional comorbid conditions (Knaus et al. 1991; Knaus 2002).

MedisGroups has assumed great importance because several states have mandated it as the risk-adjustment measure to be used by hospitals in reporting data to state agencies. The MedisGroups system produces a generic set of severity categories across illnesses, using medical record data processed by a proprietary program (Brewster et al. 1985). It uses "key clinical findings," including laboratory, radiology, pathology, and physical examination data. This information can be input into the system's coding scheme to permit the severity of patients' conditions at admission, as well as their progress over the course of the hospitalization, to be monitored. The MedisGroups system has been widely used for risk adjustment in hospital-based health services research studies (Iezzoni et al. 1998; Silber et al. 1999).

These risk-adjustment methods and severity-of-illness measures can and should be evaluated by the same criteria as health-status measures —reliability, validity, feasibility, and sensitivity. Reviews (Iezzoni 2003) and studies (Hwang et al. 2001) have summarized the information from studies comparing measures of these attributes. Because the objective methods use factual data and are computerized, they are both basically reliable and feasible. Their validity continues to be a question, and they vary in sensitivity. Risk adjustment is less well developed than outcome measurement and is impeded by the fact that much of the work is in the proprietary domain. Iezzoni, however, has contributed greatly to removing the veil from these systems.

Study Designs

Study designs for effectiveness research cover a range of possibilities (see Table 2.2). The design principles are the same as those for any study: maximize experimental variance, control extraneous variance, and minimize error variance. On one hand, applying these principles results in outcomes research designs that follow true experimental design principles of random allocation, control groups, blinding, and homogeneity and lead to efficacy studies. On the other hand are nonexperimental observational designs, in which investigators do not directly intervene but instead develop methods for describing events that occur naturally and their effect on study subjects. These types of studies characterize much of effectiveness research and are represented by examples of effectiveness studies presented later in this chapter. Alternatives include meta-analysis and decision analysis, sometimes called synthetic designs (Petitti 1999).

The assessment of efficacy—the determination of the benefits of a particular medical therapy, health service activity, or public health intervention under ideal conditions—involves the randomized clinical trial (RCT) as the primary method of analysis. The hallmark of the RCT is random assignment of patients to experimental and control groups and, hence, control of much of the extraneous variation and sources of error. Good examples of RCTs concerning medical therapies include trials of major outcomes in high-risk hypertensive patients randomized to angiotensin-converting enzyme inhibitor or calcium channel blocker versus diuretics: the Antihypertensive and Lipid-Lowering Treatment to Prevent Heart Attack Trial (ALLHAT 2002) and the Women's Health Initiative Memory Study, a trial of the effect of estrogen therapy in preventing and slowing the progression of dementia (Shumaker et al. 2003). Large, simple trials have been used to extend the range of RCTs to situations where treatment effects are modest in size (Peto, Collins, and Gray 1995).

RCT designs have been used to assess the benefits of ways in which medical care is delivered, for instance, in the evaluation of effectiveness of pharmacist care for patients with reactive airways disease (Weinberger et al. 2002) and in the comparison of sentinel-node biopsy with routine axillary dissection in breast cancer (Veronesi et al. 2003). This design was also used to assess the effects of different medical care payment plans on use and outcomes in the RAND Health Insurance Experiment (Brook et al. 1983). RCTs have been used less frequently to

evaluate public health interventions (Orleans 1995); the polio vaccine trials conducted in the 1950s are, however, an outstanding exception and a good example of large public health trials.

RCTs are a powerful method but are not often feasible because of the expense necessitated by the large sample sizes and the length of time required to conduct them. Ethical issues involved in depriving patients of treatment may also preclude their use. Another problem with RCTs is that many are done on a small, local scale, and therefore the results often are not highly significant nor widely disseminated. Because of the difficulties and expense inherent in RCTs, they must be used selectively. As a result, many important treatment questions cannot be answered by such designs.

An alternative to RCTs is the use of synthetic methods such as meta-analysis and decision analysis. *Meta-analysis* involves quantitatively and statistically combining the results of several RCTs to estimate the results of therapy when no single trial may be sufficient in number of patients to yield a statistically significant result (Pettiti 1999). Meta-analyses have been used, for example, to obtain estimates of the effectiveness of nocturnal, noninvasive, positive-pressure ventilation in patients with stable chronic obstructive pulmonary disease (Wijkstra et al. 2003), efficacy and safety of ephedra and ephedrine for weight loss and athletic performance (Shekelle et al. 2003), and health outcomes associated with antihypertensive therapies used as first-line agents (Psaty et al. 2003). Meta-analyses are also being used in the Cochrane Collaboration (2003), which is a major international effort directed to ensuring that all areas of healthcare that have been evaluated using RCTs would be covered. Collaborators in this process prepare and maintain systematic reviews of RCTs as well as other evidence where appropriate. These reviews are then maintained in a database and disseminated for use.

Decision analyses synthesize information about effectiveness to determine the value of one approach versus another for policy analysis and ultimately for clinical decisions. A decision analysis requires information on the actual treatment of patients with disease, the outcomes, and the value of those outcomes to patients. Information from large databases and other sources is used to estimate the probabilities of different outcomes from therapy for patients. Patient surveys provide information on patients' symptoms as well as their preferences for different outcomes. The advantage of a decision analysis is that it synthesizes a large amount of information relevant to effectiveness. The disadvantage

is that necessary data on patient values or preferences are often not available. An example of decision analysis using national-level Medicare data concerning breast cancer screening (Mandelblatt et al. 1992) is discussed in Chapter 9.

Observational nonexperimental designs, chiefly cross-sectional studies, are another design type, and characterize the bulk of the literature on effectiveness. Community-level epidemiological studies use existing registries or databases such as the Medicare database. System- and institution-level studies use databases appropriate to these levels, such as hospital discharge and claims data, to address issues within the organization (intraorganizational) and between organizations (interorganizational). At the patient level, when RCTs are not possible, reports of individual cases and series of them are reported. One problem with these observational studies is that the databases used may have significant biases due to missing values, lack of validating evidence, or the unrepresentativeness of the database and that these biases may limit the generalizability of study results. Another problem is that random allocation has not been possible, and therefore statistical adjustments are applied to account for the differences in mix of patients that may result from possible selection biases. Despite these limitations, however, observational studies are more feasible than experimental studies and draw on large volumes of data reflecting actual experience. Most of the studies of effectiveness, and thus much of the evidence about effectiveness to be presented and reviewed in the next section of this chapter, derive from these observational designs.

Data Sources

Where clinical trials and true experiments are not possible, effectiveness research relies on a variety of data sources for the cross-sectional studies (as shown in Table 2.2). Surveys of institutions, providers, and patients, as well as records of medical care, discharges, and claims, provide information for effectiveness research at the patient, institution, and system levels, dependent on the level of aggregation of the data. At the community level, public health surveillance systems and vital statistics data that may be used to construct population health information systems provide the data for effectiveness studies.

Community-level outcome measures such as population mortality rates can be obtained from state and U.S. Vital Statistics data, as well as from the World Health Organization and the Organization for

Economic Cooperation and Development data for international comparisons. Morbidity rates can be acquired from the National Center for Health Statistics (NCHS) National Health Interview Surveys (NHIS). These surveys are conducted annually on a sample of the U.S. population and yield, among other data, information on limited activity days and restricted activity days. Disease prevalence and incidence data can also be obtained from NHIS data, as well as from other federal sources such as the *Mortality and Morbidity Weekly Report* of the Centers for Disease Control and Prevention.

The surveys conducted by NCHS provide a rich source of information for effectiveness research. Information is gathered, for example, by the NHIS on respondents' use of medical care. Provider surveys, such as the National Ambulatory Medical Care Survey, the National Hospital Discharge Survey, and the National Long-Term Care Survey, provide aggregate information on patients and their use of healthcare services. Diseases are also recorded in national registries, some maintained by the government and some by private sources. An example of the former is the National Cancer Institute's Surveillance Epidemiology and End Results Program (2003) database.

The majority of effectiveness research studies, however, rely on medical records and related sources such as claims data collected for billing purposes and hospital discharge abstract data collected principally for quality assurance purposes. Administrative databases are maintained to keep records of delivery of healthcare services, such as reimbursing medical care providers or determining patient eligibility for certain services covered in part or full by an insurance provider. This information is collected and maintained by the payer (government or private insurer) primarily for reimbursement, but also for other primarily financial reasons such as monitoring, enrollee risk assessment, and rate setting.

The three major sources of these databases are the federal government (Medicare, VA), state governments (Medicaid), and private insurance companies (HMOs, PPOs). Other large databases such as the Health Plan Employer Data and Information Set (HEDIS) and the Computerized Needs-Oriented Quality Measurement Evaluation System (CONQUEST) have been developed for evaluation of quality and performance of healthcare organizations. The type of data collected in each of these databases varies, but most of the administrative databases today contain information on select patient demographics (maintained in an eligibility or enrollment file) and major healthcare service utilization (hospitaliza-

tions, emergency department visits, outpatient physician visits, surgical procedures). Most of these are recorded using several coding systems. The International Classification of Diseases, 9th revision (ICD-9) disease codes are most commonly used to identify the diagnosis, while a number of methods are used to classify medical procedures.

Hospitals usually use the ICD-9 procedure codes, while physicians employ the Centers for Medicare & Medicaid Services Healthcare Common Procedure Coding System, which is largely based on the American Medical Association's Current Procedural Terminology. Some payers also include prescription drug benefit as a part of the insurance, and information on this is recorded in separate outpatient pharmacy files. Medications are usually coded using the National Drug Codes (NDCs). Most of the data in these claims files exists as payment records for each service rendered. The most important structural attribute that determines the utility of the claims data for research in patient outcomes is the ability to link these service records at the individual patient level and construct "episodes of care" per patient. This is usually achieved through a common patient identification number, which is present in all the data files.

However, presence of this patient identifier (usually social security number) also raises serious concerns about patient confidentiality and privacy issues, and these numbers are usually scrambled or replaced before administrative data are released for research. These requirements have become even more stringent in light of the Health Insurance Portability and Accountability Act enforcements. However, the linkage back to the patient may be required for many effectiveness studies, and in some specific types of research where patient identification becomes necessary, full review from the Human Subjects Committee or an institutional review board may be required. Once episodes of care are created for patients, it is possible to track down serious medical events, adverse events requiring secondary or tertiary medical care, and patient mortality types of outcomes data that may be required in effectiveness studies.

The advantages provided by administrative databases include providing information over long periods of time (longitudinal records), as well as more detail regarding procedures and services received than respondents would be able to recall in surveys. Problems with use of administrative data include patients dropping out because they are no longer eligible for insurance; missing data for variables of interest; lack

of capture of variables of interest; selection of specific types of patients into insurance plans or provider groups, thus introducing bias; and logistical limitations due to extremely large sample sizes of patients (Iezzoni 2003; Quam et al. 1993). Also, one has to remember that the information provided by these databases cannot be used to establish causal temporality; rather, these observational data only imply associations between variables.

Examples

Several studies are presented as prototypical examples illustrating each level of effectiveness research—community, system, institution, and patient (see Table 2.2). They also demonstrate the use of the various effectiveness research methods discussed earlier (i.e., outcomes measured, risk adjustment used, basic study design, and data sources). The prototypical examples that will be reviewed include a community level example of the effectiveness of a breast cancer screening program among women in the Chinese-American community in one U.S. city, a system level example of effectiveness of breast cancer screening programs in European countries, an institution-level example of an effectiveness evaluation of an intervention to increase mammography utilization in an inner-city public health hospital, and finally, a patient-level example involving evaluation of a targeted, structured intervention designed to reduce barriers to diagnostic follow-up adherence and initiation of treatment in low-income women with abnormal mammogram screens.

Community-Level Example

A study by Tu et al. (2003) assesses the current screening knowledge and practices related to mammography screening behavior of Chinese American women in Seattle, Washington, in 1999. The study sample was based on a cross-sectional, complex representative sampling design **(community-level)**, and all interviews were conducted in the respondents' home by bilingual, bicultural, Chinese American female interviewers. Outcome variables included recent/past receipt of screening mammograms. Specific associations between language concordance with physician, physician ethnicity and gender, and outcome variables were examined. Seventy-four percent of the surveyed women reported prior mammography screening, and 61 percent reported receiving a screening in the past two years. Although language concordance with physician was associated with higher screening rates, similar to the general

population, a recommendation by primary care physician or nurses increased rates of mammography significantly, irrespective of language concordance. Based on this community survey study, the authors recommend a multifaceted approach to increase mammography screening by Chinese American women. This includes aggressive recommendation by the primary care provider and targeted education to address the effectiveness of screening mammography compared to other options (breast self-examination and clinical breast exams) in this specific population.

System-Level Example

A review article by de Koning (2000) assessed the potential effectiveness of a national breast cancer screening program that had been instituted in the Netherlands in 1987 in the context of similar attempts in other European countries. In particular, the driver of the national screening program in the Netherlands **(system-level)** was the data from three large community trials in Sweden as well as national programs in England and Wales that have estimated a 20 percent reduction in breast cancer mortality at the population level. The program comprised two screenings per year for all women between 50 and 70 years. As far as the Dutch program was concerned, at the time of the report's publication, it was still too early too reach any conclusions about mortality reductions. However the first short-term results of the screening program had exceeded expectations with 1.4 million of the 1.7 million who had been offered the program being examined and early-stage cancer detection rates of almost 6 out of every 1,000 women screened for the first time. The author calls for extra attention that will have to be given to characteristics of tumors that will be detected during subsequent screens in the Dutch program by comparison with findings in other countries to evaluate the overall cost-effectiveness of maintaining this program in future years.

Institution-Level Example

To increase rates of mammography among low-income, urban women in their 50s and 60s, Thompson et al. (2002) conducted a comprehensive **(institution-level)** intervention in a public hospital delivering comprehensive medical services to low-income residents of a large inner city. A total of 196 eligible women age 50–74 years who were enrolled

in the internal medicine clinic, were noncompliant with mammography screening, and had at least one routine clinic appointment during the 15-month study were entered into an RCT of a comprehensive nurse-administered motivational intervention to increase mammography rates. Overall, 49 percent of the women who received the intervention had a mammography within eight weeks of an index visit compared to 22 percent of the control women. There was an additional cost of $151 (1996 U.S. dollars) associated with receiving the intervention itself and $559 in additional cost incurred for each woman who was motivated to receive a mammogram because of the intervention. The societal perspective was used in estimating costs, meaning that the costs included those borne by the patient, the payer (insurer), and society combined. Through this study the authors demonstrated the effectiveness of the motivation program at the institutional level and developed a cost-tracking model while intervening in a clinic institution setting, thus allowing the institution to make informed decisions about implementing programs to increase the motivation of their patients to receive screening.

Patient-Level Example

A study by Ell et al. (2002) involved evaluation of a targeted, structured intervention designed to reduce the number of known barriers to diagnostic follow-up adherence and initiation of treatment among low-income women with abnormal mammogram findings in Los Angeles and New York. The intervention consisted of identification of potential barriers through a scripted, structured telephone interview. Based on symptoms reported in the interview, the women were assigned to a risk level for nonadherence based on an empirical algorithm. High-risk patients (patients with significant mental health symptoms, patients with psychosocial stressors, and women who had received a diagnosis of cancer) were then referred to the team social worker for further individual **(patient-level)** assessment and intervention. Patients also received reinforcing telephone calls every six months. The observational pilot study in 605 women in two large, urban diagnostic centers showed that adherence rates through diagnostic resolution and initiation of treatment in the intervention group was more than 90 percent. Rates of adherence among women who could not be located or who refused study consent was significantly lower (70 percent). The study results supported the combining of interventions and the practical utility of

the clinical decision-making algorithm in determining individualized risk of nonadherence, thereby effectively identifying "at-risk" subjects for further intervention.

SUMMARY AND CONCLUSIONS

In this chapter, effectiveness has been defined in terms of two complementary views—a population perspective and a clinical perspective. The population view asks what contributions medical care makes to the health of the population. The clinical view, by contrast, asks how medical care improves the health of patients who enter the system for care. This chapter presents a framework that attempts to integrate these views in identifying the medical and nonmedical determinants of population health. The key methods of effectiveness research that help provide answers to these questions have been described, discussed in terms of their strengths and weaknesses, and illustrated in a set of example studies. The next chapter illustrates the useful application of these methods in a broad range of outcomes research answering the basic effectiveness questions.

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Chapter 3

Effectiveness: Policy Strategies, Evidence, and Criteria

CHAPTER HIGHLIGHTS

1. Health policy strategies based on a *population perspective* are aimed at environment, behavior, human biology, and medical care as major contributors to population health, whereas a *clinical perspective* considers strategies related to the structures, processes, and outcomes of medical care and is directed at improving patient-, institution-, and system-level performance.
2. Increasing investments in medical care has long been the strategy of choice for improving the health of the population, but evidence of the effectiveness of medical care in achieving this goal varies across institutional settings.
3. The health of populations in general, as well as at-risk groups in particular, is most likely to be enhanced, however, by focusing more resources on nonmedical determinants of health, such as the physical, social, and economic environments in which individuals live and work.
4. To improve *clinical effectiveness*, outcomes assessment and management should foster the development of practice guidelines to increase the precision of medical care and the development of performance-monitoring systems to monitor and improve the process and outcomes of care for selected clinical conditions.
5. To improve *population-level effectiveness*, health policies and programs should be based on the results of community health–needs assessments and the development of a continuum of healthcare programs and services.

OVERVIEW

The basic health policy question, from an effectiveness viewpoint, is, What policy strategies contribute most to improving the health of the population? The answer to this question clearly depends on the perspective taken: population or clinical. This chapter begins with a review of the health policy strategies available and implemented in improving the health of the population, from both a population perspective and a clinical perspective. It then reviews health-status indicators at the population level on the assumption that, ultimately, the question is whether the strategies and interventions derived from either the population or the clinical perspective contribute to improvements in the health of the population. The evidence bearing on each of the broad policy strategies discussed is then reviewed, and a set of effectiveness criteria for evaluating various policy strategies is introduced, using breast cancer screening and treatment as an example.

POLICY STRATEGIES RELATING TO EFFECTIVENESS

Health policy strategies can be related to the overall conceptual framework of factors contributing to population health discussed in Chapter 2 (see Figure 2.1). A population perspective considers strategies aimed at environment, behavior, human biology, and medical care as major contributors to population health, while a clinical perspective considers strategies related to structures, processes, and outcomes of medical care and is directed at improving patient-, institution-, and system-level performance. Grounded in these respective determinants, an overview of broad policy strategies that have been applied is presented in Table 3.1. The population perspective yields general strategies such as investing in overall population health information monitoring systems, health protection, health promotion, and preventive services and medical care strategies such as biomedical research, investment in resources, health planning and regionalization of services, organized delivery systems, and enhanced access. The clinical perspective yields regulation of professional performance, and outcomes assessment and management with its attention to practice guidelines and performance monitoring.

Population Perspective

One set of policy strategies relates to the population perspective, a focus on health rather than illness. While the contribution of nonmedical

factors to health improvement has not had a significant influence on the formulation of health policy in general, it is broadly reflected in public health strategies. An example of a population health strategy comes from Canada, where the Lalonde (1975) report set forth an agenda for improving the population's health based on the recognition that health is determined as much by environment, lifestyle, and human biology as by healthcare services. The pursuit of this population health strategy led to a Canadian policy of equal access to *health*, as opposed to equal access to health*care* (Mhatre and Deber 1992); to a focus on the full range of health determinants in formulating health policy (Evans, Barer, and Marmor 1994); and to the adoption of a population health information system in British Columbia to guide the development of a comprehensive population health policy.

An overall population-based strategy calls for the establishment of information systems to monitor the health of the population and all of its determinants. In the Population Information System (POPULIS) in British Columbia, administrative data routinely collected as part of the national health insurance plan are used as the basis for an integrated database and population-based health information system (Roos et al. 1996, 1999). This information system is proving useful to policymakers for answering such effectiveness questions as, Which populations need more or fewer physician services? Are high-risk populations poorly served or do they have poor health outcomes despite being well served? Does high utilization represent overuse or is it related to high need? More specifically, this system provides decision makers with the capability to make critical comparisons across regions and subregions of residents' health status; socioeconomic risk characteristics; and use of hospitals, nursing homes, and physicians. The system permits policy analyses of demographic changes, expenditure patterns, and hospital performance in relation to the population served. The integrated database has also facilitated outcomes research across hospitals and countries, utilization review within a single hospital, and longitudinal research on health reform (Roos et al. 1999).

In the United States, the growing knowledge about health determinants was responsible in part for the development of an explicit policy strategy of health promotion and disease prevention, beginning with the adoption of the *Healthy People* report (U.S. DHEW 1979) and the establishment of the Office of Health Promotion and Disease Prevention. This effort began with a set of specific objectives enumerated in the

Table 3.1 Health Policy Strategies Related to Factors Contributing to Population Health

Contributing Factor	*Policy Strategy*
Population perspective	Population health information systems
Environment	Health protection
Behavior	Health promotion
Human biology	Biomedical research
	Preventive services
Medical care	
Structure	
Efficacy	Biomedical research
Quantity	Investment in resources
Distribution and organization	Health planning and regionalization of services
Process	Organized/integrated delivery systems
Utilization	Enhanced access
Clinical perspective	
Medical care	
Process	
Quality	Regulation of professional performance
Outcomes	Outcomes assessment and management
	Practice guidelines
	Performance monitoring systems

Healthy People 1990 initiative and moved on to Healthy People 2000 and Healthy People 2010 objectives. This initiative has addressed a range of health determinants and has identified health promotion, health protection, and preventive services objectives but has not explicitly identified strategies for achieving them.

In the United States, the Healthy People 2010 objectives, described in detail in Chapter 1, serve as the national-level performance-monitoring system for population health (see Appendix 1.1). Specific policy strategies relate to the various determinants of health, with the nonmedical ones also covered under health indicators such as environmental quality. Health policy strategies directed at the environment include regulation of ozone exposure and environmental tobacco smoke. Health policy strategies directed at individual behavior include lifestyle-

oriented strategies such as responsible sexual behavior and substance abuse. Health policy strategies directed at human biology include indicators such as immunizations and obesity.

These various strategies are included in one way or another in the core functions of public health. Hence, the establishment of public health itself represents a commitment to a major population health policy strategy (IOM 1988). As described in Chapter 1, public health has become a significant enterprise at the state, county, and city levels but may be viewed as now facing a crisis of identity in the medical, and increasingly managed care–dominated, healthcare and health policy environment.

Another large set of policy strategies has focused on medical care services as the means of improving population health. These strategies relate to the elements of both the structure and process of care delivery. The predominant strategy focused on investment in the structure of healthcare delivery related to the efficacy, quantity, distribution, and organization of medical care resources through such programs as the National Institutes of Health (NIH), Hill-Burton, Health Professions Educational Assistance, and Comprehensive Health Planning.

Early federal policy in this direction was reflected in the establishment of NIH with its mandate to fund biomedical research as the means of developing the knowledge base for understanding the causes and treatment of diseases—for example, for addressing the improvement of the efficacy of medical care. This effort began with the establishment of NIH from the Marine Hospital Service Hygienic Laboratory in 1930. The effort expanded to a broader biomedical research focus with the establishment of the National Cancer Institute in 1937, which was integrated into NIH along with the National Heart Institute, established in 1948 (Harden 1986). In an example of one of the great ironies of public policy, it was out of the public health service, a policy strategy clearly anchored in the population perspective, that NIH arose and became such a dominant force for the clinical perspective.

Such a successful research policy was ultimately challenged to become more practical and accountable. Accordingly, the new federal research emphasis went a step further in disseminating the results of this research into practice through the establishment of the Regional Medical Program and, later, the Consensus Development Program. The Regional Medical Program—also known as A National Program to Conquer Heart Disease, Cancer and Stroke—was established in 1965 to bring, among other

things, the results of the biomedical research to the practice of medicine through such vehicles as continuing medical education (Komaroff 1971). The Consensus Development Program, beginning in the mid-1970s, represented an additional effort to translate the research findings into medical practice. One of the problems a practitioner faces is distilling the enormous amount of research into specific medical practices. To assist in this process, NIH began a process of convening consensus development conferences to bring about this synthesis and to provide medical practitioners with more specific guidelines based on the research (Burtram 1994).

A second major structural policy strategy sought to improve the health of the population through increasing the quantity of medical care resources. The Hill-Burton legislation, enacted in 1946, enhanced the number as well as the distribution of hospitals, while the Health Professions Educational Assistance Act of 1963 increased the numbers of doctors, nurses, and other health professionals.

A third major structural policy thrust, embodied in the Comprehensive Health Planning and Regional Medical Programs, addressed the distribution and organization of medical care through regionalization (Bodenheimer 1969) and the intentional building of comprehensive healthcare systems (Kissick 1970) or organized/integrated delivery systems (Shortell et al. 1993, 2000a, 2000b, 2001). The Comprehensive Health Planning legislation, enacted in 1966, provided grants for both state- and areawide health planning, while the Regional Medical Program legislation, enacted in 1965, fostered the development of a technical infrastructure for integrated delivery systems (Kissick 1970). These programs and subsequent private-sector efforts attempted to put together healthcare services across the continuum of need to improve both the effectiveness and the efficiency of services. Organized delivery systems essentially represent networks of organizations that provide or arrange to provide a coordinated continuum of services to a defined population and are willing to be held clinically and fiscally accountable for the outcomes and the health status of the population served (Shortell et al. 1993, 2000a, 2000b, 2001).

A number of efforts in the direction of increasing the distribution and organization of services were also targeted at special populations, thus increasing access to medical care. Maternal and child health programs, state and local health departments, Medicare, Medicaid, and the Office of Economic Opportunity Neighborhood Health Centers

are examples. Some of these examples are discussed in Chapters 6 and 7 in the context of achieving equity.

Clinical Perspective

An alternative set of health policy strategies has focused on improving clinical effectiveness and includes both regulation of professional performance, a principally process-oriented strategy, and outcomes assessment and management, an outcome-oriented strategy. A burst of activity following the establishment of Medicare in 1965 sought to improve professional performance through monitoring the quality of medical care. The first federal effort in this direction was the Professional Standards Review Organization (PSRO) in 1972. This effort mandated professional review of medical care but allowed the review to be delegated to the local institutional level. When this proved unsatisfactory over time, the Health Care Financing Administration mandated state-level professional review organizations.

The establishment of the Agency for Healthcare Research and Quality (AHRQ) in 1989 as the flagship agency for outcomes research, and its subsequent development and dissemination of practice guidelines, is focused on the outcomes rather than just the process of care. Besides practice guidelines as an operational strategy at the patient level, outcomes assessment and management has also been responsible for the development of performance monitoring at the institution and system levels.

Practice guidelines are "systematically developed statements to assist practitioner and patient decisions about appropriate healthcare for specific clinical circumstances" (IOM 1992, 27) that are expected to reduce inappropriate care, control geographic variations, and improve efficiency (Woolf 1990). They are known by a variety of names: clinical guidelines (AHRQ), practice standards (Brook 1989), clinical practice guidelines (American Academy of Medical Directors), practice policies (Eddy 1990), practice parameters (American Medical Association), medical necessity guidelines (American College of Physicians), clinical indicators (Joint Commission on Accreditation of Healthcare Organizations [JCAHO]), and consensus statement guidelines (NIH).

One of the most systematic efforts has been the development of practice guidelines by AHRQ, which has developed and widely disseminated clinical guidelines for the treatment of acute pain management, urinary incontinence in adults, pressure ulcers in adults, cataracts in adults,

depression in primary care, sickle cell disease, early HIV infection, benign prostatic hyperplasia, management of cancer pain, unstable angina, heart failure, otitis media with effusion, quality mammography, acute low back problems, post-stroke rehabilitation, cardiac rehabilitation, and smoking cessation. Patient, provider, and researcher versions for the clinical practice guidelines can be found at the agency's web site (AHRQ 2003). AHRQ has also launched the Evidence-Based Practice Centers (EPCs) Initiative to assist other organizations in developing guidelines. This program began in 1997, and 13 centers were funded in the 2002 round. The EPCs study topics relevant to clinical, social science/behavioral, economic, and other healthcare organization and delivery issues—specifically those that are common, expensive, and/or significant for the Medicare and Medicaid populations—and develop evidence reports and technology assessments.

The outcomes assessment and management effort has also fostered the development of performance-monitoring systems. The Joint Commission (2003) has defined a performance-monitoring system as "an interrelated set of process measures, outcome measures, or both, that facilitates internal comparisons over time and external comparisons of an organization's performance." Performance-monitoring systems are being sponsored by state government, employer, federal government, professional, and provider organizations. One of the most prominent of these systems is the Health Plan Employer Data and Information Set (HEDIS) developed by the National Committee for Quality Assurance. "HEDIS is . . . a set of standardized performance measures to assure that purchasers and consumers have the information they need to reliably compare the performance of managed health care plans . . ." (NCQA 2003).

The six performance domains of HEDIS include effectiveness of care, accessibility and availability of care, satisfaction with the experience of care, stability of the health plan, use of services, and health plan descriptive information. The 2004 edition of HEDIS substantially expands the scope of the tool that defines how health plans evaluate themselves in key areas of clinical care and customer service.

> Seven of the 10 new measures address major public health issues: osteoporosis, urinary incontinence, colorectal cancer, appropriate use of antibiotics (2 measures) and chemical dependency (2 measures). In addition, HEDIS 2004 included 3 measures that

track performance on key aspects of customer service: the timeliness of claims processing and the performance of health plan call centers (2 measures). (NCQA 2003)

The addition of these measures brought the total number of measures to 57 (NCQA 2003).

EVIDENCE RELATING TO EFFECTIVENESS

This section addresses the evidence related to effectiveness, first, by asking what the current health status of the population of the United States is and how it compares to other developed nations; second, by asking what general evidence exists supporting the importance of nonmedical determinants of health; and third, by asking what evidence exists on the effectiveness of the various health policy strategies outlined earlier.

Population Health Indicators

Through its Healthy People 2010 objectives, and in an effort to develop a consensus set of indicators to be used by each state in monitoring its progress, the U.S. Public Health Service has developed a set of 22 indicators of population health status (Office of Disease Prevention and Health Promotion 2003). As indicated in Appendix 1.1, these include measures of morbidity, access, health behaviors, substance use, and childhood outcomes. While progress is being made on most of the 22 indicators, many of the target rates have not yet been reached. In comparison with most other developed nations—most of which spend far less on healthcare—the United States ranked at the bottom on many of these indicators, specifically for infant mortality, total mortality, and work-related injury deaths (OECD 2003). Chapter 5 provides specific evidence comparing the United States with other countries.

A mixed picture of progress has developed for racial and ethnic population groups; significant health disparities between these groups and the white population continue to exist. In addition, Hispanics appear to be faring better than blacks. The ethnic disparities are highlighted in Chapters 1 and 2.

Major Determinants of Health

At the population level, many other important determinants of health besides medical care exist. The growing body of research on the fundamental determinants of health has resulted in a redefinition of the

importance of factors other than medical care as the determinants of health. Over the past two to three decades, a great deal of evidence has accumulated documenting the importance of both the physical and the social environment as determinants of the health of populations. (See a review of the population health and related social determinants perspective in Chapter 2.)

An estimated 60 to 90 percent of cancers are environmentally caused (Blumenthal 1985; Tomatis et al. 1997), with as much as one-third of cancer deaths being attributed to diet (Scheuplein 1992). Specifically, the causes of cancer have been estimated epidemiologically as diet, tobacco, infection, occupational exposures, and geophysical factors such as radiation. Environmental risks as a group include food contamination; food additives; water pollution; air pollution; indoor chemicals; occupational exposure; toxic wastes; carcinogens; radiation; and physical agents such as trauma, accidents, and noise (Tomatis et al. 1997). Besides cancer mortality, environmental factors cause nervous-system, endocrine-system, and immune-system problems as well as acute poisoning and birth defects (Misch 1994).

The social environment, reflecting social class and status hierarchies, income, social ties, and cultural change, has also been demonstrated to be powerfully influential in determining the health of population groups (Berkman 2000; Kawachi and Berkman 2001). Status hierarchy in work has been shown to be a major determinant of the health status of individuals in England (Singh-Manoux, Adler, and Marmot 2003). In addition, it has been demonstrated that income in both the United States and internationally relates positively to mortality and poor health status (Knesebeck et al. 2003). Disruptions in social and family ties due to death, divorce, or immigration and major cultural or social changes within a society are also clearly related to mortality (Eng et al. 2002).

Evidence About the Various Policy Strategies

The above evidence on the health of the U.S. population, as well as the general evidence concerning nonmedical determinants of health, suggests the need for a careful examination of evidence on the effectiveness of the various policy strategies that have been proposed or tried as means of improving the health of the population. These findings raise questions about possible ways to improve effectiveness, but the

answers proposed depend on the perspective—population or clinical—assumed. These perspectives lead to quite different proposals regarding the problems to be addressed and the solutions for doing so.

Population Perspective: Health Protection, Health Promotion, and Preventive Services

The population perspective focuses on evidence regarding the effectiveness of public health services in general and health protection, health promotion, and preventive services in particular in improving the health of the population.

McGinnis and Foege (1993) identified and quantified the major external (nongenetic) factors that contribute to death in the United States using a variety of data sources. The most prominent contributors to mortality in the United States in 1990 were tobacco, diet and activity patterns, alcohol, microbial agents, toxic agents, firearms, sexual behavior, motor vehicles, and illicit use of drugs, all of which together accounted for more than half of the deaths. Socioeconomic status and access to medical care were also found to be important contributors.

The effectiveness of health promotion and preventive services in general is somewhat mixed. The effectiveness of various prevention strategies (heart disease, HIV infection, substance abuse, and violence prevention) over a 15-year period in New York was reviewed by Freudenberg and colleagues (2000), who concluded that most programs reached a diverse population of low-income city residents, employed multiple strategies, reported a systematic evaluation, and adhered to at least some of the principles of effective health promotion. However, many programs did not involve participants in planning, intervene to change underlying social causes, or tailor the intervention for the subpopulations they targeted, thereby limiting their potential effectiveness. Glanz, Lewis, and Rimer (2002), in a review of cardiovascular disease interventions in communities, noted that each of these interventions showed only modest, and in some cases nonsignificant, reductions in risk factors and mortality that were obscured by the strong downward trends in the risk factors in control communities.

Thacker et al. (1994), of the Centers for Disease Control and Prevention, in a review of methods for assessing the effectiveness of preventive services, presented evidence substantiating the 95 to 98 percent effectiveness of vaccinations in preventing measles, the 20 to 70

percent effectiveness of mammography in preventing breast cancer deaths, and the 50 percent effectiveness of retinal screening and treatment in preventing blindness in patients with diabetes. Finally, Bunker, Frazier, and Mosteller (1994), using statistical estimation techniques based on clinical preventive services of demonstrated efficacy, concluded that hypertension and cervical cancer screening as well as childhood immunizations have contributed significantly to the increase in life expectancy over this century in the United States.

Population Perspective: Medical Care

Increasing investment in medical care has long been the strategy of choice for improving the health of the population, but the evidence of its effectiveness in achieving this goal varies across institutional settings. The evidence is presented in terms of the strategies listed in Table 3.1 under medical care from the population perspective.

Biomedical research as a policy strategy is ultimately directed at improving the efficacy of medical care. While the randomized clinical trial is the principal method for determining the efficacy of medical care interventions, the example that follows illustrates the creative use of a cross-sectional study design—in this case, observations over time—to assess the effectiveness of medical care given efficacious therapies for the conditions under consideration.

McKinlay, McKinlay, and Beaglehole (1989) examined the contribution of medical interventions to mortality changes in coronary heart disease, cancer, and stroke, which together account for two-thirds of total U.S. mortality and consume the vast majority of available resources. Using a combined measure of mortality and morbidity (the probability of a life free of disability), the authors demonstrated that overall life expectancy has increased over several decades because of medical interventions.

Investment in resources is the second major policy strategy related to the structure of medical care. This strategy has taken, for example, the form of investment in increasing the quantity of hospitals and doctors. The relationship between the quantity of resources and outcomes at the population level has been explored in studies with nonexperimental designs using cross-sectional observational data. This approach was used, for example, by Berlowitz et al. (1998) to examine outcomes in hypertensive men at five Department of Veterans Affairs sites in New England over a two-year period. The study found that blood pressure was poorly controlled in many veterans. Those who received more inten-

sive medical therapy had better control. Many physicians treating these patients were not aggressive enough in their approach to hypertension.

Health planning and regionalization of services is a third medical care policy strategy within the population perspective. It has been suggested that it is not merely the quantity of medical care resources, but their distribution and organization as well, that is important to the health of the population, and this has been the premise for policy strategies aimed at health planning and regionalization of medical care. Lattimore et al. (2003) evaluated the use of thrombolytic therapy before and after institution of such a center in a community hospital. The establishment of a primary stroke center at a community hospital resulted in a substantial increase in the proportion of patients receiving thrombolytic therapy for ischemic stroke, a finding that established the effectiveness of the center in potentially improving patient outcomes related to stroke.

Once the efficacy of procedures has been established, the effects of greater quantities of procedures on outcomes can also be examined, and studies doing so have been used as an argument for regionalization of surgical services. An example of such a study is one that examined the relationship between the number of surgical procedures done in hospitals and each hospital's mortality experience for those operations. Carey and colleagues (2003) analyzed California's discharge abstract database to analyze the relationship between annual procedural volume and outcomes of all 119 nonfederal hospitals performing cardiac surgery from 1997 through 1999. They found that in-hospital mortality related to coronary artery bypass surgery was much higher for hospitals performing fewer than 200 procedures per year compared to hospitals performing more than 500 procedures per year. However, many low-volume providers had patients with mostly excellent outcomes. The authors, therefore, concluded that although volume is clearly related to outcome, patient-related factors and process variables may be more important. The implication of such studies is that the effectiveness of medical care, in the case of surgical procedures, can be improved. One policy alternative for doing so is to regionalize surgery services for the procedures that require a high volume to maximize effectiveness.

Shortell and his colleagues examined structural and process variables related to functional integration, physician integration, clinical integration, and governance and management in a selected sample of integrated healthcare systems (Shortell et al. 2000a, 2000b, 2001). Their

set of studies, however, did not encompass health outcomes at either the system or the population level. Lacking this direct evidence, perhaps a brief consideration of performance evaluations of HMOs, which were early forms of integrated health systems, will provide some insights. Luft (1981), in his early review of HMO performance, did examine the then few outcomes studies and concluded that HMO outcomes were not very different from those of conventional practice. In later updates, Miller and Luft (1994, 1997, 2002) found that even considering that many more studies were conducted, the conclusion was still equivocal, meaning the outcomes were in general no better nor worse on average. The primary exception was negative outcomes for Medicare enrollees with chronic conditions noted in several studies.

Enhanced access is one of the policy strategies related to the process of care involving attempts to increase healthcare utilization for certain groups. Evidence relating to process and outcomes includes studies examining the relationships between the process variables of utilization, quantity of procedures and quality of care, and various outcome variables as the measures of effectiveness. A similar conclusion—that differences in utilization have a modest relationship, if any, to outcomes—can be drawn from studies focused on the clinical perspective. The RAND Health Insurance Experiment (Brook et al. 1983) provides an example of the examination of the effects of varying utilization rates on health outcomes. The study was undertaken to determine what influence various levels of copayment in a national health insurance scheme might have, primarily on utilization and secondarily on health status. The utilization examined included outpatient treatment and hospitalization for both adults and children. The clinical outcomes assessed were blood pressure and vision for adults and anemia, hay fever, hearing, fluid in the middle ear, and vision for children. The utilization differences were 33 percent greater for adults and 22 percent greater for children in the free-care plan versus the 95 percent copayment plan (Valdez et al. 1985). These utilization differences were accompanied by only slight differences in blood pressure and vision correction in the adults and no differences in clinical outcomes in the children.

An important caveat, however, is that it may not be valid to extrapolate these results to all population groups because substantial heterogeneity exists in health outcomes across different socioeconomic and racial groups as well as differences by gender and geography. This was

pointed out in the data on disparities in population health outcomes for blacks versus whites presented in Chapter 1. It was also confirmed in the RAND (Brook et al. 1983) and the Medical Outcomes studies (Nelson et al. 1998; Ware et al. 1996), which showed that restrictions, limitations, and managing care did not negatively affect average patients, but that the poor and the elderly were adversely affected.

The importance of this caveat is emphasized by studies of what are called avoidable or preventable hospitalizations. The premise of these studies is that there are identifiable hospital diagnoses that indicate an advanced stage of disease that could have been prevented by accessible primary medical care. By studying the occurrence of these preventable hospitalizations, several studies have shown that poorer populations without access to adequate primary medical care do more often become hospitalized for preventable conditions (Begley et al. 1994; Billings, Anderson, and Newman 1996; Bindman et al. 1995).

Clinical Perspective

Regulation of professional performance has only modest evidence to support its effectiveness. At the time when the first public regulation of professional performance, the PSRO program, was implemented, no consistent evaluation evidence existed to support the effectiveness of such a strategy (Slater and Bryant 1975). Later reviews have argued that many challenges still remain in trying to make quality monitoring effective (Blumenthal 1996; Feinstein 2002; McGlynn 1997; Lohr 1997; U.S. Congress OTA 1988).

One example of the relationship of quality to outcomes from the clinical perspective is an investigation of quality and variation in hospital mortality rates. Using a cross-sectional design and a hospital database, Dubois et al. 1987 examined the relationship between hospital mortality rates for three specific conditions—heart attack, pneumonia, and stroke—and two different measures of quality of care. They found that 64 percent of the variation in outcomes was explained by the severity of illness in patients admitted to these hospitals but that there was an association between poorer quality and mortality for one quality assessment method based on a subjective judgment of preventability of death. A subsequent study (Park et al. 1990) confirmed the finding of a modest association between the quality of medical care given and the subsequent death of individual patients.

Outcomes assessment and management suggests that practice guidelines and performance-monitoring systems, such as HEDIS, can improve the outcomes of medical care. Evidence for the effectiveness of clinical practice guidelines has been slowly developing. The Consensus Development Program, an example of practice guidelines, was assessed and found lacking by Kosecoff and her colleagues (1987), who investigated the effectiveness of this process for 12 consensus recommendations. They found little impact from any of the recommendations, although physicians were aware of them. More importantly, they observed that with regard to several of the guidelines, physician behaviors were changing even before the consensus statements were disseminated. Lomas et al. (1989), investigating the effectiveness of Canadian national guidelines for cesarean-section rates, found that while the majority of obstetricians had knowledge of and agreed with the guidelines and reported reducing their cesarean-section rates, actual practice had in fact changed little. In reviewing a number of studies, Woolf (1990) found similar results, as did Grimshaw and Russell (1993, 1317), who undertook an information synthesis of 59 evaluations of clinical practice guidelines, concluding that "explicit guidelines do improve clinical practice" when the focus is on the process of care, but that less than 20 percent of these studies had looked at the impact on outcomes.

Systematic evaluations of performance-reporting systems to assess their usefulness are lacking. However, selected examples of evaluations of single-focus programs (Blumenthal and Epstein 1996; Epstein 1995) have been examined, demonstrating mixed results. A review on performance-reporting systems about the performance of hospitals, health professionals, and healthcare organizations by Marshall and colleagues (2000, 1866) concludes,

> Seven U.S. (performance) reporting systems have been the subject of published empirical evaluations. Descriptive and observational methods predominate. Consumers and purchasers rarely search out the information and do not understand or trust it; it has a small, although increasing, impact on their decision making. Physicians are skeptical about such data and only a small proportion makes use of it. Hospitals appear to be most responsive to the data. In a limited number of studies, the publication of performance data has been associated with an improvement in health outcomes.

However, outcomes have not regularly been shown to change, but reporting behavior in the form of increased severity of illness for patients has changed. It is perhaps too early to find thorough evaluations of performance-monitoring systems, but health services research can contribute to their design and implementation and hence to determining whether such systems most directly contribute to improved outcomes for patients.

Summary

From the population perspective, variations in population health by race and other characteristics (described in Chapter 1) on the one hand, and geographic variations in care resources and procedures on the other hand, have only modest relationships to one another at the population level. Research in England (Marmot 1998; Singh-Manoux, Adler, and Marmot 2003) illustrates this point: improvements in the distribution of healthcare resources and evidence of better access by the poor did not contribute over time to reducing the disparities in health between members of different social classes. The reasons are complex but can be reduced in part to the assertions that the determinants of population health include more than medical care, and medical care is not a precise science. One implication of these findings about the effectiveness of medical care is that increasing the quantity of medical care resources and improving their distribution and access do not produce substantial improvements in population mortality or morbidity and appear unable to reduce the sociodemographic disparities in population health. Healthcare in the United States seems to have reached a point of diminishing returns in improving the health of the population. This evidence is reviewed in greater detail in Chapters 4 and 5.

In summary, while it may be true that further investments in medical care will not improve the health of the population, it does not follow that the same is the case for particularly vulnerable populations, such as the poor and the elderly. An important point, however, is that if health policy is intended to improve the health of the population as a whole, it may be better directed at nonmedical contributors to population health, such as public health–oriented health-protection and health-promotion strategies.

From the clinical perspective, despite massive and expensive efforts to improve medical care effectiveness through professional performance regulation and outcomes assessment and management, there is little evidence to date of the success of these efforts. Of the various pol-

icy strategies for improving effectiveness, including enhancing public health services, health promotion and disease prevention, biomedical research and its dissemination, increasing the investments in medical care resources, improving the distribution and organization of medical care, regulation of professional performance through quality assessment, enhanced system integration, and outcomes assessment and management, the last has become the dominant focus of federal policy on effectiveness, as reflected in the research and policy agenda of AHRQ.

CRITERIA FOR ASSESSING POLICY ALTERNATIVES IN TERMS OF EFFECTIVENESS

The conceptualization by Shortell, Gillies, and Devers (1995) of the Community Health Care Management System, reflecting both the population and clinical perspectives as well as community, system, and institution levels of healthcare, provides the conceptual grounding for a set of criteria for judging effectiveness. Such a system, they suggest, begins with the assessment of needs on a community level, proceeds to the development of resources and services across the continuum of care to meet those needs, develops guidelines and protocols to guide the care, and then suggests a monitoring system to ensure that the needs are met. Specific effectiveness criteria and examples related to breast cancer screening and treatment are highlighted in Table 3.2 and the discussions that follows.

Population Effectiveness Criteria

1. The health policy option should be based on the results of a community health–needs assessment. This assumption implies that (1) there should be a community health–needs assessment from which information on policy options are derived and (2) a population-based, community-level health information system should be in place to guide health policy development for the population. The Evans, Barer, and Marmor (1994) model provides a framework for such population information and POPULIS (Roos et al. 1996, 1999) serves as a concrete example of such a system. The majority of population databases related to breast cancer in the United States contain patients with cancer (Surveillance Epidemiology and End Results system) or individuals with selected types of coverage (Medicaid, Medicare) (CMS 2002; National Cancer Institute 2003).

Table 3.2 Criteria for Assessing Health Policies in Terms of Effectiveness

Dimensions	*Criteria*	*Indicators*	*Examples*
Population Effectiveness			
Need based	Based on the results of a community health needs assessment	Population health information system	The majority of population databases related to breast cancer in the U.S. contain patients with cancer (SEER) or individuals with selected types of coverage (Medicaid, Medicare).
Comprehensiveness	Reflects an appropriate relationship to the continuum of healthcare services	Full continuum of services	Discontinuities often exist between the systems and services for breast cancer screening and the availability and coverage for breast cancer follow-up and treatment services, if needed, based on screening (e.g., National Breast and Cervical Cancer Early Detection Program).

2. The health policy option selected should reflect an appropriate relationship to the continuum of healthcare services. The resources and services for maintaining and improving health need to be integrated across the entire continuum of care, including health promotion and disease prevention, and any specific policy option needs to be clearly related to this full continuum. The continuum of services displayed in

Table 3.2 Criteria for Assessing Health Policies in Terms of Effectiveness *(continued)*

Dimensions	*Criteria*	*Indicators*	*Examples*
Clinical Effectiveness			
Precision	Specifies in advance expected guidelines for structure and process	Practice guidelines	Wide variability exists in the practice guidelines for mammography screening across agencies and organizations (e.g., American Cancer Society, American Geriatrics Society, National Cancer Institute, U.S. Preventive Services Task Force).
Performance	Monitors process and outcome indicators for selected conditions	Performance-monitoring system	Performance-monitoring systems are often proprietary or limited to members of selected health plans (e.g., HEDIS).

Figure 1.1 illustrates the range of health services that needs to be considered. Discontinuities often exist between the systems and services for breast cancer screening and the availability and coverage for breast cancer follow-up and treatment services, if needed, based on screening. For example, the Breast and Cervical Cancer Prevention and Treatment Act of 2000 provided funds to states for full Medicaid benefits for women screened through the National Breast and Cervical Cancer Early Detection Program (NBCCEDP) who need cancer treatment. However, the proportion of women eligible for NBCCEDP who

actually use it is low (~18 percent) because funds are insufficient to expand services to eligible women (CDC 2003).

Clinical Effectiveness Criteria

Shortell's framework also leads to a set of criteria for evaluating, from a clinical perspective, health policy reform options.

1. Precision of medical care will be fostered by the specification in advance of guidelines for clinical performance. Such practice guidelines, protocols, or practice parameters can reduce the uncertainty in medical care and can contribute not only to improved effectiveness but also to enhanced efficiency. Wide variability exists in the practice guidelines for mammography screening across agencies and organizations, for example, the American Cancer Society, the American Geriatrics Society, the National Cancer Society, and the U.S. Preventive Services Task Force. (See Table 9.1 and related discussion in Chapter 9.)

2. Performance of medical care can also be improved through the monitoring of process and outcomes indicators for selected clinical conditions. JCAHO- and HEDIS-type process and outcome indicators would appear to be the measures of choice. These include such items as preventive services screening rates, seniors' health status, and satisfaction with care. Performance-monitoring systems such as HEDIS are, however, often proprietary or limited to members of selected health plans, and it is therefore difficult to obtain comprehensive, objective data across an array of clinic populations (NCQA 2003). The Community Quality Index Study, conducted by the RAND Corporation, with support from the Robert Wood Johnson Foundation, does however represent an effort to gather more representative data. In that study a national sample of more than 13,000 adults in 12 metropolitan areas were interviewed by phone regarding their healthcare experiences as the basis for the *National Report Card on Quality of Care* that is to be routinely compiled and made available. Around half (6,700) of the respondents provided written consent to review their medical records to evaluate performance on more than 400 clinical indicators of quality, including breast cancer screening and treatment (McGlynn et al. 2003).

These criteria, along with indicators of their presence and adequacy, are summarized in Table 3.2. The presence of a population health information system is taken as an indicator of the possibility of population needs–based assessment. The existence of the full continuum of

services indicates comprehensiveness. Practice guidelines indicate attention to precision, while a performance-monitoring system indicates a focus on performance. The effectiveness analysis of breast cancer screening in Chapter 9 will focus in particular on the clinical perspective underlying guidelines development and performance monitoring.

SUMMARY AND CONCLUSIONS

This chapter has focused on the question, What policy strategies contribute most to improving the health of the population? The evidence reviewed from the population perspective suggests that while the point of diminishing returns from further investments in medical care with regard to improving the health of the population may have been reached, a case should still be made for investments in medical care for improving the health of vulnerable population groups. The health of populations in general, as well as at-risk groups in particular, is most likely to be enhanced, however, by focusing more resources on nonmedical determinants of health, such as the physical, social, and economic environments in which individuals live and work. Chapters 4 and 5 provide arguments and evidence that economic, as well as health, benefits may be yielded as a consequence.

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Chapter 4

Efficiency: Concepts and Methods

CHAPTER HIGHLIGHTS

1. *Allocative efficiency* depends on attainment of the "right," or most valued, mix of outputs. Primary health policy areas that reflect concerns with allocative efficiency include decision making regarding investments in medical versus nonmedical policy alternatives; coverage of preventive services; and the mix or types of treatment, in relationship to health improvements.
2. *Production efficiency* refers to producing a given level of output at minimum cost. For example, inefficiency occurs when physicians provide services that could be provided just as well by nurses or other, less expensive, health personnel and when practice does not take advantage of economies of scale, as in the production of laboratory services.
3. Societies have developed both *need* and *consumer-demand* mechanisms for making healthcare resource allocation decisions. Need exists when someone is better off with a service than without it; consumer demand refers to what consumers are willing and able to buy at alternative prices. Need primarily undergirds regulatory-based approaches, and consumer demand underlies market-based approaches to resource allocation.
4. Analysts use cost-effectiveness analysis, cost-utility analysis, cost-benefit analysis, and comparative systems analysis to examine production and allocative efficiency issues in healthcare.

OVERVIEW

The fundamental questions in this chapter related to assessing the efficiency of healthcare are (1) What is efficiency? and (2) How might efficiency be measured?

The concepts and methods of efficiency research provide guidance for societal decision making regarding the combination of healthcare goods and services to be produced with society's limited resources and the ways in which these goods and services are to be produced, as well as whether the maximum value is being achieved in terms of improving the health of the population relative to the costs required to produce these goods and services.

All modern societies allocate a large portion of their wealth to the provision of healthcare services. The United States leads the world both in the level of healthcare spending and in efforts to study the problems of access, quality, and cost of healthcare. In 1960, 5.1 percent of the U.S. gross domestic product (GDP) was spent on healthcare. By 2001, it had increased to 14.1 percent (NCHS 2003, 306). Large variations in medical practice; evidence on the possible lack of effectiveness of many medical services; and the renewed interest in disease prevention, health promotion, and nonmedical determinants of health suggest that the allocation of healthcare resources is not efficient (Deaton 2002; McGlynn et al. 2003; OECD 2002a).

In aggregate terms, both the efficiency and equity of the U.S. healthcare system compare unfavorably to Canada and several western European countries (Cutler 2002; McClellan, Kessler, and the TECH Investigators 1999). Analysts in the United States have examined those countries both to obtain points of reference for U.S. problems and to gain insight into possible solutions. Despite their relatively low expenditures and broad coverage, other countries also perceive severe problems with their healthcare systems and look to the United States for innovative healthcare delivery and financing systems. These countries look in particular to the extensive U.S. health services research base on the effectiveness, efficiency, and equity of alternative healthcare systems.

Because of the concern about past and projected public and private payer budgets for healthcare, macroeconomic cost control is a major goal of developed countries (Cutler 2002). Although it is not necessary to restrain the percentage of GDP spent on health simply because it is high or growing rapidly, the major issue is whether the services are

worth the costs. Ideally, cost containment would first be achieved by eliminating spending on services that were detrimental to, or had no effect on, patient health status. If further reductions were required, services would be ranked and funded according to their yield in health improvement per dollar. Research on the efficiency of health services delivery does provide some guidance, however, in making these decisions. Oregon has, for example, used effectiveness and cost-effectiveness evidence to ground the design of a system for efficiently rationing healthcare (Goldsmith 2003), although a substantial policy debate has surrounded the precise mechanisms for making these technically and ethically difficult decisions (Eddy 1991; Hadorn 1991; Tengs 1996).

Robert Evans and his colleagues have suggested that healthcare spending has risen to the point where it may actually cause a decline in the health of the population because it draws resources from areas such as education, housing, and the environment that provide a positive contribution to health and applies them to medical services that have low, no, or even negative effects on the health of the population as a whole (Evans, Barer, and Marmor 1994; Evans and Stoddart 1990, 2003). Others focus on the benefit versus the cost of modern medicine, the willingness of consumers to pay for those benefits, and the importance of closing distributional gaps in access to care (Chernew, Hirth, and Cutler 2003).

Thus, the questions of how much to spend on healthcare, what healthcare services to provide, and how to provide them are important policy issues. Because of the nature of health and healthcare, the status of healthcare as a good that many feel should be available regardless of one's ability to pay, the impingement of healthcare costs on public budgets, the lack of information, and other healthcare market imperfections, the solution cannot simply be left to the operation of the private market. There is a constant search for a better understanding of these problems and of the operation of the healthcare system and for policies that will improve the access, cost, and quality of healthcare.

The tools of efficiency analysis can assist in formulating these policies. This chapter introduces these tools and the theoretical underpinnings for each in the context of addressing the first question posed at the beginning of the chapter, What is efficiency? In particular, the concepts of allocative and production efficiency will be presented and defined, and the theoretical basis for need and market-demand criteria for making resource allocation and production decisions will

be discussed. The role of production functions; cost-effectiveness, cost-benefit, and cost-utility analysis; and international comparisons will be examined in addressing the second question, How might efficiency be measured? Chapter 5 (1) describes the broad approaches and specific means by which various countries have attempted to achieve efficiency goals and (2) reviews selected evidence on the performance of those policies with respect to efficiency.

CONCEPTUAL FRAMEWORK AND DEFINITIONS

For society as a whole, efficiency requires that the combination of goods and services with the highest attainable total value, given limited resources and technology, be produced (Folland, Goodman, and Stano 2001). This requires attainment of both allocative and production efficiency. *Allocative efficiency* depends on attainment of the "right," or most valued, mix of outputs (Davis et al. 1990). *Production efficiency* refers to producing a given level of output at minimum cost. As implied in the conceptual framework introduced in Chapter 1 (Figure 1.4), improving the health of communities and individuals is the desired and valued endpoint, or output, of societal investments in health programs and policies. These decisions have important implications for the equity of healthcare provision as well, based on both the fairness and the effectiveness of the allocation of resources to achieve desired health outcomes (Culyer 1992; Cutler 2002).

Allocative Efficiency

Where healthcare is viewed as an input in the production of health improvements, the focus is on allocative efficiency (i.e., maximizing health given constrained resources). Allocative inefficiency may occur even in a production-efficient health system if the system produces too many or too few services relative to health improvements. Allocative efficiency problems arise in healthcare delivery, for example, when substantial resources are allocated to treatments of questionable effectiveness while proven prenatal screening and other preventive services are neglected. Primary health policy areas that reflect concerns with allocative efficiency include decision making regarding investments in (1) medical versus nonmedical policy alternatives; (2) coverage of preventive services; and (3) mix or types of treatment, in relationship to health improvements.

Medical Versus Nonmedical Alternatives

In a broader context of health-oriented social policy, a society may achieve much greater health benefits by diverting resources from healthcare to activities that improve the physical and social environment, for example, air and water quality, education, job training, and community development (Evans, Barer, and Marmor 1994). Studies have documented that the marginal product of healthcare in the United States is small for the population as a whole but may be higher for selected population groups such as the elderly (Folland, Goodman, and Stano 2001) and for specific services such as treatments for cataracts, heart attacks, and depression (Cutler and McClellan 2001). Lifestyle factors, on the other hand, have been found to be major and significant predictors of population health status, as has education. One theory (Grossman 1999) has proposed that schooling improves the efficiency with which one produces one's own health; better-educated people know what is needed to stay healthy and know how to use medical and other inputs, as well as their time, to produce better health (Behrman and Wolfe 1989; Berger and Leigh 1989; Gerdtham et al. 1999; Wolfe and Behrman 1987). These findings present interesting challenges to state policymakers in particular, who may be confronting significant trade-offs in deciding the relative allocation of state tax dollars to Medicaid versus public education.

Preventive Services

There is concern within the healthcare sector that too much is spent on the treatment of cases for whom health improvements or survival are remote and that too little is spent on preventive services, especially populationwide approaches to health improvement such as reduction in air pollution and reductions in poverty (Hoover et al. 2002; McGinnis, Williams-Russo, and Knickman 2002; Scitovsky 1988; Waldo and Lazenby 1984; Webster and Berdes 1990). Studies of effectiveness and cost-effectiveness have been used in selecting preventive services to be covered by public and private insurance (Eddy 1980; Gold et al. 1996; Pear 1997; U.S. Preventive Services Task Force 1989). Only a very small percentage of national health expenditures has typically been allocated to preventive activities.[1] There has, however, been a shift toward greater reimbursement and provision of preventive services among federal and private payers. The growth of health maintenance organizations, which

typically provide a broad range of preventive care, was a major contributor to this shift (NCHS 2003, 339).

HMOs provide more coverage for preventive services compared to other health plans (Center for Studying Health System Change 2000; Luft and Greenlick 1996). Staff- and group-model HMOs with lower disenrollment rates have a further incentive to encourage preventive services with potential for long-term benefits. While HMOs may offer increased preventive services, however, studies also recognize the existence of appointment delay, busy schedules, and other barriers to implementation of both primary and secondary prevention services (Kottke, Brekke, and Solberg 1993; Thompson 1996).

Mix or Types of Treatment

There is also policy concern about the appropriate and efficient mix or types of services delivered in treating patients. One area of concern is the misallocation of resources to technical procedures and away from services that improve patients' understanding of their health problems and of ways they can ameliorate and possibly avoid health problems in the future. Because of the malpractice and fee-for-service reimbursement systems, physicians have been induced to perform procedures such as surgery and diagnostic tests and to spend less time taking histories and providing cognitive services to patients (e.g., health education or motivational counseling). Extensive testing provides documentation to use in the case of a medical liability lawsuit. Tests and other procedures also provide much higher remuneration per unit of time compared to cognitive services (Hsaio et al. 1988). This problem is exacerbated by physician ownership of diagnostic equipment, laboratories, and specialty hospitals (Center for Studying Health System Change 2003a; Hillman et al. 1990). Given that most diseases have a strong behavioral component, one of the most important and potentially effective aspects of patient care—patient education and counseling—may be neglected due to this concentration on procedures.

Production Efficiency

Health is viewed as the final output and health services the intermediate output of the healthcare system. Production efficiency (i.e., producing output at the least cost) is of concern for both intermediate and final outputs. Production efficiency addresses whether resources are organized and managed in a manner that minimizes the cost of pro-

duction, as well as whether personnel, supplies, and equipment are paid for at rates that represent their cost in their next-best alternative use. Inefficiency occurs when care is not managed in a way that maximizes potential productivity. For example, inefficiency occurs when physicians provide services that could be provided just as well by nurses or other less expensive health personnel and when practice does not take advantage of economies of scale, as in the production of laboratory services. These concepts of efficiency are relevant at the level of the individual patient and practitioner and at institution, system, and community levels (see Table 1.1 in Chapter 1).

Figure 4.1 displays combinations of goods and services that could be produced with society's resources during a given period of time. Within the figure, the curve AB represents the production possibility frontier. Points on the curve represent the maximum possible output of all goods and services, given current technology and the most efficient production methods. If actual production is inside the curve, as at point C, production efficiency is not being achieved. Within the shaded area, improvements in production efficiency could expand healthcare without reducing output of other goods and services, and vice versa. However, from any point on the frontier, expansion of one commodity is at the expense of the other. Thus, allocative decisions must be made in terms of the tradeoff between healthcare and other goods and services.

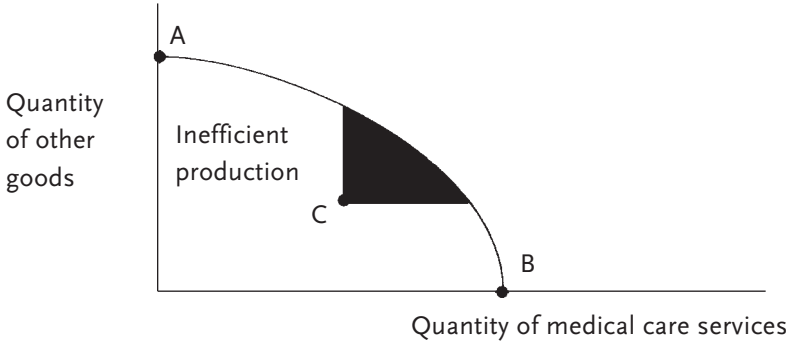
The production possibility frontier only illustrates that alternative combinations are possible. It does not identify the most efficient combination. Resource allocation is a complex, dynamic process that depends on a mix of private spending decisions and government tax and spending decisions. With a growing economy and technological base, the frontier is continually expanding, with technology itself as the focus of concern. The well-being of society is subject to decisions about the allocation of resources to technology, to healthcare, and to other goods and services.

Both allocative and production efficiency focus on providing guidance for what might be an optimal allocation of resources and associated costs relative to desired outputs (e.g., health and healthcare). The theoretical underpinnings for determining the optimal distribution of resources to produce these desired ends are described in the next section.

Criteria for Optimal Allocation

Examples of resource misallocation and inefficient production of healthcare can be documented (as will be shown in Chapter 5). Nonetheless,

Figure 4.1 Production Possibility Frontier



the optimal allocation of resources and production methods is not known. Three major problems confront analysts and policymakers attempting to evaluate healthcare resource allocation issues. The first problem is limited theoretical and empirical information on how to analyze the effects of resource-allocation decisions on social well-being. Social well-being or social welfare is simply the sum of individual utilities attained by individuals in society (Stokey and Zeckhauser 1978). Second is the related problem of limited information on the relationship between healthcare utilization and health. Third, both market and regulatory systems have proven to be highly imperfect mechanisms for allocating resources in the healthcare sector of the economy.

Philosophers have long sought to develop theories and practical guides to define and measure social welfare. Vilfredo Pareto (1848–1923)[2] provided much of the underpinnings of welfare economics, a collection of analytic devices and concepts for evaluating resource allocation decisions. Central to this work is the Pareto optimum, which occurs when all mutually beneficial exchanges have been made such that no one person can be made better off without making someone else worse off. With freedom to trade, rational individuals or their proxies make all trades that they believe benefit them. However, there are many possible Pareto optimum allocations, depending on the distribution of income. Identifying and achieving the one that maximizes social well-being involves trade-offs between winners and losers and knowledge of a social welfare function.

A *social welfare function* describes a decision maker's preferences among alternative combinations of individual utilities (Stokey and Zeckhauser 1978). It describes how the decision maker would trade off gains in utility by some people for losses by others. For example, how is social welfare affected by allocating fewer state dollars to public education and more to Medicaid for low-income families? To answer this question requires that individual preferences be combined and aggregated to provide a ranking of welfare, or likely public benefit, for society as a whole.

Arrow (1963) has demonstrated that development of such a function at the overall societal level is not possible. It has been shown in theory, however, that competitive markets can yield a Pareto optimum (Stokey and Zeckhauser 1978). Informed rational consumers make mutually beneficial trades, and competition forces producers to seek efficient methods of production and to respond to consumer preferences.

Given the uncertainty, complexity, and importance of healthcare, societies have developed mechanisms for making resource allocation decisions. These include *need*, which primarily undergirds regulatory-based approaches, and *consumer demand*, which underlies market-based approaches.

Need

Need as defined by health professionals has formed the basis for government-imposed approaches to healthcare resource allocation. Need for medical care exists when someone is better off with a treatment than without it, and the improvement is measured in terms of a person's health (Jeffers, Bognanno, and Bartlett 1971; Williams 1974). Therefore, unless healthcare professionals deem a treatment to be effective and the patient values its outcome, the treatment is not "needed."

While need is a useful concept for determining the care patients require, there are severe conceptual and practical problems with using need as a basis for resource allocation. First, there is no objective basis on which to rank health needs and to compare them with other needs of individuals and populations. Second, even with this restrictive definition, needs appear to be insatiable and thus still require rationing. When some needs are met, the healthcare industry defines new areas not previously addressed by medicine. Third, the relationship between providing healthcare services and reducing health needs is often unclear

if not nonexistent (see Chapter 3 for a detailed discussion of this issue). Fourth, resources provided to meet need as defined by health professionals or government agencies may go unutilized because the population does not demand them (e.g., preventive services) (Feldstein 1998).

Consumer Demand

Consumer demand—what consumers are willing and able to buy at alternative prices—is another important criterion for allocating resources. Need, as perceived by the consumer, is a major but not the sole determinant of demand for healthcare. Rational consumers compare the marginal benefit and marginal cost associated with alternative uses of their limited money and time resources and make allocation decisions in their own best interest.[3]

Consumer demand, as the basis for allocational decisions, underlies much of economic theory. The concept of demand is represented in Figure 4.2 as a demand (D) curve. It shows the quantities of a good or service—for example, routine doctor visits (horizontal axis)—that an individual is willing and able to purchase at alternative prices (vertical axis) during a given period of time. Consumers are assumed to be well informed about prices and services and to attempt to make choices that maximize their well-being. A host of factors affect the position and slope of the demand curve, including consumer income, preferences, need, and the prices of other related goods and services. The typical demand curve is downward sloping because (1) as price falls, consumers are able to buy more; (2) the service is less costly relative to other substitute services (i.e., services that serve the same ends, such as outpatient and inpatient surgery for minor problems); and (3) the marginal value of the service to the consumer falls as more is consumed in a given period of time. The demand curve represents the marginal value of the service to the consumer at alternative levels of consumption (Q), and the market price (P) represents the marginal cost of the service to the consumer. By consuming at the level (Q') corresponding to that level at which a given price (P') intersects the demand curve (point E'), the consumer maximizes well-being. For quantities of doctor visits that exceed Q', given the price P', marginal cost is greater than marginal benefit (D), making the consumer worse off.

Market demand is merely the aggregation of the individual demands of market participants. While demand is an individual concept and depends on individual behavior, it is aggregations of individuals that

Figure 4.2 Individual Demand Curve

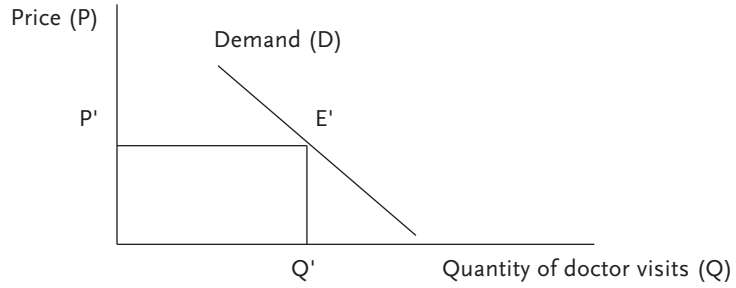
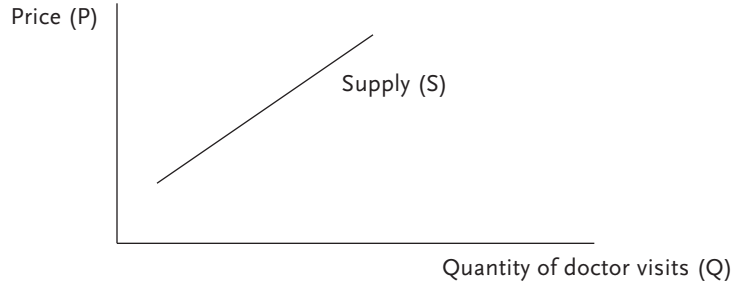


Figure 4.3 Individual Supply Curve



form markets. Prices and quantities of goods and services are then determined by the operation of supply and demand in markets.

Assumptions of a Competitive Market

In a competitive market, supply represents the amount of a good or service that suppliers are willing to sell at alternative prices during a given period of time (Figure 4.3). The curve is positive or upward sloping, meaning that greater quantities are supplied at higher prices. The position of the supply curve depends on technology (i.e., the ability to transform inputs into output), the prices of inputs such as wages and rents, and the objectives of suppliers (i.e., whether they are attempting to maximize profits or services or some combination). For example,

technological innovation in electronics has markedly increased productivity and allowed producers to offer the same products at lower prices, a shift to the right in the supply curve. Similarly, increases in wages and other input costs would require higher prices for the same number of units, resulting in a shift to the left in the supply curve. Market supply is the aggregation of individual supply of market participants.

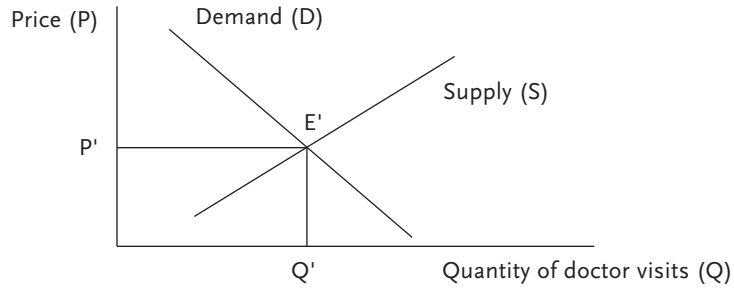
The intersection of market supply and demand determines the equilibrium (E'), market price (P'), and quantity of services (Q') for a given period of time (Figure 4.4). This is the model that undergirds the market approach to healthcare reform. As an application of theory, it is a simplification of reality, and any application must deal with the disparities between the model and the real world of healthcare. Consumer choice and self-regulating market forces are assumed in such a model.

By rapidly adjusting to changes in consumer preferences, incomes, resource scarcity, and technology, competitive markets generally provide a flexible mechanism for solving the basic economic problems of what, how, and for whom a good or service is produced. For many goods, and possibly for routine healthcare, consumers appear to be the best judge of their needs and desires relative to other uses of their resources.[4] Under competitive market conditions, producers who fail to respond to consumer demand, who charge prices above the market rate, or who use inefficient production methods are forced out of business, and consumers individually allocate resources to maximize their own well-being, leading the system toward a Pareto optimum allocation of resources.

Assumptions of the Healthcare Market

Healthcare, however, diverges from some fundamental properties of a perfectly competitive market. See Rice (2003) for a thorough critique of the use of competitive markets to achieve healthcare goals for society. The basic conditions of a competitive market are (1) free entry to and exit from the market by buyers and sellers; (2) many well-informed buyers and sellers, no one segment of which is large enough to influence market price; and (3) no collusion among buyers and sellers, that is, they act independently. Many healthcare market areas are too small to support competition, especially for services of specialists and hospitals. Historically, the market has been characterized by price discrimination and collusion, ostensibly to protect consumers and provide access for those who cannot pay. Asymmetry of information puts con-

Figure 4.4 Market Demand and Supply



sumers at a disadvantage vis-a-vis providers, and entry by providers is strictly limited by licensing and regulation of the professions and facilities (Fuchs 1972; Kessel 1958; Rice 2003).

The competitive model therefore does not fully apply to healthcare because of several inherent market limitations. There are significant externalities (i.e., instances in which one person's consumption or production affects another person's well-being). For example, a person who obtains immunizations to prevent infectious diseases provides benefits to others by reducing their risk of contracting the disease. Private markets tend to underinvest in these types of services because benefits to third parties are not directly incorporated in market demand by those who seek services. Similarly, people seem to care that others have access to basic healthcare and therefore benefit when others gain access to care that would otherwise not be available. Markets alone have no mechanism for translating this value into the desired result.

Another problem with a private healthcare market is so-called supplier-induced demand—that is, the lack of independence between demand and supply. Provider interests may affect consumer demand because of the large disparity of information between provider and consumer and the fact that a third party often pays for a substantial portion of services rendered (Grytten and Sorensen 2001; Reinhardt 1987). Thus, the provider, who is generally not financially disinterested, has a major influence over consumer demand, greatly diminishing the independent role of consumer choice in the market for healthcare services and insurance (Newhouse 2002). Because of these problems, as well as other monopoly elements such as the lack of free entry to and exit from

the industry by producers, price collusion among producers, and the fact that the available distribution of income may exclude some groups from healthcare, the market fails to achieve satisfactory allocation and distribution of healthcare resources.

The tools of efficiency analysis, grounded in theories of the competitive market, do provide, however, a conceptual point of reference and a set of methodologies for examining the extent to which the operation and outcomes of healthcare markets achieve optimal efficiency—either in the production of health or healthcare.

KEY METHODS OF ASSESSING EFFICIENCY

Economic analysis is typically divided into micro level and macro level. The micro level examines the behavior of individuals, firms, and markets. It therefore encompasses the three health services research levels defined earlier within the clinical perspective on effectiveness: patient, institution, and system. Macroeconomics focuses on the economy as a whole. Of concern are aggregate measures of employment, economic growth, foreign trade, and inflation. Analogous macro-level concerns in healthcare are the life expectancy of the population, the infant mortality rate, disability-adjusted life years, and the growth in healthcare expenditure, particularly as they compete with other health-producing investments. This parallels the population perspective on health outcomes at the community level (as shown in Figure 2.1).

Micro Level

The principal methods employed in micro-level analyses of efficiency include (1) estimating production functions and (2) cost-effectiveness, cost-benefit, and related cost-utility analyses.

Production Functions

Economists have developed a comprehensive theoretical model of production efficiency, expressing how the total, average, and marginal costs of a given product or commodity change under a given set of assumptions regarding the relationship between inputs and outputs (i.e., the production function), the cost of inputs, and technology. For example, inputs for ambulatory healthcare may include nurse and physician time, and outputs may be defined in terms of services rendered or their effect on the health of patients. Input costs include nurse and physician earnings, rents, and the cost of supplies. Technology is defined

broadly as the information and techniques required to transform inputs into outputs. The cost functions represent the minimum total and unit costs attainable for alternative combinations of inputs and the size of the production units.

It is possible to determine the cost-minimizing mix of inputs for any level of output and the cost-minimizing size of the production unit (Byrns and Stone 1995). Even when producers have the "right" combination of inputs and size, they may fail to achieve maximum output. This may be because of poor management, low employee motivation, or other unspecified production problems and is referred to as X-inefficiency (Leibenstein 1966). *X-inefficiency* occurs whenever a firm produces less than the maximum possible output from given resources. Production and cost functions can be empirically estimated for any production process and level of analysis, although they will be less precise in areas where output is often difficult to define and measure (e.g., healthcare). Production and cost models have been applied to physician, hospital, and insurance services to determine the extent to which production efficiency has been achieved and how it may be enhanced.

Similarly, production functions have been applied in the context of allocative efficiency analyses concerned with determining the optimal allocation of resources to improve the health of individuals and communities. Summaries of selected studies are provided in Chapter 5.

Cost-Effectiveness, Cost-Benefit, and Cost-Utility Analysis

Other efficiency analysis methods frequently applied in healthcare are cost-effectiveness analysis (CEA), cost-benefit analysis (CBA), and cost-utility analysis (CUA) (Drummond et al. 1997; Granata and Hillman 1998; Haddix, Teutsch, and Corso 2003). (See Table 4.1 for a comparison of these methods.) *CEA* is a systematic analysis of the effects and costs of alternative methods or programs for achieving the same objective (e.g., saving lives, preventing disease, or providing services). CEA is used to determine production efficiency, and effects are measured in non-monetary units. *CBA* is a systematic analysis of one or more methods or programs for achieving a given objective and measures both benefits and costs in monetary units. *CUA* is conducted when effects are weighted by utility measures denoting the patient's or member of the general public's preference for, or the overall desirability of, a particular outcome (Gold et al. 1996).

Table 4.1 Comparisons of Cost-Effectiveness, Cost-Benefit, and Cost-Utility Analysis

Type of Study	*Measurement/ Valuation of Costs in Both Alternatives*	*Identification of Consequences*	*Measurement/ Valuation of Consequences*
Cost-effectiveness analysis	Dollars	Single effect of interest, common to both alternatives, but achieved to different degrees (e.g., different mammography screening reminder systems).	Natural units (e.g., visits, life years gained, disability, etc.)
Cost-benefit analysis	Dollars	Single or multiple effects not necessarily common to both alternatives, and common effects may be achieved to different degrees by the alternatives (e.g., money saved from investment in breast cancer screening compared to smoking and obesity risk-reduction programs).	Dollars
Cost-utility analysis	Dollars	Single or multiple effects, not necessarily common to both alternatives, and common effects may be achieved to different degrees by the alternatives (e.g., quality-adjusted life years added from investment in mammography screening compared to smoking and obesity risk-reduction programs).	Healthy days or (more often) quality-adjusted life years

Source: Adapted from Drummond et al. (1997, Table 1.1, 2). By permission of Oxford University Press.

CBA can determine whether a program is worth establishing, in the sense that its benefits are greater than its costs (i.e., allocative efficiency). For example, do the benefits of screening, early diagnosis, and treatment for breast cancer outweigh the costs? A broader view might compare the net savings, if any, of mammography screening to other medical (e.g., hormonal therapy) or public health alternatives (e.g., smoking and obesity risk-reduction programs). Programs with the highest net benefit are most allocatively efficient. Society is worse off by adopting projects for which the costs outweigh benefits and better off by adopting projects for which benefits most outweigh costs. While it is not practical to rank all possible competing uses of resources to achieve the optimal resource allocation, projects can be considered on an incremental basis.

CEA compares the cost of alternatives in achieving a common objective (i.e., production efficiency) without determining whether the objective itself is worth achieving. For example, what are the costs per life year saved of mammography screening, early diagnosis, and treatment of breast cancer in the United States? CEA can be used as both a complement to and a substitute for CBA. For example, to evaluate mammography screening, one could use CEA to determine the most efficient way to encourage women to undergo routine screening given several available behavioral interventions (e.g., patient reminder systems, provider reminder systems, or a combination). These production efficiency results would then feed into the CBA to address the allocation question, How much, if any, amount should society invest in screening, early diagnosis, and treatment of breast cancer?

Alternatively, it may be determined that the program would not have net economic benefits (Cutler and McClellan 2001) but may yield health benefits and should be compared with other programs in terms of cost per quality-adjusted life year (QALY) gained. Effectiveness can then be measured in terms of increases in QALYs and compared to other activities on the basis of cost per QALY, which would be a cost-utility approach to evaluation. Instead of monetary values, life years would be valued (or quality adjusted), according to utility values, or how people feel about time spent in alternative health states ranging from states they feel would be worse than death to being completely healthy (Torrance and Feeny 1989; Torrance et al. 1996). For example, while being completely healthy may be assigned a utility value of 1, the condition of late-stage breast cancer may be assigned a value of 0.3

(Kerlikowske et al. 1999). If an otherwise healthy person could avert late stage breast cancer for one year, the gain would be 0.7 QALYs.

Use of CBA and CUA has largely focused on medical care services as opposed to nonmedical health investments (Blumenschein and Johannesson 1996; Cutler and McClellan 2001; Segal and Richardson 1994).

In the United States, the economic evaluation of pharmaceuticals has become an area of increasing interest with the growth in managed care and the rapidly increasing expenditure on prescription drugs. As a consequence, the field of pharmacoeconomics has emerged, in which economic evaluation methods are used to examine alternative drug treatments and to identify the costs and benefits of these treatments (Center for Studying Health System Change 2003b; Delea at al. 1999; Drummond et al. 1992; Granata and Hillman 1998; Hillman 1996; Oster et al. 1996; Power 1996). Australia, Canada, and the United Kingdom have developed guidelines for economic evaluations of pharmaceuticals as a basis for determining which might be included in national or provincial drug formularies (Alban et al. 1997; Australian Government, Commonwealth Department of Health and Aging 2002; CCOHTA 1997; Glennie et al. 1999; Government/Pharmaceutical Industry Working Party 1994; Hjelmgren, Berggren, and Andersson 2001; Mullins and Ogilvie 1998; Oostenbrink, Koopmanschap, and Rutten 2002). A vigorous debate has been waged in the U.S. Congress regarding development of research on the economic evaluation of pharmaceuticals related to the coverage of prescription drugs under Medicare (Pear 2003).

Expanded government funding of medical effectiveness, outcomes research, and clinical guidelines provides information to carry out further economic evaluation of healthcare services. AHCPR established Patient Outcome Research Teams (PORTS) to carry out broad investigations of alternative services or procedures for managing specific clinical conditions (see Chapter 3). The second phase of PORTS (PORT II) pursued the same objectives of improvement in healthcare quality and effectiveness, but offered investigators more flexibility in selecting research designs and effectiveness (AHRQ 2003).

Effectiveness information can also feed into policy models designed to integrate issues of quality of life, patient functional status, and costs. Kaplan and Anderson (1988) developed a measure that integrates the health benefit and utility frameworks for the evaluation of healthcare programs. Specifically, their measure integrates point-in-time estimates of function, transition among functional levels over time, utilities of

health states, and mortality. It has been applied to several prevention and treatment program evaluations.

Oregon policymakers applied CUA, if not the exact methods, to the allocation of scarce health resources. The Oregon program attempted to rank health services according to their potential benefits, with services being limited by the total budget that is politically allocated. In this way more people could obtain basic coverage, but some services judged to be of less value were not covered (Eddy 1991; Goldsmith 2003; Hadorn 1991). The economic evaluation of services was limited by political considerations, and therefore the methods were not rigorously applied (Tengs 1996). This may simply reflect the fact that the allocation of public resources is ultimately a political decision, and political factors may override strictly economic considerations.

Macro Level

The principal macro-level approaches to efficiency analysis are based on international comparisons of the performance of healthcare systems in different countries. While there are major problems with comparisons at the system level, such as measurement of health outcomes, cultural and demographic differences, and data comparability, such comparisons do serve to raise questions about the efficiency and equity of health systems and to stimulate inquiry into reasons for major observed differences (Anderson et al. 2003; Kanavos and Mossialos 1999; Reinhardt et al. 2002).

Researchers at the Organization for Economic Cooperation and Development (OECD) have collected data and attempted to develop standardized international health accounts for the organization's 30 member countries. Comparisons rely on aggregate measures of life expectancy, infant mortality, and cause of death–specific mortality. Simple correlations between healthcare spending per capita and aggregate health measures are examined along with differences in input prices, the production structure of the health sector (e.g., amount spent on hospitals and doctors), input volumes, administrative costs, and appropriateness of care. Disease-specific comparisons for the United States and Canada have been made for cardiovascular disease, cancer, and psychiatric services (Cutler 2002).

Many analysts emphasize the need to broaden the policy framework beyond healthcare to include the social and physical environment and to focus more on primary prevention and health promotion services

that are usually underfunded because of the large expenditure on medical care treatment. Healthcare purchasers in a few countries (e.g., Canada, United Kingdom, Ireland, Iceland, and New Zealand) have established health goals for the population and are searching for alternative ways of achieving those health goals, including preventive healthcare and more effective integration of health and other policy issues such as education, housing, and social policy. Methods of allocative efficiency analysis have been applied in making these decisions (OECD 2002a). McGinnis, Williams-Russo, and Knickman (2002) have called for an improved science base on the effectiveness and cost-effectiveness of alternative population-based health promotion interventions to guide resource allocation.

SUMMARY AND CONCLUSIONS

The principal objectives of this chapter are to (1) define efficiency and (2) describe how it might be assessed. To address the first objective, the major types of efficiency analyses—allocative and production efficiency—and the theoretical assumptions underlying them are presented. With population health as the goal, allocative efficiency concerns attainment of maximum population health from the limited resources that society has available for that objective during any given period of time. This requires that societies maximize efficiency by choosing the "right" most-valued mix of medical and nonmedical services and by producing them at minimum cost.

Analysts have developed both micromethods and macromethods for efficiency assessment. Micromethods include the normative microeconomic theories of markets, including production and cost functions as applied to healthcare. Also, the techniques of CBA, CEA, and CUA are used to examine the efficiency of healthcare production and efficiency in the mix of specific healthcare services and programs. Extensive data on the OECD countries permit international comparisons at the macro level in terms of spending, utilization, and health indicators for the population (OECD 2002b).

Chapter 5 provides a selected summary of evidence on the efficiency of the United States' and other countries' healthcare systems and discusses the major policy strategies for improving efficiency.

NOTES

1. Spending for environmental activities (e.g., air and water pollution abatement, sanitation and sewage treatment, water supplies) is excluded from the national health accounts (Cowan et al. 2002).

2. For a detailed explanation of Pareto's work, see Kohler (1990, 484–519).

3. "Marginal" refers to the next unit of a good or service that the consumer is considering. This differs from the average total value of all units consumed. A "rational" consumer would not purchase the next unit of a good or service if he or she perceived the benefit of that next unit to be less than the cost of the unit.

4. Even for sophisticated tertiary care, doctors have long acknowledged, if not always fostered, the patient's right to be part of the decision-making team when alternative courses of action are contemplated that include alternative levels of risk, benefit, and costs. Patient values are now being fully integrated with clinical information in patient outcome studies (Ware et al. 1996).

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Chapter 5

Efficiency: Evidence, Policy Strategies, and Criteria

CHAPTER HIGHLIGHTS

1. While all health systems are mixed, they can be characterized by the degree to which they utilize private markets in the allocation of resources and the production and distribution of health services. *Market-minimized* systems tend to rely more on direct government or quasi-government controls to achieve the desired results, whereas *market-maximized* models rely primarily on the private market to allocate resources and use the government to subsidize care for the most vulnerable segments of the population.
2. Efficiency strategies used by market-oriented systems include copayments and other targeted financial incentives, utilization management, managed competition, healthcare reimbursement accounts, and consumer-driven health plans.
3. Regulated systems tend to rely on fee controls, supply controls, global budgeting, needs-based resource allocation, and limited internal markets for achieving efficiency goals.
4. Criteria for evaluating healthcare systems and policies in terms of efficiency would focus on success in macro cost control (reducing healthcare spending as a percentage of gross domestic product) and dynamic efficiency (finding innovative ways to improve efficiency), as well as allocative and production efficiency.

OVERVIEW

The major questions addressed in this chapter ask (1)What policy strategies are available to achieve efficiency? (2) What is the evidence regarding

the efficiency of these strategies? and (3) What criteria should be used in judging the efficiency of policy alternatives?

This chapter first describes the theoretical assumptions and specific strategies for achieving efficiency that underlie both market-maximized and market-minimized healthcare systems. Secondly, evidence is provided regarding the allocative efficiency of healthcare systems in improving health outcomes at the macro level based on the performance of the U.S. healthcare system compared to selected western nations. Micro-level evidence on production efficiency focuses on physicians, hospitals, and health plans.

POLICY STRATEGIES RELATING TO EFFICIENCY

Anderson (1989, 19) noted that countries in the developed world vary along a continuum, from left to right, of market-minimized to market-maximized organization and financing of their healthcare sectors:

> On the right-hand side of the continuum, one is likely to believe in cash indemnity for health services and financial controls on patients. This view regards providers of services as essentially autonomous sellers of services; patients, as it were, hire a physician to manage their service needs. On the left-hand side of the continuum, with the highly structured and completely government-owned health service, there would be no charge to the patient at the time of service. Charges to the patient at that time, no matter how small they were, would be regarded as an undesirable barrier to access to services for prevention, early diagnosis, and treatment. At the right-hand extreme, patients are assumed to know their self-interest well enough not to be inhibited by charges at the time of service.

While all countries seek to achieve equity, efficiency, and overall cost control in their health sectors, each has a unique approach to addressing these basic economic and social issues. The market-minimized systems tend to rely more on direct government or quasi-government controls to achieve the desired results, while market-maximized models rely primarily on the private market to allocate resources, and use the government to subsidize care for the most vulnerable segments of the population. Each country's approach to healthcare may depend

largely on its culture, history, and political situation (Cutler 2002). The theoretical foundations for the market-maximized and market-minimized strategies, and implications of each for the formulation of health policy, are reviewed in the sections that follow.

General Efficiency Strategies

Market-Maximized Models

Paul Feldstein (1998) used a qualitative method to evaluate the allocative efficiency of the healthcare sector from a market perspective. The assumption of consumer power in the marketplace and the criterion of maximum consumer well-being form the basis of his economic evaluation. The various sectors (e.g., insurance, hospital care, and physician care) are examined in terms of the degree to which observed behavior is consistent with the predictions derived from the basic economic model of the competitive market. For example, do producers strive to minimize the cost of production, and are the mix and quality of goods and services guided by consumer choices? When inconsistencies occur, the basic assumptions of the competitive model are reexamined and altered in an attempt to better explain observed behavior. Feldstein found distortions in the insurance and healthcare markets that have led to misallocation of resources and inefficient production methods. Cost-based reimbursement of hospitals resulted in nonprice competition, excess capacity, and high cost. These problems have been addressed through government policy changes and market reform. For example, the Medicare prospective payment system reversed the economic incentives facing hospitals for beneficiaries, and health plans based on the principles of managed care are forced to compete on price to serve health plan enrollees.

Enthoven (1990) proposed managed competition as a comprehensive solution to market failure in the healthcare sector in the United States in the early 1990s. This approach depended on market incentives to motivate health plans and providers to be efficient and responsive to consumer needs and demands. Private sponsors of health benefits (e.g., employers) and public sponsors (e.g., state government agencies) would aggressively monitor and manage competition among health plans in the healthcare market. Employers would play their traditional role as suppliers of health benefits, and a public agency or agencies

designated by the state would serve as a broker for self-employed and other persons who chose to obtain health insurance through the state sponsor. Fixed contributions (with a limit on tax deductibility) from sponsors would provide incentives for cost-conscious choice by consumers. By spending their own after-tax dollars beyond the employer contributions, consumers would determine the growth in healthcare spending in the United States.

In 1992, President Clinton selected managed competition as the strategy for achieving universal coverage and cost control. Failure of that federal legislative effort left the states and the private sector to deal with the problem. Major features of managed competition that are still lacking in most states include regional purchasing cooperatives, limits on tax deductibility of health insurance, mandatory provision of health insurance benefits or payments by all employers, and a means to finance and enroll all of the uninsured.

Because of the success of managed care and competition in controlling cost in the mid-1990s, the private sector and most states expanded enrollment of beneficiaries in managed care, and many states established rules and regulations to control some of the negative consequences of healthcare competition (Jensen et al. 1997). States served in the sponsor role for Medicaid managed care patients and the federal government established mechanisms for Medicare beneficiaries to enroll in competing risk-based health plans through Medicare+Choice. While the Medicare+Choice managed care initiative enrolled up to 12 percent of all Medicare enrollees in 2002, it largely failed to meet its major objectives. Overall Medicare spending continued to increase. Medicare+Choice was also beset by health plans withdrawing from the program as well as declining consumer enrollment (Thorpe and Atherly 2002).

Medicaid managed care fared better with a 40 percent growth in enrollment between 1997 and 2001. However, the number of plans willing to serve Medicaid has declined and payment rates have presented a major issue in states' efforts to sustain Medicaid managed care (Holahan and Suzuki 2003).

The tight labor market in the late 1990s, reductions in capitation payments by government payers, and the consumer and provider backlash against the constraints imposed by managed care led to the development of less restrictive managed care, reductions in government beneficiary enrollment in managed care plans (Commonwealth Fund

2002), and renewed double digit healthcare cost growth (Center for Studying Health System Change 2003; Draper et al. 2002; Lesser, Ginsburg, and Devers 2003). The private sector has pulled back from integrated delivery systems and management of care in favor of "consumer driven" health plans (Casalino and Robinson 2003; Gabel, Lo Sasso, and Rice 2002). These plans offer extensive choice regarding benefits, premiums, and out-of-pocket payments (Robinson 2002). They feature healthcare reimbursement accounts, where consumers spend their own funds set aside for out-of-pocket costs, and extensive information for health plan and healthcare decision making. While employers play a role in offering these plans through the workplace, consumers play a much more direct role in determining their benefit levels, healthcare utilization, and cost. While still limited, this model is a major thrust toward an even more market-maximized health system than was managed competition. A survey of 1,000 employers in 2003 found that 30 percent planned to offer consumer-directed health plans within the next five years (Deloitte & Touche 2003).

Market-Minimized Models

Williams (1990) contrasted the market-maximized approach with a public-sector political framework that is also concerned with production efficiency. However, in the political framework, the electorate judges allocative efficiency by the extent to which the healthcare system improves the health status of the population in relation to the resources allocated to the system, and priorities are determined by social judgments of need. European nations with social insurance systems or tax-financed national health systems have traditionally used this approach to healthcare resource allocation. In such systems, solidarity is promoted over individual rights and choice.

Recent thinking and policy has recognized the limits to both market and nonmarket approaches to healthcare and that each approach needs the other to achieve the social goals of efficiency and equity (Rice et al. 2000). Furthermore, all systems de facto represent some mix of market elements and nonmarket controls.

National Health Services in Britain and New Zealand largely exemplify systems that use a needs-based approach to establish the allocation of resources to the health sector and the type of services to be provided. In the 1990s, however, internal markets were developed to achieve

efficiency of production (Cooper 1994; Glennerster 1995; Street 1994). The goal was to provide incentives for efficient production and allocation of resources while retaining overall control of spending and tax-based financing of healthcare and maintaining or improving equity in access to care. These markets separate the responsibility for ensuring that patients receive care from the responsibility for the direct provision of that care. Under this system a health authority uses its budget to purchase services from other health authorities, general practitioners, private hospitals, nursing homes, and local government social service departments.

These authorities in turn identify healthcare needs and priorities for their area and determine the best way to spend funds allocated by the central government to meet area health needs and priorities. General practitioner groups are also responsible for the health needs of their patients and are encouraged to be conscious of the costs of their clinical decisions. Physician groups receive budgets to provide primary care services and purchase selected secondary health services (e.g., diagnostic services, elective surgical procedures, and prescription drugs) for their registered patients (Department of Health 1997; Klein 2001; Le Grand, Mays, and Mulligan 1998; Maynard and Bloor 1996; R. Robinson 1996).

The National Health System (NHS) in the United Kingdom has suffered from underfunding for many years. This has resulted in long waiting lists for hospital appointments and poor-quality hospital buildings. The government attempted to rectify some of the problem by increasing healthcare spending by 6 percent per annum in real terms. Except for some reduction in high-profile waiting times, the market-based reforms and increases in budget allocation have not achieved the expected improvements in the system (European Observatory on Health Care Systems 2002; Le Grand 2002). Subsequent reforms to NHS have continued a move toward decentralization and local accountability but moved away from the former conservative government's language of market competition (Lian 2003).

The United States, which may be the most market-oriented system, implemented administered price systems for federal beneficiaries under the Medicare program. The 1989 resource-based relative value scale (RBRVS) for physician service fees was developed to complement the 1983 prospective payment diagnosis-related group (DRG)–based fee system for hospitals. The 1997 Balanced Budget Act extended the per-

case payment system to all types of postacute care (McCall et al. 2003). In addition, work continues on medical effectiveness studies, guidelines, incentives for improved quality, and methodological improvements for cost-effectiveness studies that will yield information for better public and private decisions on resource allocation (Gold et al. 1996; National Health Care Purchasing Institute 2002a).

The precise strategies employed to ensure efficiency differ across systems, but include efforts to control both the prices and volume of healthcare services through the methods of paying providers and of managing and overseeing utilization, respectively.

Specific Efficiency Strategies

Payment Methods

Alternative methods of paying physicians and hospitals provide different incentives regarding efficiency (D'Intignano 1990; Newhouse 2002b). Market-minimized models tend to rely more on global budgets and strict fee controls, while market-maximized models encompass a range of alternatives, including fee-for-service, salary, capitation, and prospective payment.

Physicians. The United States has many different payment methods and sources operating simultaneously, while other countries have a primary method for hospital and physician reimbursement and funnel payment through relatively few channels. For example, physicians in the United States are paid by local, state, and federal agencies; by over 1,500 insurers; and by direct out-of-pocket payments from patients. Methods of payment include fee-for-service, salary, and capitation. Until the 1990s, fee-for-service based on usual and customary fees was the dominant payment method in the United States. Fee-for-service is now based on administered prices for most services covered by private insurance and for government beneficiaries in the Medicare and Medicaid programs. While fee-for-service provides an incentive for high productivity, administered prices tend to be rigid and may be set at levels that result in over-service (cost of the service exceeds the value) and economic rent (profits beyond the "normal" rate of return on investments), especially when there are thousands of services and rapid technological change in the industry (Newhouse 2002a).

Canada and Germany have also relied on fee-for-service payment for physicians, but fees have been strictly controlled. Germany developed a volume control mechanism whereby quarterly, every office-based physician invoiced his or her physicians' association for the total number of relative value service points delivered. The physician's income was calculated by multiplying the service points by the point value. The point value was determined by dividing the regional budget for ambulatory care by the total number of service points submitted (European Observatory on Health Care Systems 2002). An increase in the aggregate number of services causes the per-unit payment for services to decline, providing an indirect incentive to control the volume of services.

Hospitals. Per diem prices, prospective payment by DRGs, and prepayment are the primary methods for paying for hospital services in the United States. Each method provides different financial incentives for the hospital and has different implications for efficiency (Dowling 1974; McClellan 1997). Although traditionally hospitals have been paid per diem rates plus fees for individual ancillary services, with the passage of the prospective payment system (PPS) under the Medicare program in the United States in 1983, the U.S. system of paying for hospital services began to assume more market-minimized methods of financing. That system was designed to pay a fixed amount per episode of hospital care defined by one of about 500 diagnostic groups. It provided an incentive for hospitals to encourage doctors to reduce length of stay and to provide hospital services more efficiently, because each hospital could retain any surplus in payments over costs. Peer review organizations were to monitor the necessity of admissions and the adequacy of care to offset the incentive to admit more patients, to underserve them, and to discharge them prematurely.

Canada has global hospital budgets and regional health planning to control the cost of hospital care and the diffusion of medical technology, and many provinces have also introduced caps on physician spending. Germany applies target budgeting to hospitals for operating costs. Target budgets are negotiated with sickness funds. Payments are adjusted based on the extent to which hospitals meet or exceed their service targets for the year. The German Reform Act of Social Health Insurance of 2000 mandated the introduction of a hospital PPS based on DRGs for 2004 (European Observatory on Health Care Systems 2002).

Since the 1980s, many countries focused their efforts to control healthcare expenditures on global budget or sectoral budget caps. The key to cost control in these macromanaged systems is having a dominant source of payment that fixes the budget for a given period of time. Growth in the budget is generally limited by growth in the economy. Thus, the Canadian and German systems rely on control of the funds available to physician and hospital providers rather than on the mixture of market incentives and controls present in the United States.

Although global budgets may control the rate of increase in spending, they do not necessarily lead to either greater allocative efficiency or greater production efficiency. In the process of controlling total costs, perverse incentives may be created for both allocation and production. Hospitals may pressure physicians to keep beds full to justify a continuation or expansion of the hospital budget. More efficient, innovative outpatient delivery may lag because of the lack of incentives to develop new services. Recognition of these issues has contributed to concern about the ability of global budgets to continue to control healthcare expenditure increases.

Concerns have also been expressed about the ability of these health delivery systems to respond to changing patient needs and demand for healthcare. Spending constraints, for example, have resulted in declining levels of satisfaction with access to care in Canada (Blendon et al. 2002). These problems have prompted Canadians to register dissatisfaction with their healthcare system and call for fundamental reforms. This is a major change from the early 1990s when Canada was alone among developed countries with a majority of the citizenry satisfied with their healthcare system (Tuohy 2002).

With the increasing use of capitation and prospective payment methods, the United States has taken on more of the characteristics of market-minimized models for paying providers, while countries that have traditionally been more market-minimized have attempted to incorporate some of the market methods to control the rising cost of both physician and hospital services in their own countries and to make their systems more responsive to consumers.

Utilization Management

The market-maximized model that dominates approaches to controlling cost and resource allocation in the United States has been characterized as micromanagement, in contrast to the macromanagement

strategy practiced in other countries (Reinhardt 1990). Micromanagement tends to rely on incentives such as copayments for consumers and capitation payment for providers in competing managed care health plans, whereas macromanagement relies on controls such as fee schedules, global budgets, and limits on diffusion of technology to achieve health system objectives.

In the market-based U.S. healthcare delivery system, emphasis is on affecting the behavior of individual providers and patients with a mix of incentives and controls. Thus, in managed care plans, there are elaborate methods for utilization review, selective contracting, capitated payment of providers, and practice guidelines. For patients, there are provider gatekeepers and control of referrals, copayments, coverage limits, limits on provider choice, financial penalties for not complying with plan requirements, and information services to aid consumer choice of health plans and healthcare services. Targeted financial incentives are now being used to improve the quality and efficiency of the care process by inducing practice founded on evidence-based guidelines (Center for Studying Health System Change 2002b; National Health Care Purchasing Institute 2002b). Competition is introduced through an array of health plan choices for those with public or private insurance (Gabel, Lo Sasso, and Rice 2002).

The key to macromanagement is limiting the sources of payment and controlling the payment amounts. Regional planning to limit the physical facilities and assure fair distribution, thereby providing the constraints within which providers work is one example of macromanagement. The system results in queues for expensive high-technology procedures and equipment, forcing physicians to allocate services based on the urgency of the cases.

Evidence regarding the success of market-maximized models in comparison with the success of market-minimized models will be discussed in the section that follows.

EVIDENCE RELATING TO EFFICIENCY

Allocative Efficiency

As indicated in Chapter 4, allocative efficiency is most essentially concerned with maximizing health, given constrained resources. Three general health policy strategies that reflect a concern with allocative efficiency were reviewed, related to investing in (1) medical versus nonmedical

policy alternatives; (2) preventive services; and (3) mix or types of treatment, in relationship to health improvements. Specific micro-level evidence, based on related micro-level methods (e.g., health production functions and cost-effectiveness analysis), is presented with respect to the likely allocative efficiency of these alternatives. Macro-level evidence is provided on the comparative experiences of the United States and other countries to provide a sense of whether market-maximized or minimized models of healthcare system design might be more successful in achieving allocative efficiency.

Micro Level

Medical Versus Nonmedical Alternatives. A basic conclusion of Chapter 3 was that personal healthcare provides a contribution to population health that is modest compared to those of human biology, environment, and behavior. Thus, critics have long been concerned that modern developed countries allocate too many resources to the delivery of personal health services and too few to broader public health and social interventions at the population level (Fuchs 1974; McKeown 1990; Tarlov and St. Peter 2000).

Folland, Goodman, and Stano (2001, 97–100) have provided an overview of the contributions of medical and nonmedical interventions to health, grounded in the concept of a production function for health—that is, the relationship of inputs to health outputs. They conclude that the production function for health tends to exhibit diminishing marginal returns to healthcare, particularly in developed countries. Historical declines in mortality rates may be most accurately attributed to improved environment and nutrition, rather than to medical care per se. Studies have demonstrated that the marginal product of healthcare in reducing mortality in the United States does not differ significantly from zero, although it is higher for certain groups, such as the elderly. As indicated in Chapter 4, lifestyle and education as measured by years of schooling appear to be significantly related to population health. The findings, based on estimating the production function for health, argue for a broader focus on public health and nonmedical interventions to serve the allocative efficiency, as well as the population effectiveness, objective. Countering this view is research suggesting that innovations in medical treatments for heart attacks, cataracts, depression, and low-birthweight infants have been well worth the investment, as the

aggregate dollar value of the benefits have greatly exceeded the aggregate cost (Cutler and McClellan 2001).

Preventive Services. Economic evaluations of the cost-effectiveness of specific prevention-oriented interventions provide another important type of evidence for making resource allocation decisions. Mammography screening for early detection of breast cancer was underprescribed and underused prior to assessment of its effectiveness and cost-effectiveness. Several studies in Europe and the United States show that screening and early treatment yielded a cost per life year saved that was well below the $50,000 per life year saved norm (de Koning 2000). Partly based on economic evidence, Medicare began to cover mammography screening without an age limit for its beneficiaries. An important current issue is the possible underuse of mammography screening among women 70 years of age and older. Screening declines with age, while breast cancer mortality increases. Remaining life years and quality-of-life issues need to be factored into the analysis (Kerlikowske et al. 1999). If mammography is cost-effective at higher ages, it will be important to identify cost-effective methods to increase screening rates among the elderly (Fishman et al. 2000; Vernon et al. 2000).

The Harvard study of 500 life-saving programs (Tengs et al. 1995) surveyed the literature on the cost-effectiveness of life-saving interventions in the United States.[1] Life-saving interventions were defined as any behavioral or technological strategy that reduced the probability of premature death among a specified target population. Programs were categorized by sector, including healthcare, residential, transportation, occupational, and environmental; and by three levels of prevention, including primary, secondary, and tertiary. The 587 interventions ranged from those that save more resources than they consume to interventions that cost more than $10 billion per year of life saved. Cost-effectiveness varied by sector; however, in medicine, primary prevention programs cost only $5,000 per life year saved, compared to about $23,000 for secondary and tertiary programs.

Because this research represents a synthesis of existing studies, there were significant methodological limitations: the validity of the conclusions is dependent on the accuracy of data and analyses in the original studies; due to publication bias the studies represented a nonrandom sample of life-saving programs; and some benefits and effects were not measured (e.g., efforts to save the lives of some people may have reduced

injuries in others and environmental programs may improve the quality of life as well as save some lives). The study does, however, illustrate the potentially large variation within and between sectors and levels of prevention and, therefore, the potential efficiency gains associated with targeting limited resources at those programs that achieve the greatest health improvement per dollar of investment.

Mix or Types of Treatment. The RAND Health Insurance Experiment addressed the allocative efficiency of coinsurance in the context of the U.S. healthcare financing and delivery system. The study examined the effect of copayments on utilization and expenditures for healthcare services and the extent to which increases in utilization associated with "free" care affected health status. The basic finding was that free care, compared to higher copayment levels, resulted in a 50 percent increase in expenditure with no significant effect on the health status of the typical person. Those, however, who were sick, poor, or both at the time of enrollment obtained significant health status benefits from increased utilization. Reducing the price to the "typical" consumer below the cost of production results in consumption of services for which the marginal value is below the marginal cost of production with little or no effect on health. This is the basic condition of resource misallocation—resources would provide more benefit if allocated elsewhere.

From the Health Insurance Experiment findings, Manning et al. (1987) estimated that of the $200 billion that the under-65 population of the United States spent on healthcare in 1984, a $37 billion to $60 billion welfare loss—the allocation of resources to procedures or services with minimal or no benefits—would have been incurred by moving from the 95 percent copayment plan with a $1,000 maximum out-of-pocket expenditure to the free plan. This is the estimated amount of overspending that would have occurred under the free plan given the low marginal value of the added healthcare services that would have been consumed. Manning and colleagues note that some of the strong assumptions required to obtain this estimate might not hold and therefore might lead to an overestimate of loss. However, this is probably more than offset by the fact that the estimate ignores the incentives to employ new technologies associated with more generous insurance plans. New technology is often used in ways that produce low marginal benefits for patients relative to cost, thereby adding to welfare loss (Cutler 2000).

The RAND Health Insurance Experiment established a baseline of the probable magnitude of inefficiencies in the U.S. system and identified some areas for potential savings. For example, Siu et al. (1986) found that the introduction of cost sharing had an impact on both appropriate and inappropriate hospital admissions. The percentage of hospital admissions deemed inappropriate (22 percent) was only slightly lower than the proportion of inappropriate hospital admissions for free care (24 percent). This information suggests that significant portions of hospital admissions are inappropriate and potentially avoidable. A follow-up to the RAND studies by McGlynn et al. (2003) found that patients receive about half of the recommended care for their condition based on quality standards. This was true for both treatment and prevention and varied substantially by medical condition.

The welfare loss estimate given by Manning et al. (1987) was based on the 1984 structure of the U.S. healthcare system with its mix of regulatory and competitive features. The estimate also does not represent a forecast of the effects of fundamental restructuring of the healthcare delivery and financing system to approximate systems in Canada, Europe, or the present-day United States. While other systems provide first-dollar coverage, they also include budget caps, volume controls, and stringent control over capital expenditure on healthcare technology and facilities. Compared to 1984, the current U.S. system is more competitive, and utilization and cost are more constrained by managed care, even though the managed care constraints have recently been reduced.

Comparing U.S. spending, utilization, coverage, and health outcomes with those of other democratic, industrialized countries provides an important macro-level perspective on the U.S. healthcare system. Although observed differences may be a function of a variety of factors, they nonetheless pose questions, as well as point to answers, regarding ways in which present and future system performance might be improved (Cutler 2002; Rice et al. 2000).

Macro Level

The 30 member countries of the Organization for Economic Cooperation and Development (OECD) include democratic countries that range from economic powers, such as the United States, Germany, and Japan, to smaller countries with more modest economic achievement, such as Greece and Portugal (OECD 2002, 2003).

Table 5.1 provides data by country on the role of the public sector in the provision of health insurance coverage and payment for healthcare services. Until the market reforms of 1991, the United Kingdom represented the extreme of market-minimization with a national health service; government ownership of hospitals; direct employment of hospital physicians, nurses, and allied health workers; and central budgetary control. The market-maximized extreme is represented by the United States, with a majority of private hospitals and private physicians and other health workers, private insurance covering a majority of the population, out-of-pocket payment representing 20 percent of expenditures, and a plethora of payers without coordination of payment. Between these two extremes, but leaning more toward market-minimization, are the Scandinavian countries and France. Countries leaning more toward market mechanisms, although far left of the United States on the continuum, are Australia, Canada, Germany, Switzerland, and Japan. Less than half of the cost of inpatient and outpatient medical care is covered by public sources in the United States, compared to universal or near-universal public coverage in the other countries.

Relative to the United States, the Canadian system has high utilization of inpatient days and constraints on high-cost surgical and diagnostic procedures (Table 5.2). Germany has much higher inpatient utilization rates than Canada and the United States. Differences in average length of stay may reflect different policies regarding the use of hospitals for long-term and geriatric care (European Observatory on Health Care Systems 2002). Consultations and visits per capita were also higher in Germany and Canada than in the United States (Table 5.2). This may reflect the relatively high out-of-pocket payments at the point of service in the United States—traditionally the highest such payments among the developed nations.

Canada, Germany, and the United States each experienced a compound annual growth in per capita healthcare expenditure on the order of 10 percent per year until about 1990 (Table 5.3). Since the early 1990s, Canada and Germany have been more successful in controlling healthcare costs than has the United States.

The differences in the organization and utilization of services between the United States and other western healthcare systems are also likely to be mirrored in differences in their costs and population health outcomes. Table 5.4 shows a comparison among seven of the major

Table 5.1 Public Coverage Against Cost of Medical Care, Selected Countries, 2000

Country	*Inpatient Hospital Care (%)*	*Outpatient Medical Care (%)*
United Kingdom	100	100
Finland	100	100
Norway	100	100
Sweden	100	100
France	99.8	99.8
Australia	100	100
Canada	100	100
Germany	92.2[1]	92.2[3]
Switzerland	100[2]	100[2]
Japan	100	100
United States	47[1]	45[3]

[1] 1997
[2] 1999
[3] 1995

Source: OECD (2002).

industrialized OECD member countries. With 13.9 percent of its gross domestic product directed to health in 2001, the United States spent 3.2 percentage points more than the second-ranked country, Germany. The U.S. per capita healthcare expenditure was $4,887 in 2001, about 2.3 times greater than France, about twice the per capita health expenditure in Canada, and 2.6 times more than the sixth-ranked country, the United Kingdom. This large expenditure gap was apparently not offset by health outcome advantages for the United States, which had the highest infant mortality level of the seven countries and life expectancy figures that were lower than all of the other countries. Furthermore, the United States was the only country of the seven with a significant population lacking health insurance. (See the discussion of the uninsured in Chapter 7.)

Overall, then, the U.S. healthcare system, which represents the market-maximized end of the health policy continuum, appears to be far-

Table 5.2 Use of Inpatient Healthcare in Selected Countries

	Bed Days in Inpatient Care (number per capita)			*Inpatient Admission Rates per 100 of Population*			*Average Length of Stay in Inpatient Care (days)*			*Doctors' Consultations (number per capita)*[1]		
Year	U.S.	Canada	Germany	U.S.	Canada	Germany	U.S.	Canada	Germany	U.S.	Canada	Germany
1975	1.9	2.0	3.6	16.8	16.5	16.9	11.4	11.2	21.3	5.1	4.9	10.9
1980	1.7	2.1	3.6	17.1	15.0	18.8	10.0	13.1	19.0	4.8	5.6	11.4
1985	1.4	2.2	3.5	15.3	14.8	19.9	9.1	13.8	17.4	5.2	6.2	—
1990	1.2	2.0	3.3	13.5	13.6	20.0	9.1	13.0	17.2	5.5	6.7	—
1995	1.0	1.2	2.9	12.7	11.2	21.8	7.8	10.7	14.2	5.8	6.5	6.4
1999	0.9	1.1	2.7	12.5	10.2	23.1	7.0	8.7	12.0	5.8	6.4	6.5
2000	0.8	1.1[2]	2.7	12.4	10.2[2]	23.5	7.0[2]	8.7[2]	11.9	5.8	6.4	6.5

[1] 1996
[2] 1999

Source: OECD (2002).

Table 5.3 Healthcare Expenditures in Selected Countries

	Percentage of GDP			*Per Capita Outlays in US$*		
Year	U.S.	Canada	Germany	U.S.	Canada	Germany
1975	7.8	7.1	8.6	586	512	591
1980	8.7	7.1	8.7	1,055	770	1,160
1985	10.0	8.2	9.0	1,759	1,114	938
1990	11.9	9.0	8.5	2,738	1,860	2,063
1995	13.3	9.2	10.6	3,654	1,821	3,193
2000	13.1	9.2	10.6	4,540	2,095	2,398
2001	13.9	9.7	10.7	4,887	2,161	2,407

Source: OECD (2003).

ing poorly in comparison to other countries in terms of allocative efficiency—that is, maximizing health benefits in the aggregate relative to the magnitude of aggregate healthcare expenditures. This is consistent with the micro-level evidence on allocative efficiency presented earlier in this chapter, which documented that the marginal product of expenditures for healthcare in terms of health improvements is small relative to the contribution of other nonmedical social, economic, and public health investments.

In addition to concern about misallocating resources to services that provide low benefit relative to cost, there is concern in the United States and other countries that healthcare services of given quality are not being produced at minimum cost; efficiency strategies can also be judged by this criterion. While the least-cost production scale and methods are difficult to determine for medical services, several studies have produced evidence of inefficient production. The evidence regarding the production efficiency within the United States and other countries is reviewed next.

Production Efficiency

Healthcare services can be provided in many different ways with different combinations of personnel, facilities and equipment, production levels, and sites of service delivery. Production efficiency is achieved when

Table 5.4 Comparative Expenditures and Health Indicators for Seven Industrialized Countries

Country	*Total Expenditure on Health as Percentage of GDP, 2001*	*Total Expenditure on Health Per Capita in US$, 2001*	*Percentage of Population With Healthcare Coverage Under Public Programs for Inpatient and Acute Care, 2001*	*Infant Mortality per 1,000 Live Births, 2001*	*Life Expectancy at Birth in Years, 2001*	
					Male	*Female*
United States	13.9	4,887	25.3	6.9[2]	74.1	79.5[2]
Canada	9.7	2,245[1]	100.0	5.3[2]	76.7	82.0[2]
France	9.5	2,104	99.9	4.6	75.2	83.0
Germany	10.7	2,407	90.9	4.5	74.7[4]	80.7[4]
Italy	8.6[1]	1,580	100.0[3]	4.3	76.3	82.9
Japan	7.6[2]	2,864[2]	100.0[2]	3.1	77.7	84.9
United Kingdom	7.6	1,848	100.0	5.5	75.4	80.4

[1] 2002
[2] 2000
[3] 1997
[4] 1999
Source: OECD (2003).

production units are of optimal size and the mix of inputs is such that the marginal output per dollar of cost is equal across all inputs. Only then is the cost minimized for a given level of output of health services.

Economists and other health services researchers have conducted numerous studies of production efficiency, concentrating on (1) general administrative costs of health systems; (2) the size and mix of personnel within physician practices, as well as the method of paying physicians; (3) the optimal bed size for hospital care, the mix of inpatient and outpatient service delivery, the effect of payment methods on hospital costs; and (4) the utilization and cost impact of managed care. Evidence regarding each of these dimensions will be reviewed in the discussion that follows.

Administration

One result of micromanagement is very high administrative costs. It is extremely difficult to measure and compare administrative costs, and critics have argued that the U.S. costs are overestimated and comparisons between nations with fundamentally different healthcare systems may not be relevant (Aaron 2003; Gauthier et al. 1992). Systems that both supply and finance care in the public sector, such as those in Canada and the United Kingdom, appear to have lower administrative costs. An estimate based on 1999 data places such costs at 37 percent of healthcare costs in the United States versus 18 percent in Canada. Such costs are greater in insurance-based systems such as those in the United States and Germany; in Canada, administration is simplified by having one or few sources of payment and sets of rules. With global hospital budgets and fixed fee schedules, there is less need for close monitoring of provider behavior (Woolhandler, Campbell, and Himmelstein 2003), and costly activities associated with marketing are prohibited or severely restrained in the market-minimized systems. Whether or not a country is able to make fundamental reforms, administrative costs are substantial and may yield ongoing savings if processes can be streamlined and information technologies applied (Mehrotra Dudley, and Luft 2003).

Physician Services

Personnel Mix. Research employing different analytical approaches to the optimal use of physician aides has arrived at the same general con-

clusion: physicians could raise the productivity of their practices and lower the cost per office visit by employing more aides (Brown 1988; Reinhardt 1972, 1975; Smith, Miller, and Golladay 1972). Adding a nonphysician provider in managed care organizations has been documented to yield a 50 percent increase in a physician's panel size (Kindig 1996).

An array of studies had projected that the growth of managed care would have a significant impact on physician requirements. Generally, the number of physicians required was expected to decline while the percentage of primary care specialists was expected to grow (Ginzberg and Ostow 1997; Greenberg and Cultice 1997; Kindig 1996; Reinhardt 1996). While this scenario was coming true in the late 1990s, the collapse of tightly managed care has resulted in increasing specialization, increasing physician incomes, and pressures to increase the number of specialists (Grumbach 2002; Salsberg and Forte 2002).

Economies of Scale. Studies on economies of scale (i.e., the tendency of average cost to decline as the scale of production increases) in physician practice conclude that group practice is more efficient than the traditional solo practice of medicine (Lee 1990). Reinhardt (1975), for example, found that physicians in group practices generated 5.0 percent more patient visits and 5.6 percent more patient billings than physicians in solo practices. A case study by Newhouse (1973) documented that slight economies of scale accrued to group practices but that the savings were offset by higher costs associated with X-inefficiency. Because of sharing arrangements, individual physicians were less likely to conserve resources.

Survivor analysis has also been used to test for economies of scale in physician practice. In this type of analysis, the fastest-growing size of practice for a given period of time is judged most efficient. The documented growth of large multispecialty group practice arrangements in the United States since the mid-1960s further attests to the production efficiency of this mode of practice (AMA 1996a; Frech and Ginsburg 1974; Marder and Zuckerman 1985).

Medical practice and technology have changed dramatically during the last 30 years, permitting many more surgical and diagnostic services to be done in an outpatient setting. Capital required to provide more sophisticated outpatient services can be financed and efficiently used in group practice settings. Studies of group-, staff-, and independent practice association–model HMOs have also provided evidence of economies

of scale in healthcare (Given 1996; Wholey et al. 1996). In addition to the production efficiencies of group practice, high practice start-up costs, greater competition in a period of growing supply and budget restraints, and national and state health policies that favor HMOs all point to continued growth in group medical practice.

Physician Payment. The RBRVS physician payment counterpart to the PPS for hospitals under Medicare was intended to reduce the rate of increase in physician expenditures under that program. There is some evidence that these policies initially reduced the rate of increase in physician costs and redistributed payments from procedural to primary care services. The difficulty lies in separating the effects of the RBRVS from other major changes taking place in healthcare. The change to managed care may be more important in explaining the rising incomes of generalists and the falling incomes of most specialists (AMA 1996b).

Total payments (program expenditure plus beneficiary cost sharing) for Medicare physician services reached $56 billion in 2001. These payments have increased at an average annual rate of 4.9 percent since 1991 and were expected to continue increasing at a rate of 2 to 4 percent per annum through 2006 (MedPAC 2003). This growth persisted even with negative updates of payment values due to the balanced budget act of 1997, which tied updates to growth in the national economy. Medicare physician expenditures were not being controlled by the administered price system and may result in substantial misallocation of resources due to errors of measurement and other problems associated with attempting to set relative prices for more than 7,000 procedures (Newhouse 2002b).

Hospital Services

Economies of Scale. Considerable research has been conducted on the degree to which community hospitals are subject to economies of scale. In ways similar to methods of physician services analysis, the methods of hospital cost function analysis have been somewhat crude, because measures of input and output do not take account of the great complexity of hospital-based care (Berki 1972; Cowing, Holtmann, and Powers 1983). Nevertheless, Feldstein (1998) concluded that slight economies of scale were taking place, with the optimum-sized community hospital at between 200 and 300 beds. Rigorous studies that

controlled for hospital differences and examined long-run cost functions raise questions about the existence of economies of scale and even suggest the possibility of diseconomies of scale (Fournier and Mitchell 1992; Hansen and Zwanziger 1996; Vita 1990). While hospitals in the United States are increasing in size, the average number of beds per hospital is still less than 200 (NCHS 2003, 299).

A more important issue for hospitals, and one that also raises the quality question, is the efficient size of a given service or department within the hospital (Getzen 2004; Grannemann, Brown, and Pauly 1986). Luft, Bunker, and Enthoven (1979) show a positive relationship between volume of heart surgery and outcome. Katz et al. (2002, 2003) report lower mortality and greater patient satisfaction among patients undergoing total hip replacement surgery in high-volume compared to lower-volume facilities and surgeons. These findings are consistent with a broad literature review of the volume outcome relationship (IOM 2000). This relationship is related to the development of so-called "focused factories" or specialty hospitals in the areas of cardiac care and orthopedics (Casalino, Devers, and Brewster 2003). These hospitals are poised to take advantage of specialization-driven economies of scale and volume-performance enhancements to outcome. However, there are serious concerns about the impact on overall access, quality, and cost when these organizations "cream skim" highly profitable procedures from community hospitals, which may then be forced to cut back on access to general medical care. The data on the impact of specialty hospitals is limited (The Lewin Group 2002).

Thus, in cases where higher service volume results in improved outcomes, efficiency can be improved by producing services at a lower cost per unit and by achieving more positive outcomes. Whether determined by regulation or market competition, such specialized services should be regionalized. Medicare, for example, instituted a program to regionalize heart surgery in "centers of excellence," and large, private-sector HMOs such as Kaiser have traditionally regionalized delivery of costly, infrequent procedures.

Payment Methods. While extremely difficult to demonstrate conclusively, PPS has generally been judged successful in containing hospital costs under Medicare without harming patients (Kahn et al. 1990; Russell 1989). However, one result of PPS was rapid expansion in outpatient hospital services, home care, and long-term care. Although hospitals

profited initially under PPS, subsequent restrictions on payment increases reduced operating profit margins to near zero and total margins to 4.4 percent by 1993 (AHA 1996; Guterman, Ashby, and Greene 1996; MedPAC 2003). Cost-cutting strategies improved total margins to 6 percent by 1996, but total margins subsequently declined to 3.4 percent in 2000 (MedPAC 2003). Hospital cost-containment strategies in the 1980s, including PPS, did not affect the underlying increase in healthcare costs in the United States. Reductions in the rate of growth in Medicare expenditures were offset by an increase in non-Medicare spending (Altman and Levit 2002; Schwartz and Mendelson 1991).

Health Plans

While input mix and economies of scale are important issues, the extent of service use by the population and the intensity of care are more important determinants of healthcare expenditure. These have been affected by significant changes in the financial and organizational structure of the healthcare system induced by managed care and related market competition (Glied 2003). Evidence regarding the dynamics and impacts of managed care on state, national, hospital, and employer expenditure trends are reviewed below.

State- and National-Level Expenditures. Research suggests that managed care plans reduced the overall rate of growth in healthcare costs in the 1990s by inducing price competition and reducing utilization and costs (Gaskin and Hadley 1997). Comparative state and national data confirmed that the development of managed care and competition was restraining the growth of medical care costs. Zwanziger and Melnick (1996) compared the cumulative growth in total real per capita health expenditures and in selected components of health expenditures for several states and the United States as a whole between 1980 and 1991. California was the state with the most highly developed managed care and the most competition during that period. Maryland, New York, and other northeastern states were among states with less competition and more regulation. California's healthcare cost growth rates were substantially lower. For example, the total percentage growth was 63 percent for the United States, 39 percent for California, and 59 percent for Maryland during that period. Comparable figures for hospital services were 54 percent, 27 percent, and 34.1 percent, respectively. California fared even better in the areas of physician services and drugs.

Other studies suggest that managed care may have lowered cost growth by also reducing the rate of technological diffusion (Baker 2001; Baker and Phibbs 2002) and by increasing production efficiency among tertiary hospitals (Brown 2003).

These state and national healthcare expenditure trends suggest that managed care played a leading role in abating cost growth in the heavily market-based U.S. healthcare system (Levit, Lazenby, and Sivarajan 1996). While payers seemed to appreciate the value associated with managed care, consumers and providers did not (Christianson and Trude 2003). Their backlash against these constraints coupled with a very tight labor market in the late 1990s resulted in a reduction in "tight" managed care, consolidation of providers, and reduction in capitation payment of providers (Center for Studying Health System Change 2002a). Given the underlying forces of rapid technological development, high consumer expectations, and the aging population, the forces of healthcare cost inflation were unleashed again, with employer health insurance premiums increasing at double-digit rates by 2001 (Strunk and Ginsburg 2003).

Hospital Expenditures. Evidence confirming the likely role of managed care in accounting for these trends is provided by research on the role of managed care in reducing overall hospital expenditures. J. C. Robinson (1996) examined the impact of HMOs on hospital capacity, utilization, and expenditures in California between 1983 and 1993 and reports that hospital expenditures grew less rapidly (44 percent) in markets with high HMO penetration than in markets with low HMO penetration. The majority of the reduced growth rates of hospital expenditures was the result of reduced volume and mix of services (28 percent), although some was due to reduction in intensity of service (10 percent) and reduced bed capacity (6 percent). The number of inpatient surgical procedures declined more quickly in markets with high HMO penetration, and the volume of outpatient procedures increased more slowly in these markets. There was a significant reduction in inpatient psychiatric days in high HMO markets compared to low HMO markets. J. C. Robinson (1996) also argues that the sustained impact of HMOs on hospital expenditures is most likely to be realized from consolidations of hospitals and reductions in excess capacity by hospital systems.

Research by Conrad et al. (1996) confirms the impact of various hospital managed care strategies on cost per hospital discharge.

Their analysis suggests that the proportion of hospital revenues derived from case or capitation payment was consistently associated with lower costs per discharge. Similarly, hospital approaches to providing resource-use information to clinicians and formal care management were related to lower costs per discharge and efficiency. Gaskin and Hadley (1997) also found that hospital costs declined in areas with greater HMO penetration, and Baker (2001) found that hospitals in areas with greater HMO penetration purchased fewer magnetic resonance imaging machines and that these machines were less likely to be used.

In contrast to these findings in the late 1980s and early 1990s, hospital outpatient spending was the key cost driver in the resurgence of healthcare cost inflation following the reduction in tightly managed care. Hospital costs increased 16.3 percent per capita in 2001 and 14.6 percent in 2002, reflecting increases in both payment rates and use of hospital services (Center for Studying Health System Change 2003; Levit et al. 2003).

Employer Expenditures. Large employers (those with 200 employees or more) attribute much of their success in slowing the growth of healthcare costs to the managed care and competition strategy pursued during the late 1980s and early 1990s. After an annual percentage growth in the upper teens in the employment-based insurance during the period 1988 to 1990, premium growth rates began to decline and were 0.5 percent in 1996, followed by two years of low growth of about 2 to 3 percent per year before accelerating to 12.5 percent growth in 2002 (Strunk, Ginsburg, and Gabel 2002). Wickizer and Feldstein (1995) used premium data to study the competitive effects of HMOs on indemnity plan premiums between 1985 and 1992. The degree of HMO participation in the market was found to have a significant and negative effect on the rate of growth in indemnity insurance premiums (5.9 percent growth in indemnity plan premiums rather than 7.0 percent with a 25 percent increase in HMO penetration). On the other hand, Feldman, Dowd, and Gifford (1993) found that including an HMO option in employment-based health insurance plans actually increased the average family premium by $25.14, as compared with premiums for plans offering only traditional fee-for-service indemnity insurance. Findings regarding the impact of tightly managed care on employee premiums, then, remain mixed. What is clear is that once managed care was loosened,

premiums began to increase to double-digit annual rates not seen since the early 1990s (Lesser, Ginsburg, and Devers 2003).

Plan-Level Utilization. Studies of the impact of HMOs on patterns of utilization of hospital and physician services provide evidence of the dynamic through which HMOs produce the system and institutional impacts on expenditures just reviewed. Based on a review and synthesis of early research on HMOs, Luft (1981) concluded that they provided care for 10 percent to 40 percent lower costs than comparable fee-for-service systems; the quality was no worse on average; and most of the savings were due to fewer hospital admissions. Through randomization into one large, well-established HMO, the RAND Health Insurance Experiment supported Luft's conclusion. It found savings of 25 percent with no adverse health effects on the general population, although persons who were initially sick and poor at the beginning of the experiment did fare better under the free fee-for-service plan than in the HMO (Ware et al. 1996).

Later reviews of research on HMOs and other forms of managed care by Miller and Luft (1994, 1997, 2002) confirmed that HMOs had lower hospital admission rates, shorter hospital lengths of stay, lower utilization of expensive procedures, and greater utilization of preventive services than traditional indemnity plans. The fear that HMOs were uniformly associated with poor quality care and the hope that they would lead to improvement in quality were not borne out in empirical studies (Miller and Luft 1994, 1997, 2002). Overall, enrollee satisfaction with services was lower in HMOs than in traditional fee-for-service plans, but HMO enrollees reported greater satisfaction with costs. The relatively high level of HMO enrollee satisfaction and somewhat greater use of ambulatory physician services by HMO enrollees was confirmed by subsequent empirical research (Blendon et al. 1994; Center for Studying Health System Change 2000, 2002a; Mark and Mueller 1996).

In summary, the 1990s growth in managed care and competition dramatically altered the economic incentives in healthcare. Providers were induced to consider adopting more efficient means of production, reduce prices, and provide care demanded by consumers and payers. Physician practice moved toward more efficient personnel mix and scale, the average size of hospitals increased, and very expensive and

difficult services were regionalized. These changes reduced the rate of growth in cost in the mid-1990s. These trends were reversed at the beginning of the new millennium because of very tight labor markets, a backlash against tightly managed care, and consolidation among providers. The initial reaction to increasing cost was to increase copayments to consumers and establish consumer-driven health plans with reimbursement accounts (Gabel, Lo Sasso, and Rice 2002; Robinson 2002). Indicators of improved health outcomes and expanded coverage for the population are lacking. Other democratic, developed countries appear to have been more successful at controlling spending, insuring their populations, and achieving health outcomes in the past. Whether they are truly more efficient or not is impossible to determine from aggregate data. Even so, attaining cost-control and access goals are important social achievements. Health services research can play a significant role in documenting the extent to which the emerging form of managed care and consumer-driven health plans in the United States are likely to improve the performance of the U.S. healthcare system.

CRITERIA FOR ASSESSING POLICY ALTERNATIVES IN TERMS OF EFFICIENCY

Policymakers strive to provide the right mix of regulation and market competition that will provide incentives and controls for consumers and their physicians in choosing efficient amounts and types of healthcare services and for providers in adopting efficient practices to deliver those services.

When evaluating policies or programs for efficiency concerns, several criteria might be applied (Table 5.5). These relate to both the macro level and micro level of analysis and concern both allocation and production issues. First, a state or nation is concerned about spending an appropriate fraction of gross domestic (or state) product on the health sector. Given the lack of any objective method to determine the allocation of resources that maximizes the well-being of the population, deciding what is "appropriate" is a political judgment. For many nations during the past 15 years, a guiding principle was that health spending should not increase faster than the rate of growth in national income or wages. This criterion reflects a judgment based on assessment of the health needs and the effectiveness of additional spending on health services—a growing share of national income spent on healthcare is not spent in the best interest of the society. This judgment of the

appropriate share of income going to healthcare will differ by state and over time, depending on the dominant political and economic circumstances. Any particular service, such as mammography, may warrant a faster or slower growth in spending, depending on the health returns per dollar of investment, compared to returns associated with other services.

Second, allocative efficiency requires a mix of health services that maximizes a combination of positive health outcomes and consumer satisfaction for the available share of resources expended on health services. A judgment must be made concerning the existing allocation of resources and whether it should be altered to obtain greater health and well-being for the society. The evidence reviewed earlier on the allocative efficiency of the U.S. healthcare system, for example, suggests that it suffers from an overinvestment in secondary and tertiary treatment and an underinvestment in population health–oriented disease prevention, health promotion, and health protection strategies relative to the observed, as well as to the likely, health return on these investments. Given that mammography screening is cost-effective and that at least 30 percent of women do not meet recommended guidelines for regular screening, shifting more resources toward increased screening and away from less cost-effective services may improve allocative efficiency.

Third, production costs should be minimized for health services in the interests of production efficiency. The output of the production process should consider the health of the individuals receiving care, their satisfaction with the method of service delivery, and any health consequences to others who may be indirectly affected by health programs. Costs should include the direct cost of services plus indirect time costs incurred by participants and other affected parties. Service quality and cost should therefore be compared to the comprehensive national benchmarks deemed to be the most efficient producers of the services in question. There are efficiency studies and related recommendations for the most cost-effective methods to achieve the recommended screening rates among alternative populations of women (Fishman et al. 2000; Saywell et al. 1999).

A number of organizations have developed information on standards of performance for healthcare providers (Cleverly 1997; Solucient 2003) and health plans (NCQA 2003a). For example, the Health Plan Employer Data and Information Set (HEDIS) was developed by the National Committee on Quality Assurance (NCQA) primarily for employ-

Table 5.5 Criteria for Assessing Health Policies in Terms of Efficiency

Dimensions	*Criteria*	*Indicators*	*Examples*
Macro cost control	Spend an appropriate fraction of gross state product on the health sector.	Health spending should not increase faster than the rate of growth in income in the state.	This criterion relates to aggregate spending on healthcare and is therefore not relevant to investments in any particular area of healthcare.
Allocative efficiency	Ensure a mix of health services that maximizes a combination of health outcomes and consumer satisfaction for the available share of resources expended on health services.	A majority of new health spending should be directed to disease prevention, health promotion, and improvement in the social and physical environment for persons likely to achieve the greatest gains from such investment.	The majority of new spending for breast cancer is allocated to basic biomedical research and clinical treatment, rather than breast cancer screening, environmental determinants research, and related interventions.
Production efficiency	Produce health services at a minimum cost.	Produce services at a cost at or below nationally recognized benchmarks of cost per unit of service.	Variant types of mammography screening reminder systems (e.g., general patient reminders, tailored patient reminders, provider reminders, joint patient-provider reminders) have yielded variant results in terms of increases in the number of screenings relative to costs.
Dynamic efficiency	Search for technological and organizational advances that raise the productivity of given resources.	Encourage research and development of new health services and efficient ways to organize and deliver them.	Significant research is underway in developing and testing new technologies for the early detection of breast cancer (e.g., IOM 2001b).

ers that required performance information for making a selection from competing managed health plans. The HEDIS instrument was subsequently adapted for use by the Medicaid program. It covers effectiveness of care, availability, satisfaction with care, cost of care, use of services, and plan-descriptive information. HEDIS tracks mammography screening rates for women 52 to 69 years of age. NCQA (2003b) reported a mean rate of 75.5 percent for commercial health plans in 2001 compared to 73.9 percent for Medicare and 54.9 percent for Medicaid in 2000.

Finally, a search should be made for technological and organizational advances that raise the productivity of given resources (i.e., dynamic efficiency). Policies should therefore encourage development of and experimentation with new services and with more efficient ways to organize and deliver healthcare, including mammography screening for the early detection of breast cancer (IOM 2001b).

SUMMARY AND CONCLUSIONS

Three major questions are addressed in this chapter: (1) What policy strategies are available to achieve efficiency? (2) What is the evidence regarding the efficiency of these strategies? and (3) What criteria should be used in judging the efficiency of policy alternatives?

In response to the first question, national healthcare systems can be positioned on a continuum ranging from market-maximized demand-based systems of healthcare to market-minimized need-based systems. The former is characterized by individual choice by consumers; nonuniversal coverage; private insurance; numerous sources of payment; high out-of-pocket payment; the private practice of medicine; and the private ownership of healthcare facilities, many of which are operated on a for-profit basis. The interaction of supply and demand forces within a market context, however imperfect, guides the allocation of resources within healthcare and between healthcare and other sectors. Market-minimized systems are characterized by community need-based determinations, universal and public coverage, relatively few sources of payment, low out-of-pocket payments, public practice or public control of private practice, and public ownership or control of healthcare facilities operated on a not-for-profit basis. Professional and bureaucratic determinations of need guide the allocation of resources to and within the health sector.

National healthcare systems generally have some combination of regulatory and market aspects. Many payment schemes (e.g., prospective payment of hospitals by DRGs) and utilization management schemes (e.g., managed care tools) have been developed to enhance efficiency. In response to the second and third questions posed at the beginning of the chapter, the evidence regarding the efficiency of these strategies, particularly the market-maximized strategy that dominates in the United States, will be reviewed in its relationship to the criteria for assessing efficiency outlined earlier (see Table 5.5): macro control of healthcare costs, allocative efficiency, production efficiency, and dynamic efficiency.

Macro Cost Control

The United States eschews the proven macro approach that stabilized health spending in Canada and western Europe as a percentage of gross domestic product by exerting direct government and community control over healthcare spending (European Observatory on Health Care Systems 2002). The United States continues to develop an unstructured market that minimally regulates insurers and gives providers and consumers strong incentives for making efficient decisions at the micro level. Cost control achieved through the macro approach does not necessarily translate directly into efficiency, but micro incentives have yet to demonstrate long-term cost control, making allocative efficiency uncertain.

Allocative Efficiency

Considerable evidence exists that the United States fails to achieve maximum value from the resources allocated to healthcare services and that it may perform less well than many other developed countries. Indicators include evidence on variability in the use of services; the lack of evidence-based best practices in the practice of medicine by most physicians (IOM 2001a); substantial underinvestment in selected preventive services, including prenatal care; a focus on procedure-oriented care that is costly and may add little to health at the margin; high rates of spending; and a relatively poor showing on many indicators of the U.S. population's health compared to that of other countries.[1]

Production Efficiency

Additionally, numerous studies have documented that hospital, physician, and insurance services are not produced in the most efficient manner, and comparative data suggest that prices and associated incomes

are set higher than necessary to attract the required resources to healthcare. Evidence includes excess capacity, a lack of attention to the most efficient personnel mix, and a failure to take advantage of potential economies of scale.

Dynamic Efficiency

As evidenced by the data comparing the healthcare investment experiences of different countries reviewed earlier, U.S. policymakers can perhaps begin to discern new answers to the troubling question of how best to improve the efficiency of the U.S. healthcare system. This effort at discernment can begin with a study of alternative models that have been developed on a state, regional, or national basis and with the development of a program to measure their effects on population health costs (Roos et al. 1996, 1999). Health services researchers and policy analysts can contribute to the fund of knowledge by assessing ways in which other countries have dealt with (1) the role of environmental, social, behavioral, and medical determinants of health in the formulation of health policy; (2) the design of population-oriented data systems directed toward assessing these policies; and (3) the extent to which the effectiveness, efficiency, and equity objectives of health policy have been or are likely to be achieved.

NOTE

1. Harvard researchers continue to track economic evaluations of medical care and public health intervention studies (www.hsph.harvard.edu/cearegistry). This is not to imply that all preventive services are cost-effective. Haddix, Teutsch, and Corso (2003) describes the methods to assess preventive programs on the cost-effectiveness criteria. Russell (1986) provides an excellent analysis of the economic assessment of preventive programs.

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Chapter 6

Equity: Concepts and Methods

CHAPTER HIGHLIGHTS

1. Equity is concerned with maximizing the fairness in the distribution of healthcare (*procedural equity*) and minimizing the disparities in health across groups (*substantive equity*).
2. Assessments of equity can be based on the *distributive, deliberative*, or *social justice paradigms*. The distributive justice paradigm focuses on individual rights to medical care; the social justice paradigm addresses the medical and nonmedical determinants of the health of populations as a whole; and the deliberative justice paradigm attempts to balance conflicts between these two perspectives by ensuring full participation of affected parties at all levels of decision making.
3. The principal equity criteria applied from each of these perspectives are *distributive justice*—freedom of choice and cost-effectiveness of healthcare services; *social justice*—similar treatment, common good, and need across populations; and *deliberative justice*—participation of affected parties in decision making.
4. Equity analyses entail gathering data on indicators of each of these criteria and the conduct of *descriptive, analytic*, and/or *evaluative* research to assess the performance or impact of healthcare programs and policies.

OVERVIEW

The fundamental questions posed in this chapter are (1) What is equity? and (2) How should equity in healthcare be assessed? Chapter 7 asks (3) To what extent has equity been achieved?

With the failure of universal health insurance reform in the United States and the dominance of managed care in the medical care marketplace, concerns with equity have surfaced along an array of dimensions. A large number of Americans are uninsured. Many public and private managed care plans have attempted to exclude or limit the coverage of particularly high risk groups. Policies and procedures, such as mandatory enrollment or "gag rules" regarding what providers are allowed to tell patients, have raised serious questions of fairness. Traditional safety-net providers in many communities face financial instability and risks of closure. Health and healthcare inequalities persist in areas ravaged by social, economic, and healthcare disinvestment.

A variety of approaches have been developed and applied in health services research and policy analysis to assess equity. They have focused primarily on potential or realized barriers to access to medical care, the extent to which subgroup variations exist in the utilization of medical care services relative to need, and the conceptual foundations of distributive justice and associated individual rights required to ensure equity (Aday and Andersen 1981; Aday et al. 1993).

These conceptualizations have, however, failed to encompass the weight of the empirical evidence regarding the limited role of medical care relative to other inputs or sectors for improving health and the corollary concerns with the common good and health of populations and communities. They have also failed to acknowledge or accommodate philosophical criticisms of the distributive justice and associated individual rights framework as a basis for judgments of equity, as well as criticisms of the fairness of the deliberative processes and procedures in policy debates on the allocation of public and private resources.

An implicit assumption underlying the perspective on equity presented here is that the conventional lenses for viewing equity have failed to penetrate the origins of, or envision other promising remedies for, the persistent health and healthcare inequalities that plague our national conscience. An explicit aim is to provide a broader and deeper vision of the foundations of fairness undergirding the formulation and evaluation of health policy. Alternative paradigms, or defining frameworks, of justice and their implications for conceptualizing and measuring equity will be presented to address the questions posed at the beginning of this chapter: What is equity, and how should equity in healthcare be assessed? The chapter that follows addresses the question, To what extent has equity been achieved?

CONCEPTUAL FRAMEWORK AND DEFINITIONS

Contrasting Paradigms of Justice

Three primary philosophical traditions, grounded in different paradigms of justice, can be used to illuminate the correlates and indicators of equity in health and healthcare. These include the distributive, deliberative, and social justice paradigms (Table 6.1). The paradigms focus, respectively, on individuals, institutions, and the community in judging justice (Daly 1994; Habermas 1996; Kolm 1996; Mulhall and Swift 1996). The major theories underlying the different paradigms are summarized in Table 6.2. These theories will be discussed later in this chapter in examining the theoretical basis for specifying empirical criteria of equity.

The distinctions between the individual and community perspectives are most deeply lodged in the debate between liberal and communitarian values. The liberal political tradition focuses on the norms of personal well-being and individual freedom. Policies grounded in this tradition have been concerned with protecting or ensuring individual rights and its underlying *distributive justice* paradigm. Rights are those benefits to which one has a claim, based on assessing what might be a fair distribution of benefits and burdens. This assessment encompasses a consideration of both negative and positive rights—that is, noninterference and freedom of choice, as well as a positive conferring of specific material or nonmaterial benefits. The question of equity posed from this point of view is, What can I justly claim?

This framework argues for minimal state interference in the organization and delivery of healthcare and maximizing freedom of choice and individual rights (i.e., a focus on the I). This perspective has guided policy debates regarding universal health insurance, Medicaid and Medicare reform, and the impact of immigration and welfare reform (Callahan 2001; d'Oronzio 2001; Hanson and Callahan 2001). The rising costs of medical care, the failure of universal healthcare reform at the national level, and the dominance of managed care in both the public and private sectors have raised significant questions regarding to whom and to what extent benefits of coverage might be extended, as well as how corresponding costs should be allocated. Increasing emphasis is being placed on consumer choice, personal responsibility, experience rating, actuarial fairness, and free riders. The answer to the question "What can I justly claim?" is more and more sharply focused on the attributes and actions of the I.

Table 6.1 Contrasting Paradigms of Justice

	Paradigm		
	Distributive Justice	*Deliberative Justice*	*Social Justice*
Focus	Individuals	Institutions	Community
Theory	Liberalism • Libertarian • Utilitarian • Contractarian	Deliberative democracy • Discourse	Communitarianism • Community • Egalitarian • Need based
Principles	Personal well-being Individual freedom	Public governance Popular sovereignty	Common good Social solidarity
Issues	What can I justly *claim?*	Who *decides*, and how?	What's *good* for us?
Policies	Minimalist state: Individual rights	Responsive state: Civic participation	Responsible state: Public welfare
Strengths	Promotes individual freedom Protects personal privacy Applies universal norms	Balances the strengths and weaknesses of distributive and social justice paradigms through rational public discourse (participation and empowerment)	Promotes social responsibility Protects public good Applies community norms
Weaknesses	Diminishes the social Sacrifices public for private Promotes self-centeredness Blames the victim	Great variability exists in the actual implementation of the principles of deliberative democracy at both the macro and micro levels	Diminishes the individual Sacrifices private for public Promotes dependency Encourages paternalism

Communitarian sentiments are based on norms of the common good, social solidarity, and protection of the public welfare (Daly 1994; Etzioni 1998, 1999, 2000a, 2000b, 2003a, 2003b). The concept of justice on which this perspective is based is concerned with the social, economic, and environmental underpinnings of inequity. Rather than focusing on conferring or ensuring positive or negative rights or benefits to individuals, this paradigm encompasses a broader consideration of public health and the social and economic interventions required to enhance the well-being of groups or communities as a whole. The essential question of justice posed from this perspective is, What's good for us?

The *social justice* paradigm is reflected in traditional public health policy and practice, with its emphasis on the public welfare and the use of medical police power (e.g., public health regulations, inspections, quarantines) to protect the population's health (Beauchamp 1976, 1985, 1988, 1998; Beauchamp and Steinbock 1999). Critics have argued, however, that public health planning and practice have focused less on what communities may view as good for them and more on what public health professionals determine communities need, based on agency- or administratively driven data-gathering or needs-assessment activities (Kretzmann and McKnight 1993; Labonte, 1994; Robertson and Minkler 1994). In many communities, the consequence is that the social, economic, and environmental issues that determine the health of the public are not adequately addressed, and the capacities of affected populations to ameliorate them are untapped or, at worst, undermined.

The distributive and social justice paradigms offer contrasting and complementary strengths as foundations for judging justice. The distributive justice paradigm ensures and promotes individual freedom and autonomy, protects personal privacy, and applies nondiscriminatory universal norms in identifying basic human rights. On the other hand, the social justice paradigm importantly balances considerations of social responsibility, the protection of the public good, and the application of community-centered norms and values in formulating program and policy priorities.

Criticisms of the distributive justice paradigm as applied to medical care and the social justice model underlying public health mirror the array of criticisms that have been raised about liberal and communitarian theories (Daly 1994; Habermas 1996; Mulhall and Swift 1996). The dominance of the liberal paradigm in shaping health and social

policy in the United States has, it is argued, served to weaken social and communal sentiments, such as civility and reciprocity; sacrificed considerations of the public good to serve private interests; promoted self-centeredness; and blamed the victim for circumstances that likely were created by society or others. On the other hand, communitarianism is charged with weakening individual autonomy; sacrificing the ability of the public to make rational, informed choices because of the increasing bureaucratization of what are judged to be paternalistic public institutions; and the attendant shift of individuals served by institutions into the role of dependent clients.

Contemporary social theorists, most notably German philosopher Jürgen Habermas, have addressed the weaknesses of the liberal and communitarian traditions by arguing for a new synthesis for the foundations for fairness, based on a theory of deliberative democracy (Habermas 1995, 1996, 2002, 2003). Policies attuned to this perspective address the extent to which norms of civic participation appear to guide decision making. The question of justice posed from this point of view is, Who decides, and how? The foundation for the enlargement of *deliberative justice* is the growth and promotion of a public sphere of secondary associations, social movements, and an array of civil and political forums for influencing the formal policymaking process. The deliberative justice paradigm recognizes and attempts to resolve conflicts rooted in the other dominant paradigms of fairness through rational discourse on the part of affected groups and individuals. Such discourse is oriented primarily toward mutual understanding. Habermas argues that strategic or technical-rational aims of decision makers at either the macro level or micro level (e.g., implementing a state Medicaid managed care program or achieving patient adherence to therapeutic regimens) are unlikely to be orchestrated and achieved unless affected stakeholders (e.g., providers, patients, taxpayers) have the opportunity to present and have their points of view heard and respected in the process.

A central criticism of the deliberative justice paradigm, however, is that it has been variably implemented in practice. Participation exists along a continuum from relatively passive consultative input on the part of patients and affected populations to a dominant, determining role in decision making at either the micro or macro level. Further, the impact of fuller participation of patients or the public in health policy

decisions affecting them in terms of health and well-being is not yet confirmed (Boyce 2001; Crawford et al. 2002; Morgan 2001; Wagner et al. 2000; Zakus and Lysack 1998).

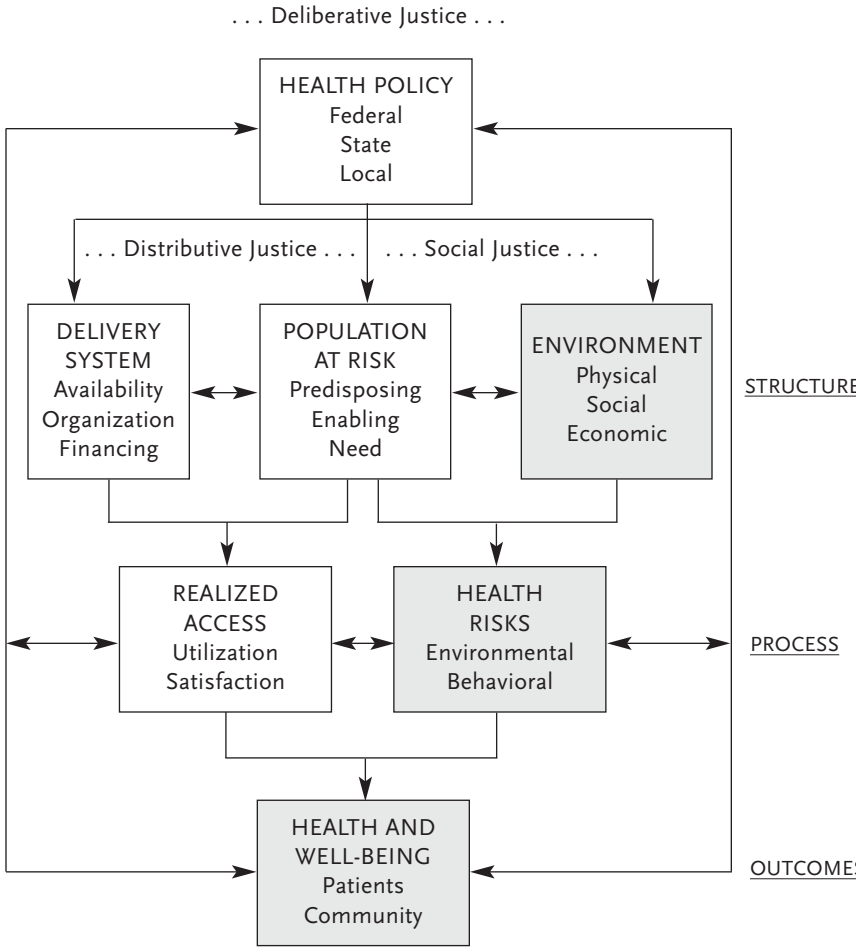
The discussion that follows presents a framework of equity, incorporating elements of the deliberative, distributive, and social justice paradigms—and the relationships implied among them—as a foundation for guiding health services research on the equity of healthcare provision. The framework attempts to acknowledge and integrate the complementary strengths of each of the respective paradigms.

A Conceptual Framework of Equity

Figure 6.1 displays how the conceptual framework for guiding health services research and policy analysis introduced in Chapter 1 might reflect and integrate the deliberative, distributive, and social justice paradigms. The unshaded boxes represent a conceptual model of equity of access to medical care developed by Ronald Andersen, Lu Ann Aday, and their colleagues to guide the conduct of national and community surveys of access; this method is rooted in the distributive justice paradigm (Aday and Andersen 1981; Aday, Andersen, and Fleming 1980). The shaded boxes represent components that are reflective of the broader social justice paradigm, grounded in research on the social-structural factors that influence the health and healthcare needs of vulnerable populations (Aday 2001; Beauchamp 1976, 1985, 1988, 1998; Beauchamp and Steinbock 1999).

The original access framework begins with the role of health policy in influencing the characteristics of the health delivery system and the population to be served by it. A dimension in the expanded model is the deliberative justice character of health policy that focuses on the institutions and procedures through which policy is formulated and implemented. Placing the governing norm of deliberative justice above health policy in the expanded framework is intended to convey that conflicts between the disparate paradigms of distributive justice and social justice—which have tended to guide medical care and public health policy, respectively—must be effectively addressed if the health and well-being of individuals and communities are to be enhanced. Ensuring that those most affected by health policy decisions at both the macro level and micro level are involved in shaping them constitutes the means for doing so. The deliberative paradigm has not been

Figure 6.1 Conceptual Framework of Equity



fully explored as a basis for the equity of health policy. It is, however, implicit in the focus on consumer involvement and community participation in the design and implementation of private and public health programs in the United States and other countries (Minkler 1997; Minkler and Wallerstein 2003).

The unshaded boxes in Figure 6.1, encompassing the delivery system, population at risk, and realized access, define the major distribu-

tive justice components of the conceptual framework that has guided much of health services research on access to medical care (Aday and Andersen 1981; Aday, Andersen, and Fleming 1980). Relevant characteristics of the health system include the availability, organization, and financing of services. Predisposing characteristics of the population at risk include those that describe the propensity of individuals to use services, including basic demographic characteristics (e.g., age, gender), social-structural variables (e.g., race and ethnicity, education, employment status, occupation), and beliefs (e.g., general beliefs and attitudes about the value of health services and/or knowledge of disease). Enabling characteristics include the means individuals have available to them for the use of services. Both financial resources, such as family income or insurance coverage, and organizational resources, such as having a regular source or place to go for care, specific to the individuals and their families are relevant here. Need refers to health status or illness as a predictor of health service use. The need for care may be perceived by the individual and reflected in reported disability days or symptoms, for example, or may be evaluated by a provider and reflected in actual diagnoses or complaints.

Realized access refers to the objective and subjective indicators of the actual process of seeking care. These are, in effect, indicators of the extent to which the system and population characteristics predict the demand for care (i.e., how much care is used, if any) and how satisfied potential or actual consumers are with the healthcare system.

As indicated by the shaded boxes in Figure 6.1, in the social justice component of the model, there is an explicit acknowledgment of the ultimate outcome of interest that was only implicit or assumed in the original model: the health and well-being of individuals and communities. The model acknowledges that the physical, social, and economic environment in which individuals live and work can also have consequences for their access to health and healthcare. The model also indicates that the environment directly influences the likelihood of exposures to significant environmental and behavioral health risks.

The social justice component of the model may be viewed as focusing on the community level of analysis. It primarily examines the characteristics of the physical, social, and economic environment; the population residing within it; and the health risks that the population experiences as a consequence. The distributive justice component of

the model relies on individuals as the ultimate unit of analysis. Their attributes and behavior may, however, be aggregated to reflect the characteristics of patients within a given health system or delivery organization or of the population resident within a designated geographic area. The distributive justice paradigm has led to an emphasis on the equity of the medical care delivery system, while the social justice paradigm is reflected in public health and in social and economic policies directly or indirectly related to health.

The goal of health policy, as indicated in the expanded equity framework, is to contribute to improving the health of individuals and communities. The ultimate test of the equity of health policy from the social justice perspective is the extent to which disparities or inequalities in health among subgroups of the population are minimized (Lurie 2002). Substantive equity is reflected in subgroup disparities in health. Procedural equity refers to the extent to which the structure, process, or procedures intended to reduce these disparities may be judged to be fair, grounded in norms of deliberative, distributive, and social justice. The normative import of these procedural factors for substantive equity can be empirically judged by the extent to which these factors are predictive of inequalities in health across groups and communities. The equity framework (Figure 6.1) is intended to provide normative and empirical guidance for assessing both substantive and procedural equity.

Based on the synthesis and integration of the theoretical underpinnings of substantive and procedural equity reviewed here, the answer to the first question posed at the beginning of this chapter—What is equity?—may be summarized as follows: equity is concerned with health disparities and the fairness and effectiveness of the procedures for addressing them. The response to the second question—How should equity in healthcare be assessed?—is this: examine and account for health disparities.

The effectiveness of medical and nonmedical investments in producing health is essentially an empirical question. The fairness of the means for doing so is a normative one. The expanded framework, however, implies that health policymaking must take into account norms of distributive and social justice and that conflicts between affected stakeholders grounded in these contrasting norms must be resolved through deliberative discourse if the resultant policies are ultimately to contribute to improving health and minimizing health disparities. Both the effectiveness and equity criteria demand it.

KEY METHODS OF ASSESSING EQUITY

Conceptual Frameworks

Conceptual frameworks provide useful analytic guidance for selecting empirical indicators and generating hypotheses about interrelationships between them. The framework Aday, Andersen, and their colleagues developed for the study of access has guided a great deal of research on equity (Aday and Andersen 1981). Integral to that framework is the value judgment that the system would be deemed fair or equitable if need-based criteria, rather than resources such as insurance coverage or income, were the main determinants of the amount of care sought. As indicated earlier (and displayed in Figure 6.1), the developers as well as others have continued to extend their framework to encompass social and environmental factors, as well as access to medical care, as factors that ultimately influence health. They continue, furthermore, to acknowledge the interdependence of the equity, effectiveness, and efficiency norms in assessing the performance of health policies and programs (Aday 2001; Andersen 1995; Andersen and Davidson 2001; Bradley et al. 2002; DuPlessis, Inkelas, and Halfon 1998; Gelberg, Andersen, and Leake 2000; Lurie 2002; Phillips et al. 1998).

Other frameworks have been developed that are useful in exploring the distributive, social, or deliberative justice paradigms in the context of the growth of managed care. In general, frameworks grounded in the distributive justice paradigm may be seen as primarily turning inward, assessing the fairness of the healthcare system for the patients directly served by the system. Social justice–oriented frameworks direct attention outward to the community to assess the equity of health and health risks of the population who reside within the community. Conceptual approaches to equity influenced by the deliberative justice paradigm attempt to enhance the dialog between those who design and those who are affected by health policies, in order to develop more effective policies.

Frameworks grounded in these respective paradigms of justice, their relationship to the expanded framework of equity (Figure 6.1), and the defining focus of each will be reviewed in the discussion that follows.

Distributive Justice

Docteur, Colby, and Gold (1996) developed an access framework that identifies a variety of components that are relevant in influencing and

assessing access for managed care enrollees. The framework includes the structural, financial, and personal determinants of patients' plan selection; the associated characteristics of the health plan delivery system itself; the influence of these patient and plan characteristics on plan choice and subsequent use of services; the mediators and determinants of the continuity of plan enrollment; and the ultimate clinical and equity outcomes for enrollees and users. This framework then focuses the lens of distributive justice on the availability, organization, and financing of services within a particular delivery system and the utilization and satisfaction of individuals and their families who choose to enroll in it.

Social Justice

A growing body of research documents the role of fundamental social and economic factors in influencing health and the accompanying need for integrative analytic frameworks to guide more innovative health-centered social and economic policy development (Albrecht, Freeman, and Higginbotham 1998; Graham 2002; Link and Phelan 1995; Spitler 2001). Aday's (2001) framework for the study of vulnerable populations delineates the social and economic factors determinant of health risks and argues for community and individual levels of analyses in exploring the correlates of vulnerability to poor health. Her perspective argues for the development of a broader continuum of health services, encompassing prevention-oriented services, long-term community-based services, and acute medical care services (displayed in Figure 1.1) to address the health and healthcare needs of the most vulnerable. The U.S. Public Health Service and the World Health Organization (WHO) Year 2010 objectives and accompanying empirical and programmatic emphases also provide guidance for identifying and tracking the indicators and predictors of subgroup disparities in health (NCHS 2002, 2003; WHO 2002).

Stephen Shortell, based on his and his colleagues' research on organized (or integrated) healthcare delivery systems, has argued convincingly for the importance of a population health–oriented perspective in designing and assessing these systems (Shortell, Gillies, and Anderson 1994; Shortell et al. 2000). There is, however, wide variability in the de facto implementation of a population health perspective in emerging systems of care (Kindig and Stoddart 2003). A focus on the health of populations and the integrated array of programs and services needed

to address the health needs of the most vulnerable provides conceptual and analytic guidance for assessing the extent to which the health of communities as a whole is enhanced in the evolving managed care environment. (These issues are discussed in the context of the criteria for assessing the effectiveness of healthcare in Chapter 3.)

Deliberative Justice

Community participation and empowerment have ostensibly been central components of the design of social and health programs in the United States as well as in other countries. The extent to which individuals affected by these initiatives have been fully involved in shaping them, however, has often been less than fully realized in practice. Widespread criticism exists that public health and health promotion professionals have often imposed interventions they deem necessary on selected target communities or populations without either soliciting or fully taking into account the wants of the affected groups and individuals (Israel et al. 1998). Program developers may claim that communities have been involved in shaping such interventions when, in fact, there has been little or only token participation on the part of affected groups.

Habermas's discourse theory provides a template for examining the nature of these exchanges and the aims and actions of the institutional and individual actors involved in them. For Habermas, communication directed toward a mutual understanding between affected parties can best establish the foundations of trust and collaboration needed for solving their common problems despite their potentially different points of view (Habermas 1995, 1996, 2002, 2003). Opportunities for analyzing the form and quality of participation may range from the microcosm of the patient-physician relationship to the design of consumer-oriented healthcare programs and neighborhood services or communitywide needs assessment and program development efforts to broader social change–oriented movements that have important impacts on the health of individuals and communities (e.g., environmental justice, AIDS advocacy) (Labonte 1994; Waitzkin, Britt, and Williams 1994). The fairness of healthcare programs and policies—assessed through qualitative interviews or more structured quantitative scales of participation to key informants—may be judged by the extent to which affected parties are involved in shaping them. (Some of these approaches will be discussed later in this chapter in reviewing selected empirical indicators of equity.)

The discussion that follows reviews how the dimensions of equity reflected in these respective frameworks would be operationalized in the conduct of health services research to assess the extent to which equity has been achieved.

Criteria and Indicators of Equity

Table 6.2 highlights the major theories and related principles of justice underlying the principal justice paradigms to provide guidance for identifying specific empirical criteria of equity. Table 6.3 summarizes empirical indicators of equity in relationship to the primary dimensions of the expanded equity framework (Figure 6.1) and to the related criteria of justice underlying them. Data may be gathered to descriptively assess dimensions of procedural and substantive equity as well as to conduct analytic or evaluative research, exploring those factors that are most predictive of the persistent, substantive inequities mirrored in subgroup variations in health. The ultimate test of equity is the extent to which these disparities are minimized. The challenge to analytic and evaluative public health and health services research is determining how best to design studies and gather data to assess the factors most likely to influence this endpoint and the health policy interventions suggested as a consequence. Later in this chapter and in Chapter 7, the methods and empirical evidence for assessing the extent to which these criteria have been realized and their import for reducing health disparities will be presented.

Health Policy

Criterion: Participation. Habermas's discourse theory is most directly concerned with the extent to which those likely to be affected by decisions participate in shaping them (Habermas 1996). The defining normative underpinning for Habermas's theory is grounded in his discourse principle: "Only those norms are valid to which all affected persons could agree as participants in rational discourse(s)" (Habermas 1996, xxvi). "Rational discourse" in this case refers to communication directed toward mutual understanding, rather than strictly ends-oriented communication directed toward instrumental (i.e., technical-rational or strategic) aims. Habermas's discourse principle is grounded in fundamental democratic ideals, in which the power to govern is ultimately vested in the people and exercised by them directly or indirectly through

Table 6.2 Theories Underlying Paradigms of Justice

Theory (major theorists)	*Justice Principles*	*Health Policy Focus*
Liberalism		
Libertarian *(Robert Nozick)*	**Property** *Entitlement:* Individuals are entitled to what they possess, provided they acquire and transfer it through just means. *Libertarianism:* The state should enforce these property rights and *not* interfere in redistributing assets (i.e., let "the invisible hand" work).	**Free Market**
Utilitarian *(Jeremy Bentham, David Hume, John Stuart Mill)*	**Payoffs** *Utility:* Promote the greatest good for the greatest number. *Consequences:* Gauge the worth of actions by their consequences (i.e., ends justify means).	**Cost-benefit/ Cost-effectiveness/ Cost-utility analysis**
Contractarian *(John Rawls)*	**Priorities** 1. *Greatest equal liberty:* Every person should have an equal right to the most extensive system of equal basic liberties available to all. 2. *Fair equality of opportunity:* Persons with similar skills and abilities are to have equal access to offices and positions. 3. *Difference principle:* Social and economic institutions should be arranged to maximally benefit the *least well off*.	**Social exclusion**
Communitarianism		
Community *(Dan Beauchamp, Amitai Etzioni, Alisdair MacIntyre, Michael Sandel, Michael Walzer)*	**Public good** *Social solidarity:* The unity produced by or based on community, group or class interests, objectives, and values is primary. *Common good:* The well-being of the community as a whole (commonweal) must be protected and ensured.	**Public health**

Table 6.2 Theories Underlying Paradigms of Justice *(continued)*

Theory (major theorists)	*Justice Principles*	*Health Policy Focus*
Egalitarian *(Robert Veatch)*	**Personhood** *Equal worth:* Principle of equality rests on the assumption of the equal intrinsic worth of *all* human beings. *Equal opportunity:* Everyone should have an opportunity for access to health and healthcare equal, as far as possible, to another person's.	**Health and health care disparities**
Needs based *(Norman Daniels)*	**Potential** *Normal species functioning:* Meeting health and healthcare needs helps to maintain normal species functioning. *Fair equality of opportunity:* Society should ensure fair equality of opportunity of access to normal species functioning.	**Health and healthcare objectives for the nation**
Deliberative Democracy		
Discourse *(Paulo Freire, Jürgen Habermas)*	**Participation** *Discourse principle:* Only those norms are valid to which all affected persons could agree as participants in rational discourse. *Balance of individual and group interests:* Laws and institutions must balance private and public autonomy (i.e., self- and societal rule).	**Participation and empowerment**

a system of representation, involvement in a public political sphere, and free elections. The discourse principle characterizes policy or development activities that are oriented toward gaining a reasonable consensus about the definition of the problem and the best possible ways to address it on the part of the stakeholders most likely to be affected by the resulting policy. Communication grounded in mutual respect among stakeholders is essential to ensuring the realization of this principle in the formulation of policy at the micro level or macro level. For

Habermas, the foundations of trust and collaboration required to be successful in addressing more instrumental aims are established through such "communicatively rational" discourse. These norms of deliberative justice would be attended to at the micro level in forging effective patient-physician relationships, in shaping culturally sensitive service provision at the institutional level, and in ensuring the full participation of affected populations in the design of health policies and programs at the system and community levels.

This philosophical and programmatic thrust, as well as the parallel participatory action research agenda, developed by Brazilian social activist Paulo Freire (Freire 1970, 1995), are intended to encourage researchers and policymakers to more fully listen to and learn from communities. Communication with and involvement of affected parties in the design and implementation of programs are seen as essential. This emphasis acknowledges that by giving voice to concerns in their own syntax and semantics, affected parties learn together how best to address their concerns. This perspective is manifest in the formulation and implementation of community-based health education and health promotion initiatives (Minkler 1997; Minkler and Wallerstein 2003).

Norman Daniels has argued for incorporating the norms of "deliberative democracy" in developing managed care policies and procedures. By that, he means that participation on the part of affected parties (e.g., patients, providers) must be assured in decision making regarding the protection of normal functioning of a given population within defined constraints (e.g., limited resources). This perspective would, for example, oppose the "gag rule" that inhibits physicians providing full information to patients about their treatment options, make explicit the rationale for decisions about covering new technologies and provide an opportunity for public discussion of this rationale, and streamline and make less adversarial patient grievance and dispute resolution procedures (Daniels 1996, 1998, 2001; Daniels and Sabin 2002; Daniels, Kennedy, and Kawachi 2000).

Indicators: Participation. Empirical indicators of deliberative justice attempt to express the type and extent of involvement of affected groups' participation in formulating and implementing policies and programs. Arnstein (1969) conceptualized a ladder of citizen participation, with each respective rung representing a gradient ranging from nonparticipation to tokenism to increased levels of citizen power and control.

Charles and DeMaio (1993) incorporated this and other dimensions reflecting the perspective being adopted (i.e., that of a user versus a policymaker) and the decision-making domain (i.e., individual treatment, overall service provision, or macro policy formulation) in constructing a framework for assessing lay participation in healthcare decision making. Promoting lay participation and empowerment has been a particular focus of health education and health promotion activities in the United States, Canada, and other countries. Related indicators with particular relevance in the managed care context focus on the nature and quality of communication between patients and providers, the extent to which norms of "deliberative democracy" guide the development of organizational policies and procedures, and the magnitude of trust that healthcare consumers have for providers or organizations (Daniels 1996; Mechanic 1996).

Critiques of the shift to a population health perspective in Canada and the United States have, however, argued that this has led to a diminishment of a focus on the active engagement of populations and patients in health policy and program development at the macro and micro levels. This is, they argue, because of the fact that, in contrast to the health promotion movement, population health researchers document but do not identify participatory political mechanisms for addressing the fundamental determinants of health and health disparities (Coburn et al. 2003; Hawks 1997; Raphael and Bryant 2002).

Delivery System

Criterion: Freedom of Choice. The freedom-of-choice norm emphasizes the importance of personal autonomy in determining who receives what care. This criterion conforms most closely to Nozick's libertarian theory of justice, which emphasizes that equity is rooted in the freedom to possess and use one's property and resources as one chooses (Nozick 1974, 1990, 1993). People are entitled to what they have as long as they acquire or transfer it through just means—that is, through their own labor or as a result of a gift, an inheritance, or a voluntary exchange with others. Further, the state should not interfere with or attempt to regulate these transactions. Instead, the "invisible hand" governing the free marketplace should be allowed to operate unhindered. The only appropriate intrusions would be made to correct situations in which there is clear historical evidence that the property or resources some

people possess were not acquired through just means (such evidence is often difficult to assemble or document, however). This perspective endorses policies that maximize consumer preferences (i.e., choice and satisfaction) in the medical care marketplace. Proponents of this approach endorse the operation of market-based forces of supply and demand for the allocation of healthcare.

Indicators: Freedom of Choice. Empirical indicators of access based on the freedom-of-choice norm are the distribution and availability of healthcare resources to consumers. For example, personnel- (e.g., primary care physicians or specialists) and facility- (e.g., hospitals or hospital beds) to-population ratios and related inventories of healthcare personnel or providers (e.g., HMOs, PPOs) in a given target, or market, area are indicators of the basic supply of providers and delivery sites available to consumers.

Lists of preferred providers affiliated with employer-sponsored health insurance plans also effectively define the range of enrollees' choices for a regular source of medical care. Other indicators of the extent to which patients' decisions may be constrained include data on the hours of clinic operation and provider availability at night, on weekends, or in emergencies; the average distance to the nearest medical facility and the available modes of transportation for getting there; and the average time it takes to get an appointment, as well as the waiting time to see a physician or other provider once on site.

Characteristics of the system of financing in an area—such as the type and scope of benefits provided by major employers and the local public or private arrangements for people who have no third-party coverage—also dictate the options consumers can realistically afford. Substantial cost-sharing provisions or uncovered healthcare expenses also can result in decisions to forgo goals or sacrifice personal resources intended for other uses—such as an elderly woman using her and her husband's life savings (or "spending down") so that he can qualify for nursing home coverage under Medicaid.

Realized Access

Criterion: Cost-Effectiveness. Utilitarians advocate access to those services for which the measured benefits (e.g., in terms of health, well-being, or productivity) would be maximized relative to the costs necessary

to provide them. Utilitarian theory has its origins in the writings of David Hume, Jeremy Bentham, and John Stuart Mill (Culyer 1992; Kolm 1996). It is principally consequentialist or ends oriented. The value of any decision or action is judged by its consequences; the principal goal is to maximize utility or individual preferences. Based on the utilitarian theory of judging the rightness or wrongness of actions by the balance of benefits and burdens produced, cost-efficiency analyses, cost-benefit analyses, and associated cost-utility analyses (discussed in Chapter 4) have become an increasing focus in weighing the types of programs that should be funded and the categories of services to be covered under public or private insurance schemes and in determining whether the services actually being used are appropriate, effective, and satisfactory to consumers.

Indicators: Cost-Effectiveness. Components of the costs and benefits of care are reflected in the type and comprehensiveness of services received and the level of patient satisfaction relative to some standard. The Institute of Medicine Committee on Monitoring Access to Personal Health Care Services, for example, developed a set of indicators of services likely to have beneficial health consequences if used—including an array of preventive services (e.g., prenatal care, immunizations, breast cancer screening), as well as timely and appropriate care for acute or chronic illness (IOM 1993). These and other indicators were confirmed in later Institute of Medicine committee reports documenting the resulting impact of disparities in access to essential preventive and/or treatment services on disparities in the quality and effectiveness of care by race/ethnicity, socioeconomic status, and urban-rural place of residence (IOM 2002, 2003). The U.S. Public Health Service Year 2010 Objectives for the Nation also encompass a series of goals regarding the use of preventive services (NCHS 2003). These provide foundations for systems of monitoring the extent to which these services are received. A variety of scales for measuring satisfaction with medical care, physicians, and hospitals have been employed extensively in developing report cards or other reports on provider performance (Gold and Wooldridge 1995; Rao, Weinberger, and Kroenke 2000; Wensing and Elwyn 2002; Wensing and Grol 2000). These scales and the standardized reporting systems being developed offer an opportunity to assess the extent to which effective care has been rendered from the point of view of patients or the

general public. Critics of existing measures of patient satisfaction point out the need for the design of instruments that more directly engage those surveyed in identifying the key issues and questions to be addressed in such studies (Harris et al. 2001).

Population at Risk

Criterion: Similar Treatment. The similar-treatment criterion emphasizes that age, sex, race, income, or type or amount of insurance coverage should not dictate that people with similar needs enter different doors (e.g., private physicians' offices versus hospital emergency rooms) or be treated differently in terms of the type or intensity of services provided. This criterion is a defining tenet of the egalitarian concept of justice. From an egalitarian point of view, the perspective that all individuals are of equal worth and should be treated equally is of primary importance. As Robert Veatch (1981, 1989) points out, egalitarianism may focus on either procedural or substantive equality (i.e., similarity in treatment or outcome, respectively). Procedural equality ensures equal opportunity for every individual to obtain care, regardless of personal characteristics such as age, gender, race, income, type of coverage, or whether one lives in the city or suburbs. Substantive equality emphasizes minimizing the health status differentials or variations between groups, such as disparities in infant mortality between black and white populations. Considerations of equity from an egalitarian point of view focus on how to narrow or eliminate these disparities in health and medical care.

Egalitarian norms have also been central to the social justice paradigm in the context of examining varying exposures to health risks as a function of environmental, social, or economic conditions. Research on environmental justice has, for example, documented that toxic and hazardous waste sites are more likely to be located in racially segregated or socioeconomically disadvantaged neighborhoods (Brown 1995; Bullard 2000; Evans and Kantrowitz 2002). "Social exclusion" programs and policies take into account what can happen when people or areas differentially suffer from a combination of linked problems such as unemployment, poor skills, low incomes, poor housing, high-crime environments, bad health, and family breakdown. The Social Exclusion Unit in the United Kingdom, for example, was set up by the prime

minister's office to help improve government action to ameliorate the impact of these combined and differential risks (Office of the Deputy Prime Minister, Social Exclusion Unit 2001).

Indicators: Similar Treatment. The similar-treatment norm attempts to evaluate intergroup differences that may indicate inequalities in health or access to care. The convenience and characteristics of the places to which people go for medical care provide data on whether there is differential treatment of individuals in these different settings. Nonmedically motivated transfers of patients, or "dumping," principally as a function of fiscal rather than physical diagnostics, or so-called "wallet biopsies," are indicative of inequity under the similar-treatment norm. Certain institutions or providers assuming a disproportionate burden of uncompensated care for the medically indigent population calls into question whether they are assuming more than their fair share from an egalitarian point of view. Health inequalities and the factors that give rise to them also surface as issues under the similar-treatment norm. To the extent that differential exposures or access to resources for obtaining services give rise to these inequalities, they would be judged inequitable from this perspective (IOM 2002, 2003).

Environment

Criterion: Common Good. The concept of the common good is grounded in communitarian theory and focuses on the community as the unit of analysis (Daly 1994; Mulhall and Swift 1996). The primary normative referents are the well-being, or welfare, of communities and the criteria of social solidarity, or unity, and the common good. These norms find expression in more universal modes of financing medical care; in traditional public health policy and practice, with its emphasis on promoting and protecting the health of the public; and in investments in the array of institutions and resources such as families, schools, businesses, and government that are essential for maintaining the health and vitality of communities as a whole. The role of interventions is not on altering individual actions and motivations, but on the distal, foundational roots of health problems, such as the social-structural correlates of health and healthcare inequalities rooted in the physical, social, and economic environments in which individuals live and work. Health risks in the physical environment include toxic and environmental con-

taminants transmitted through the air, soil, or water in a given neighborhood or community. The social environment encompasses a look at the social resources, or social capital, that may be available to individuals associated with the family structure, voluntary organizations, and social networks that both bind and support them. The economic environment encompasses both human and material capital resources, reflected in the schools, jobs, income, and housing that characterize the community (Aday 2001; Mooney 1998; Robertson 1998).

Indicators: Common Good. Empirical indicators related to the common good encompass a look at the array of social status, social capital, and human and material capital resources available to the population at risk in a given area, as well as the significant physical environmental exposures that are likely to exist. Ensuring health protection is one of the U.S. Public Health Service Year 2010 Objectives for the Nation and is measured by a series of environmentally related health indicators (e.g., unintentional injuries, occupational safety and health, environmental health, food and drug safety) (NCHS 2003). The WHO Year 2010 Health for All program sets forth indicators for tracking the social, economic, and physical environments and their influence on health (WHO 2002).

Health Risks and Health

Criterion: Need. Norman Daniels's needs-based theory of justice points out the factors that are necessary to address minimal human needs for "normal species functioning" (Daniels 1985). Health policy initiatives are justified in terms of their role in ensuring that there is an equality of opportunity for living a normal life. This perspective prompts consideration of what such needs might be and of the basic decent minimum set of services that should be provided to meet them. Daniels suggests the following for consideration: adequate nutrition and shelter; sanitary, safe, unpolluted living and working conditions; exercise, rest, and other features of a healthy lifestyle; preventive, curative, and rehabilitative personal medical services; and nonmedical personal and social support services. The basis for deciding what goods and services might be included and how they could be fairly distributed remains controversial, however.

John Rawls's contractarian theory is based on an argument regarding what reasonable people would decide if they were asked to come

together to derive a fair set of criteria for distributing societal goods, operating under the hypothetical assumption that they could by chance be in any position in a society in which such criteria would be applied—including the least socially or economically advantaged (Rawls 1971, 2001a, 2001b). Rawls reasoned that under these circumstances, the following criteria would be endorsed, in order of importance: (1) maximize everyone's rights to liberties compatible with a similar system of liberty for everyone, (2) ensure fair equality of opportunity for people with similar abilities and skills, and (3) make sure that those who are the worst off benefit. The first two criteria have a strong egalitarian orientation, and the third emphasizes that if any group "counts" more than another, it is those who are worst off, financially or otherwise. This perspective focuses on those least able to buy care or be cured.

Daniels's needs-based theory, as well as the difference criterion recognizing the needs of the least well off within contractarian theory, lend support to a primary focus on meeting basic needs. Assessing who needs care may be both difficult and expensive (Braybrooke 1987, 1998). Economic theory argues that expressed demand is the most rational basis for allocating scarce medical care resources. Needs may, in fact, be quite subjective and ungovernable, unless constrained by some sense that people are willing to pay to have their tastes and preferences satisfied. Further, societal or professional consensus may be required to determine which needs to meet when resources are limited. Needs assessments have been an important component of public health–oriented planning and program development activities at the community level. Contemporary needs assessments focus on inventorying the assets, as well as the problems, that exist in the target communities of concern (Kretzmann and McKnight 1993).

Indicators: Need. Indicators of equity from the perspective of need attempt to assess the magnitude of health risks and health disparities in a population. Sometimes survey respondents are asked questions to obtain their subjective perceptions of the extent to which their needs have been met: "During the past year, did you or a family member need to see a doctor but not see one for some reason? If so, why?" Other indicators of need summarize respondents' objective reports of the number of physician visits relative to the number of disability days they experienced in the year (i.e., the use-disability ratio) or compare the

number of people who actually contacted a physician for a set of symptoms with the number of people that a panel of physicians thought should have seen one (i.e., the symptoms-response ratio) (Aday, Andersen, and Fleming 1980). The quality and outcomes of care are directly linked to the utilization of appropriate services to address identified needs (IOM 1993, 2002, 2003).

The social justice perspective on substantive equity, based in the need criterion, is concerned with subgroup variations in health. The primary goals of the U.S. Public Health Service Year 2010 Objectives for the Nation include increasing the span of healthy life for all Americans as well as reducing health disparities between and among groups (NCHS 2003). Indicators of mortality, morbidity, and years of potential life lost or quality years of life gained are illustrative of the types of indicators that could be used to trace trends and subgroup variations reflective of the extent to which needs are actually met. There is an increasing interest as well in developing summary measures of population health that integrate mortality and morbidity data, such as health-adjusted life expectancy or other quality-adjusted life-year estimates (IOM 1998; Kindig 1997, 1998; Murray et al. 2002).

In summary, an array of empirical indicators might be developed and used in assessing the equity of health policy design and implementation. Health services research and policy analysis can assist in the conceptualization and measurement of these indicators and in determining what factors appear to be most predictive of the ultimate equity outcome of interest—reducing subgroup disparities in health.

Data Sources

As implied in the expanded conceptual framework of equity (Figure 6.1), studies of equity could focus on the delivery system as a whole, particular institutions within it, groups of patients, the communities that are the target of health policy initiatives, or various combinations of these levels and their interrelationships. Further, studies could be carried out at the national, regional, state, or local level. Such studies may entail collecting new (primary) data, as well as using data collected for other purposes (secondary data). Both quantitative and qualitative data may be needed to fully capture the array of factors reflected in the expanded framework of equity. The data sources used in health services research conducted at the community, system, institution, and

Table 6.3 Criteria and Indicators of Equity

Dimensions	*Criteria*	*Indicators*
	Procedural Equity	
Deliberative Justice		
Health policy	Participation	Type and extent of affected groups' participation in formulating and implementing policies and programs
Distributive Justice		
Delivery system • Availability • Organization • Financing	Freedom of choice	 • Distribution of providers • Types of facilities • Sources of payment
Realized access • Utilization • Satisfaction	Cost-effectiveness	 • Type and volume of services used • Public opinion, patient opinion
Distributive and Social Justice		
Population at risk • Predisposing • Enabling • Need	Similar treatment	 • Age, gender, race, education, etc. • Regular source, insurance, income • Perceived, evaluated
Social Justice		
Environment • Physical • Social • Economic	Common good	 • Toxic, environmental hazards • Social capital (family structure, voluntary organizations, social networks) • Human and material capital (schools, jobs, income, housing)
Health risks • Environmental • Behavioral	Need	 • Toxic, environmental exposures • Lifestyle, health-promotion practices
	Substantive Equity	
Health • Patients • Community	Need	 • Clinical indicators • Population rates

patient levels are summarized in Table 1.2. The sources of primary and secondary data that are particularly relevant for examining the various dimensions of equity are reviewed in the discussion that follows.

Community

Environment. Environmental indicators focus on the community itself or definable geographic areas within it as the unit of analysis. They are explicitly intended to reflect the structural or environmental context in which residents live and work that significantly affects health risks or health. The WHO Healthy Cities and Healthy Communities movement has, for example, identified a range of community-level variables related to air and water quality, housing availability and quality, and economic development, which can be used in profiling the health and well-being of communities (WHO 1999). These data are available from planning agencies, business censuses, U.S. census data on household characteristics, and local public health environmental surveillance systems. Qualitative studies using participant or nonparticipant observation methods may also be useful for profiling the social and environmental context that may affect the health or healthcare of individuals within a designated neighborhood or ethnic group (Devers, Sofaer, and Rundall 1999).

Population. Population-based studies include individuals who do not use a given delivery system or institution as well as those who do. The denominator for population-level analyses represents individuals residing in a designated geographic area. Surveys are particularly useful in measuring the attitudes or barriers that preclude targeted individuals or subgroups from seeking care. A number of large-scale, national surveys have examined access and trends over time for the U.S. population as a whole. These include the Robert Wood Johnson Foundation surveys, the Agency for Healthcare Research and Quality Medical Expenditure Panel surveys, and the continuing National Center for Health Statistics (NCHS) National Health Interview Survey. Such surveys are, however, complex and expensive to conduct (Aday 1996). A special supplement to *Health Services Research* reviews the issues of measuring access to care through population-based surveys in a managed care environment (Bindman and Gold 1998). State or local agencies may

lack the resources and expertise for conducting such studies. Qualitative or semistructured interviews and focus groups may also be instructive in profiling the health and healthcare experiences of a population at risk, as well as informing the design or interpretation of more structured surveys of a representative sample of the target population (Devers, Sofaer, and Rundall 1999).

Public health population surveillance systems, disease registries, census or vital statistics data, and synthetic estimates based on national sources are some of the major types of secondary data used in profiling the health and healthcare of a population at the state or local level. For example, the Surveillance, Epidemiology, and End Results Program of the National Cancer Institute (2003) compiles cancer registry information on cancer incidence and survival in the United States. Small-area estimation procedures make use of data gathered at the national level on utilization rates for certain age, gender, or racial groups to impute what the estimates are likely to be at the state or local level given the age, gender, and racial composition of the state or community. Geographic and related information system methods are also being increasingly used to identify and analyze the impact of environmental and contextual factors on health and healthcare disparities and access (Ricketts 2002; Ricketts et al. 1994). Managed care plan enrollment files also provide data on the denominator of individuals residing in a given geographic area who are eligible to use plan services.

System

Descriptors at the system level focus on the availability, organization, and financing of services as aggregate, structural properties. Secondary data sources are most often used for this type of analysis. The National Center for Health Workforce Analysis, Bureau of Health Professions within the Health Resources and Services Administration has, for example, compiled the computerized Area Resource File (ARF), which has an array of health and healthcare data by county or metropolitan statistical area (QRS 2003). The American Medical Association and the American Hospital Association, as well as other provider groups, routinely publish directories and, in some instances, have computerized data available on the characteristics and distribution of medical personnel. NCHS collects data on the characteristics and utilization of hospitals, nursing homes, and outpatient medical care practices. The

Centers for Medicare & Medicaid Services and the Health Insurance Association of America also periodically publish information on the amount and distribution of expenditures by the major public (i.e., Medicare and Medicaid) and private third-party payers. These data sources are particularly useful for describing the delivery system at the national level and, to some extent, at the state level (NCHS 2002, 2003).

The Community Tracking Study, conducted by the Center for Studying Health System Change (2003) with support from the Robert Wood Johnson Foundation, represents a major data collection and analysis effort. It draws on site visits, consumer surveys, and secondary data sources to monitor and understand the dimensions and impact of healthcare system change in more than 60 randomly selected communities and a random subset of 12 intensive study sites throughout the United States.

Public health departments or private providers such as national HMO firms considering entering a market may want either more current or more detailed information on the types of services being provided or the profile of clients seen by facilities in a given area than is available from existing sources. In this case, the interested agencies or organizations could collect primary data based on interviews with key community informants, telephone requests to providers for brochures describing their services, or full-fledged surveys of providers to gather data on the programs and services being offered and the clients being served. The National Committee for Quality Assurance (NCQA) Health Plan Employer Data and Information Set (HEDIS) data system provides a profile of the organizational and financing features of participating plans (NCQA 2003).

Chapters 4 and 5 review an array of indicators from the Organization for Economic Cooperation and Development and other sources for describing and comparing the healthcare systems in different countries.

Institutions

Secondary data used most often by institutions or organizations for assessing access to a particular facility include enrollment, encounter data, claims, and medical records. Financial records provide an indication of the level of uncompensated or undercompensated care that the facility provides and for what types of patients and services. Other institutional sources, such as clinic logbooks or emergency room referral

records, are used in conducting studies of the magnitude and profile of unscheduled walk-in visits and of nonmedically motivated transfers within an institution. Surveys might also be conducted of administrators, providers, or patients to gather data on the operation of the institutions relevant to access or availability issues.

Patients

Patient surveys are the major sources of primary data for evaluating access at the institutional level. Patient surveys tap individuals' subjective perceptions of their experiences at a given facility (e.g., how long they had to wait to be seen), which may or may not agree with more objective institutional records or data sources (e.g., average clinic waiting time estimates). These subjective perceptions are, however, more reflective of the extent to which people actually are satisfied and loyal users of a facility than are objective, records-based indicators. A variety of standardized instruments have been developed and used for this purpose, such as the Consumer Assessment of Health Plans Survey, NCQA's HEDIS, and Press-Ganey satisfaction questionnaires (Aday 1996; AHRQ 2003; Bindman and Gold 1998; Gold and Wooldridge 1995). Focus groups and ethnographic interviews conducted with patients may also assist in explaining problems that providers have encountered in dealing with certain types of patients or in designing more culturally sensitive or consumer-oriented services.

Patient-origin studies use patient address and zip code information to determine the areas from which most patients are drawn. Patient record data could also serve as the basis for generating profiles of the demographic composition (i.e., age, sex, and race) or the major presenting complaints of patients seen at the facility.

A variety of research designs may be employed in assessing equity at the community, institution, and system levels that also focus on different components of the conceptual framework of equity and their interrelationships (Figure 6.1). Some of these are discussed in the following sections.

Study Designs

Three major types of health services research designs may be drawn on to define and clarify the objective of equity and how well programs and policies have succeeded in achieving it. These include descriptive, analytic, and evaluative research designs (Aday 1996).

Descriptive

Descriptive research focuses primarily on profiling the discrete indicators of equity. In effect, they reflect data that are collected to operationalize the dimensions represented in the respective boxes in Figure 6.1. (Also see Appendix 7.1.) They can also be used to make normative assessments of procedural or substantive equity, based on the criteria of equity they are deemed to most directly express (Table 6.3). Descriptive analyses may, however, be viewed as essentially identifying the symptoms of a problem. More probing analytic research is required to diagnose the underlying etiology, or origins, of a problem and the likely health and healthcare consequences of the problem.

Analytic

Analytic research is directed toward understanding hypothesized cause-and-effect relationships between the structure, process, and intermediate outcome components of the model, and ultimately toward the primary outcome of interest—improving the health of individuals and communities. The hypotheses to be explored in such studies are implicit in the arrows between components of the expanded equity framework (Figure 6.1). These studies are useful, for example, in illuminating the impact of policy-relevant variables, such as the type and extent of insurance coverage, on the use of services and associated clinical outcomes.

A particular challenge for public health and health services researchers with respect to future analytic research on the equity objective as defined here is identifying the factors that are most predictive of improved health. Evaluating access to medical care for problems that medical care cannot address does little to prevent or remedy these problems. Analytic research on the correlates and consequences of health and human functioning can help to address questions regarding whether investments in medical care or in other systems or services are the most relevant bases for allocating scarce societal resources.

Evaluative

Evaluative research assesses how well programs and services that have been developed and implemented based on previous descriptive and analytic research have done in achieving a desired equity objective. Evaluative studies rely primarily on experimental or quasi-experimental designs to determine program or policy outcomes. Evaluations of Medicaid and Medicare managed care demonstrations have, for example,

provided useful information for assessing the access, quality, and cost impacts of these models for low-income enrollees, as well as informing the design of state Medicaid managed care policies and the Medicare+Choice managed care program (Hurley and McCue 2000; Hurley, Freund, and Paul 1993; Langwell and Hadley 1990).

SUMMARY AND CONCLUSIONS

The answer to the first question posed at the beginning of this chapter—What is equity?—may be summarized as follows: equity is concerned with health disparities and the fairness and effectiveness of the procedures for addressing them. The response to the second question—How should equity in healthcare be assessed?—is this: examine and account for health disparities.

This chapter has reviewed major paradigms of justice and their implications for an operational framework for the conduct of health services research on equity. The ultimate dependent variable in that framework and the corollary bottom line for assessing the impact of health policy is the health of individuals and populations (Figure 6.1). The distributive justice paradigm evaluates the characteristics of the system and population that contribute to differentials in the distribution of medical care. The social justice paradigm examines the factors in the social, economic, and physical environment that contribute to disparities in the prevalence of poor health. The deliberative justice paradigm provides the blueprint for the design of more effective health policies at the macro level and micro level by ensuring that parties affected by such policies participate in shaping them.

The conceptual framework of equity presented here, based on these perspectives, points to more focused, expanded, and explanatory health services research to assess equity. The framework fully addresses the linkage and integration of concepts and methods from research on effectiveness and efficiency, in addition to equity, in assessing system performance. Health services research, based on this framework, would be more focused on improving the health of patients and communities as the ultimate goal of health policy. The resulting research agenda related to this substantive objective must, of necessity, be grounded in the concepts and methods that underlie the population perspective on enhancing the health of populations and the clinical perspective on improving the outcomes of patients. Health services research would be expanded to encompass broader epidemiological, ecological, and related public

health theories, methods, and research questions in understanding the medical and nonmedical factors that contribute to health. It would draw on the studies of allocative efficiency related to what types and what mix of inputs are most likely to be productive of health and the corollary concerns of production efficiency regarding the most efficient means for producing these inputs. Finally, health services research would be more explanatory in that a greater emphasis would be placed on analytic and evaluative research to generate and explore relevant hypotheses regarding the array of factors, and the relationships between them, that are most likely to contribute to the health of individuals and populations. The conceptual framework of equity presented here (Figure 6.1) is intended to provide guidance for developing this more explanatory and health-centered health services research agenda.

Chapter 7 reviews the available evidence to address the third question posed at the beginning of this chapter: To what extent has procedural and substantive equity in health and healthcare actually been achieved in the United States? The distributive justice paradigm has primarily served to guide health services research on equity. The arguments and evidence presented here are intended to document that norms of deliberative and social justice must also be taken into account if the ultimate health policy goals of improving health and narrowing health disparities are to be achieved.

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Chapter 7

Equity: Policy Strategies, Evidence, and Criteria

CHAPTER HIGHLIGHTS

1. The principal policy strategies, grounded in the distributive, social, and deliberative justice paradigms, respectively, are to (1) enhance access to medical care, (2) reduce health disparities, and (3) ensure affected parties' participation in policy and program design.
2. The weight of the evidence regarding the extent to which equity has been achieved is mixed: (1) wide variations in healthcare coverage and access exist; (2) health disparities persist and may in some instances be widening; and (3) the norm of participation is neither routinely nor fully considered in the health policy formulation and implementation process.
3. The application of the equity criteria (introduced in Chapter 6) in assessing the incidence, screening, and treatment for breast cancer in general documents that major procedural and substantive equity issues exist.

OVERVIEW

This chapter highlights alternative policy strategies for enhancing the equity of healthcare grounded in the conceptual framework of equity introduced in the previous chapter (Figure 6.1) and assembles empirical evidence to address the question regarding the extent to which the goal of equity has been achieved in the United States. Findings from health services and public health research are presented regarding the correlates and indicators of equity based on the distributive, deliberative, and social justice paradigms. The criteria for assessing health policy in terms of the equity objective are defined and illustrated in the context of breast cancer prevention and treatment.

POLICY STRATEGIES RELATING TO EQUITY

Three primary policy goals may be identified as the focus of strategies for enhancing the equity of healthcare provision that are lodged in the distributive, social, and deliberative justice paradigms, respectively: (1) enhance access to medical care, (2) reduce health disparities, and (3) ensure affected parties' participation in policy and program design.

The distributive justice paradigm and its attendant concern with ensuring equity of access to medical care have tended to dominate equity assessments of health policy in the United States since the mid-1960s. This perspective has focused most notably on the availability, organization, and financing of medical care services by providing support for major federal investments in the training of medical care providers, the construction of healthcare facilities, and the coverage of the poor and elderly through Medicaid and Medicare, respectively. It culminated in the early 1990s in a major national universal health insurance reform effort and, in the latter half of that decade, led to a plethora of state healthcare financing reform measures. As indicated in previous chapters, assessments of policy initiatives directed at improving access to medical care often have not gone far enough in considering the extent to which such initiatives ultimately have served to enhance the health of individuals and populations.

The population perspective on the health of communities as a whole and on the medical and nonmedical factors that give rise to health disparities challenges health services researchers and policy analysts to consider broader conceptions of equity rooted in the social justice paradigm. Population health concerns have traditionally been the domain of public health policy and service provision. Although the U.S. and international public health communities have undertaken a number of major programmatic initiatives—such as the World Health Organization (WHO) Year 2010 objectives and Healthy Community programs—to attempt to ameliorate health disparities, such disparities continue to persist in the United States and other countries and, in some cases, have widened across racial and socioeconomic groups. Clinical effectiveness research has documented those investments in medical care that can make a difference, as well as how the effectiveness of medical care can be improved. Population effectiveness research has confirmed that public health, social policy, and economic policy investments are also needed to enhance the health of communities and of the individuals residing within them. Health policy ultimately directed toward

improving the health of individuals and of populations, then, demands investments in more comprehensive, integrated, and effective medical and nonmedical interventions.

The deliberative justice paradigm compels the parties affected by health policy and program design to participate in shaping them. This translates at the macro level to mechanisms such as the assurance that stakeholders' interests as well as those of the general public are reflected in the policy formulation process, and it translates at the micro level to a fuller involvement of patients and their families in clinical decisions affecting health outcomes. Justice is judged by the extent to which those directly affected directly participate. The implication of the equity framework introduced in the previous chapter (Figure 6.1) is that the ultimate goal of health policy—improving the health of individuals and communities—is not likely to be achieved if the norm of deliberative justice fails to influence the policy formulation process.

The discussion that follows presents evidence regarding the equity of healthcare provision based on the respective paradigms of justice and accompanying policy strategies.

EVIDENCE RELATING TO EQUITY

The review of evidence in this chapter begins with the endpoint for assessing the equity of the healthcare system based on the expanded equity framework (Figure 6.1)—the extent to which subgroup disparities in health persist. This focus is defined most directly by the social justice paradigm. Evidence regarding the magnitude and correlates of substantive equity reflected in these disparities will be presented first, to set the stage for assessing the extent to which this ultimate and defining equity objective has been achieved. Then, evidence regarding the distributive equity of health services provision will be reviewed, with a particular focus on whether it indicates the amelioration or the exacerbation of health disparities. Finally, evidence related to the deliberative justice paradigm will be inventoried, delineating in particular the broader health services research and policy agendas to which it points. The distributive and deliberative justice dimensions point primarily to evidence regarding procedural equity. The import of these factors for substantive equity is reflected in their likely contribution to minimizing health disparities.

The bulk of the health services research evidence regarding the equity of healthcare provision is rooted in the distributive justice paradigm.

Evidence regarding the social justice dimensions of equity is drawn primarily from public health and related social science research documenting the predictors and indicators of population health and health disparities. Although the deliberative justice paradigm has not been as explicitly employed in the evaluation of healthcare services or public health policy, it finds expression in assessments of the role of healthcare consumers in clinical decision making, the cultural sensitivity of care, and community participation in the design of healthcare programs and services.

Social Justice

The social justice dimension of equity encompasses a look at the physical, social, and economic environment; health risks; and associated health disparities between groups. The U.S. Public Health Service and WHO Year 2010 health objectives provide a framework and a set of indicators for monitoring progress toward this goal. The findings with respect to selected indicators and environmental and behavioral predictors of health disparities in the United States are reviewed in Chapter 1.

Basically, the evidence documents that disparities between groups—particularly between whites and racial or ethnic minorities—persist (Aday 2001; NCHS 2003a, 2003b). Very young, minority, poorly educated mothers are much less likely to have adequate prenatal care and are more likely to bear low-birthweight infants. The rates of teenage pregnancy, preterm and low-birthweight babies, inadequate prenatal care, and infant and maternal mortality remain two to three times higher among African American women than among white women, and these rates show no sign of diminishing. The prevalence of chronic disease increases steadily with age, as do the incidence of death and the magnitude of limitation in daily activity due to chronic disease. At any age, men are more likely to die from major chronic illnesses such as heart disease, stroke, and cancer than are women, although elderly women living with chronic illness have more problems with carrying out their normal daily routines. African Americans—particularly African American men—are more likely to experience serious disabilities and to die from chronic illness than are whites. Early in the course of the AIDS epidemic, homosexual or bisexual males were most likely to be affected. In recent years, more and more mothers and children are at risk because of intravenous drug use among women or their sex partners. Higher proportions of African Americans and Hispanics than of

whites are likely to be HIV-positive, to develop and die of AIDS, and to have contracted the disease through drug use or sexual contact with drug users. Young adults in their late teens and early twenties—particularly men—are more likely to smoke, drink, and use illicit drugs than are their younger or older counterparts. Native American youth are much more apt to use alcohol, drugs, and cigarettes than are white or other minority youth. Minority substance users are also more likely to develop life-threatening patterns of substance abuse, as evidenced by the higher rates of addiction-related deaths among minority groups. Death rates for cirrhosis and other alcohol-related causes are greater among Native Americans compared to whites or other minorities. A disproportionate number of medical emergencies and deaths due to cocaine abuse occur among minorities—particularly among African Americans.

The physical, social, and economic environments to which individuals are exposed can have a profound effect on their health and healthcare. The movement of businesses and industries out of central cities has contributed to high unemployment rates, particularly among young inner-city minority males. Men's wages remain higher on average than women's wages, and the national minimum wage has not kept pace with inflation, further exacerbating the economic burden on female heads of households with dependents. In addition, men are generally made better off financially by divorce or separation, whereas women's economic situations usually worsen (Aday 2001; U.S. Department of Commerce 2002).

The percentage of families with children headed by only the mother has increased among all racial and ethnic groups. The average salaries of working African American women are lower than those of either white women or African American men, and the average welfare benefits have continued to decline in real purchasing power. Although more African American men have entered the workforce in recent years, the rates of unemployment among young African American men remain high. Lack of employment opportunities; poverty; and the associated problems of crime, substance abuse, and violence plague many inner-city neighborhoods. The socioeconomic status of Hispanic and Native American families resembles that of African Americans more than that of whites. Nonetheless, the reduced number of jobs in the manufacturing and industrial sectors, the growth in the number of minimum wage and part-time jobs in the service sectors, and the increased tax

burden on low-income and middle-class taxpayers have also resulted in many working poor families—including white families and those with two breadwinners—experiencing increased economic difficulties. These aspects of the social and economic environment can have profound consequences for the health and health risks of the most vulnerable (Aday 2001; U.S. Department of Commerce 2002).

A challenge in the design, conduct, and interpretation of research examining these issues is determining the essential meaning of factors such as race and poverty and the dynamics through which these factors operate to influence health. A particular criticism of the application of these variables is that they are typically used as attitudinal, biological, or behavioral descriptors of individuals—especially in studies based on large-scale surveys or databases—while insufficient attention is paid to the social-structural, cultural, and environmental contexts that fundamentally shape these individuals' access to health and healthcare. As a consequence, the focus of health policy informed by such research remains aimed at intervention at the individual level, not at the broader social and economic conditions, as well as the systems of opportunity that influence the health risks of socially and economically disadvantaged populations (Krieger 2002; LaVeist 1994; Lillie-Blanton and LaVeist 1996; Link and Phelan 1995; Williams 1994).

The weight of the evidence in the United States and other countries documents that health disparities based on social and economic disparities between and among groups persist and have, in fact, widened in some cases. Medical care and public health have been ineffective in significantly narrowing these disparities. These findings indicate the necessity of considering broader roles for medical care and for public health, social, and economic policy to influence health and to reduce persistent racial and income-related health disparities. Aday (2001) points out the importance of the considerations in addressing the fundamental origins of poor physical, psychological, or social health among particularly vulnerable populations (e.g., persons with HIV/AIDS, the mentally ill, alcohol and substance abusers, families living with physical or emotional abuse, the homeless, immigrant and refugee populations). The challenge to both the medical care and the public health communities is to create and extend the partnerships between them and with other sectors within their communities to more fully and effectively address persistent or widening subgroup disparities in health.

Distributive Justice

The distributive justice paradigm has dominated the conceptualization and measurement of the equity of the U.S. healthcare system. Illustrative examples and estimates of indicators of equity based on the distributive justice framework are highlighted in Appendix 7.1. The presentation and discussion of findings in the context of this paradigm focus principally on trends in potential access indicators, their relationship to predicting people's actual utilization, and their levels of satisfaction with care, as well as likely health consequences.

Delivery System

Availability

Potential access. The number and distribution of providers and, more importantly, the effect of service availability on the decision to seek care have been and continue to be a focus of health policy efforts regarding access. Post–World War II policies supporting medical personnel training and new hospital construction led to overall increases in the number of providers and facilities. These increases were mirrored in steady rises in the traditional provider-to-population and facility-to-population ratios. Wide variability nonetheless persists in the geographic distribution of providers. Many areas are facing critical shortages of nurses. Managed care has also affected the overall availability of and need for different types of physicians by increasing the demand for primary care physicians and diminishing the use of specialists in many areas. A related issue of availability is the willingness of providers to see patients who are publicly insured or uninsured. Physicians' refusal to see Medicaid clients and strategies by managed care plans to locate in areas that are not readily accessible to these populations create significant barriers to care for low-income, pregnant women—particularly those residing in rural or inner-city areas with large minority populations. A large proportion of physicians in certain specialties, such as obstetrics-gynecology, have closed their practices to medically indigent and Medicaid patients because of the low rates of public, third-party reimbursement and the heightened fears of medical malpractice liability (Mullan 2002; NCHS 2003b; Perloff et al. 1997; Salsberg and Forte 2002; Silverstein and Kirkman-Liff 1995; Simon, Dranove, and White 1998; Sochalski 2002).

Realized access. Provider-to-population ratios alone do not determine actual rates of use. Even in areas of ostensible shortage, residents with transportation and financial resources travel out of the neighborhood or to adjoining towns for care. Insurance coverage and the availability of a racially and ethnically diverse provider workforce may be equally as important or more important than overall physician supply in influencing access to care, especially in high-risk, underserved communities. There is heightened concern over the effect of a number of national and local trends on the availability of providers and the resultant utilization patterns of residents in rural and inner-city communities. These include the buyout and conversion of not-for-profit community hospitals by for-profit healthcare corporations; closures of rural hospitals and of financially stressed safety-net providers serving poor, inner-city populations; and primary care provider reluctance to locate or to remain in these same areas (Center for Studying Health System Change 2002; Claxton et al. 1997; Gray 1997; Grumbach, Vranizan, and Bindman 1997; Ricketts 2002).

Health impacts. The effect of these changes on actual patterns of service use and health depends to a large extent on whether alternative and appropriate service delivery arrangements subsequently become available to the populations previously served by these providers (e.g., through reconfiguring a formerly inpatient-oriented rural hospital to a primary care or emergency care service provider). The shortage of nurses in many communities has raised concerns about the resulting quality of hospital nursing care being provided. The lack of an adequate system of primary care in general, and maternity and prenatal care services in particular, for low-income inner-city women and for poor minorities living in isolated rural counties or communities has been found to contribute to their lower rates of use of effective preventive and illness-related care. Health services research has documented higher rates of avoidable hospitalizations and ambulatory care–sensitive conditions (i.e., disease occurrence that could have been prevented with adequate primary care) among racial/ethnic minorities and in medically underserved areas with lower socioeconomic status (Billings, Anderson, and Newman 1996; Bindman et al. 1995; Buerhaus et al. 2002; Davis, Liu, and Gibbons 2003; Epstein 2001; Gaskin and Hoffman 2000; IOM 1993; Lee et al. 1999; NCHS 2001; Ward and Berkowitz 2002).

Organization

Potential access. The organization and financing of healthcare in the United States increasingly reflect a multiple-tiered system of benefits including, in descending order of generosity, the privately insured middle and upper class, the elderly who have Medicare only, the Medicaid-eligible indigent or working-class poor population, and individuals and families with neither public nor private coverage. Such disparities have, in some sense, always been a fact of life in the U.S. healthcare system. They emerge as a particular paradox now, however, because as the overall public and private commitment of expenditures for medical care continues to rise, so does the number of Americans who have no or inadequate protection against these burgeoning increases. The U.S. healthcare system has been characterized as a "medical-industrial complex," referring to the large network of private corporations engaged in the business of supplying medical care to patients for a profit, such as chain hospitals, walk-in clinics, dialysis centers, and home healthcare companies. The diverse and evolving forms of private medical practice are also increasingly linked to methods of paying for care. These include group practice–based HMOs, individual practice associations, preferred provider organizations, and point-of-service plans.

The organizational distinctions between these different arrangements are becoming increasingly obscure. All of these alternatives have attempted to develop systems of cost-conscious medical practice and methods of reimbursement. Managed care organizations (MCOs) typically limit consumer choice of providers to participating physicians and emphasize primary care gatekeeper arrangements and less use of specialists. The competition among MCOs has resulted in the reduction of cross-subsidies to the safety-net providers that serve large numbers of the uninsured or medically indigent and either threatened or led to the closure of many safety-net institutions, such as community health centers and Medicaid-dependent hospitals. The growing number of elderly—particularly the oldest old—and the impetus for shortened lengths of hospital stays resulting from diagnosis-related groups (DRGs) have put increasing pressures on families and other home- and community-based long-term care arrangements. The deinstitutionalization movement in mental healthcare also led to the discharge of large numbers of the mentally ill and a greatly increased burden on community-

based mental health services provision (Aday 2001; Bazzoli et al.1999; Burns et al. 2000; Dranove and White 1998; Felt-Lisk, McHugh, and Howell 2002; Gaskin, Hadley, and Freeman 2001; McAlearney 2002; Penrod et al. 1998; Relman 1980).

Realized access. The major concerns underlying the realized-access impact of the corporatization of medical practice relate to the fact that private and for-profit institutions are less likely to serve the poor and medically indigent and that large-scale, bureaucratic, publicly supported providers are less likely to be convenient and satisfactory to consumers. They are also more subject to closure or to be purchased by for-profit entities in an increasingly competitive healthcare environment. MCOs primarily enroll employed individuals, especially employees of large firms. Employers and insurers have typically restricted enrollment and coverage for employees' dependents and for particularly vulnerable or high-risk populations (e.g., persons with AIDS). Both not-for-profit and for-profit private institutions are less likely to serve patients without insurance and have much lower rates of uncompensated and undercompensated care than publicly supported institutions—teaching hospitals in particular. The poor and elderly generally have been underrepresented as well in HMO and private insurance plans. States have responded by enacting laws to ensure access and to improve the accountability of MCOs for the quality and appropriateness of care for managed care enrollees in general and for the most vulnerable enrollees in particular (Gosfield 1997; Marsteller, Bovbjerg, and Nichols 1998; Proenca, Rosko, and Zinn 2000).

Health impacts. Users of publicly supported facilities such as public health clinics, hospital outpatient departments, or emergency rooms often may have to wait hours to be seen when they are ill or injured or may be told that it will be weeks or even months before they can get an appointment for a routine or prevention-related visit (e.g., for prenatal care), which could have serious health consequences. MCOs' restrictions on gatekeepers and specialty referral patterns may also have health consequences for chronically ill patients, women, or others who require access to a broader array of providers (e.g., obstetrician-gynecologist, cardiologist) to fully monitor and maintain their health. Research based on the Medical Outcomes Study documents that although physical and mental health outcomes did not differ for the average patient seen in

HMOs compared to fee-for-service arrangements, elderly, poor, and chronically ill HMO patients experienced poorer physical health outcomes (Berk, Schur, and Cantor 1995; IOM 1993; Miller and Luft 2002; Moore, Fenlon, and Hepworth 1996; Ware et al. 1996; Weisman and Henderson 2001).

Financing

Potential access. The advent of Medicaid and Medicare in the mid-1960s led to a significant increase in the proportion of personal healthcare expenditures paid for by the federal government. Private health insurance also assumed a larger role in financing healthcare through employer-based coverage, while the proportion of out-of-pocket expenditures borne by households or individuals declined (see Appendix 7.1). As the costs of care have continued to rise, public and private third-party payers became increasingly interested in reducing the amounts they had to pay for medical care. They have, therefore, imposed stricter eligibility criteria; cutbacks in covered services; fixed, predetermined (i.e., prospective) rates of reimbursement by service or diagnosis, such as DRGs; and greater consumer cost sharing. State Medicaid expansions and the State Children's Health Insurance Program have attempted to expand public coverage to a large number of uninsured families and children in many states. Nonetheless, as will be discussed later in this chapter, the number of uninsured Americans remains high. Further, an ambitious effort to enroll Medicare-eligible individuals in managed care plans through the Medicare+Choice option turned out to be a largely failed experiment as managed care plans began to pull out of this market largely because of profitability concerns (Casey, Knott, and Moscovice 2002; Gold 2001; Levit et al. 2003; NCHS 2003b; Racine et al. 2001; Rosenbaum et al. 1998).

Realized access. Empirical findings related to the major prospective pricing initiative (i.e., reimbursement for hospital services under Medicare on the basis of DRGs) on hospital utilization and expenditures show that admission rates, total days of care, and average length of stay have declined since its introduction. These trends, however, are confounded with trends that were already underway in the organization and delivery of medical care prior to the introduction of DRGs, such as an increased emphasis on ambulatory care. (See the discussion

of these trends in Chapters 1 and 5.) The tendency to discharge Medicare patients when they reach the limit of reimbursable days under DRGs has exposed deficiencies in the system of posthospitalization care for the chronically ill and elderly in many communities, such as inadequate discharge planning, an insufficient number of nursing home beds, lack of community support services, and corollary stresses on the patient's family and on others.

Health impacts. The RAND Health Insurance Experiment documented an inverse relationship between the amount of physician and hospital services consumed and the amount of consumer copayment—the more consumers had to pay, the less medical care they consumed. The office-based medical use rates for children in particular were likely to be lower for those in cost-sharing plans compared to those in free-care plans. Although the Health Insurance Experiment documented minimal overall negative health consequences as a result of plan cost-sharing provisions, the negative effects found were primarily among low-income, chronically ill individuals. Medical care expenses tend to absorb a much higher proportion of the total income of low-income families than that of families with higher incomes. Policies that encourage greater cost sharing by consumers will undoubtedly lower the overall use of services. The resultant economic and health effects are most likely to fall on the poorest and sickest (Anderson, Brook, and Williams 1991; Angelelli et al. 2002; Gross et al. 1999).

Population at Risk

The focus in reviewing evidence on distributive justice at the population level is the effect of an array of predisposing, enabling, and need factors on the population's use of and satisfaction with medical care. Equity in this context is grounded in the similar-treatment norm—variations in medical care utilization should be primarily a function of need, rather than of socioeconomic or related healthcare factors. These differences are particularly important to probe in better understanding the origins and consequences of health and healthcare disparities.

Predisposing. Age is significantly associated with all different types of medical care service use, primarily because it is an important indicator of age-associated morbidity. In general, women use more health services than do men; this is to some extent a function of their obstetrics-

related care needs, their greater longevity, and the perception that it is more socially acceptable for women to engage in help-seeking behaviors. As noted earlier, however, substantial availability, organizational, and financial barriers exist for certain categories of women—especially low-income, uninsured, or Medicaid-eligible women—seeking needed prenatal and maternity care services. Welfare reform has also resulted in many women losing Medicaid benefits. Education is an important predictor of the use of preventive services. Better-educated people are, for example, more likely to have had a general physical, immunizations, tests, and procedures for preventive purposes; and better-educated women are more likely to have sought care early in their pregnancy. Racial/ethnic disparities in health and healthcare have persisted and show little sign of diminishing (Fiscella et al. 2002; Gilbert et al. 2002; IOM 2003; Commonwealth Fund 2001a, 2001b; Henry J. Kaiser Family Foundation 2002; Mayberry, Mili, and Ofili 2000; Mueller et al. 1999; NCHS 2003b; Short and Freedman 1998; Weinick, Zuvekas, and Cohen 2000).

The influence of predisposing factors such as age, gender, race/ethnicity, and education on utilization has remained relatively stable over time. (See Appendix 7.1.)

Enabling. According to the 1999 National Health Interview Survey, 6.3 percent of children (i.e., 0 to 17 years of age) and 15.4 percent of the adult population did not have a usual source of care. Among those who did, a private doctor's office was used most frequently, followed by health centers and similar sites, such as company or school clinics, and hospital outpatient departments or emergency rooms. Blacks, Hispanics, the poor, males, and residents of large metropolitan statistical areas were least likely to have a regular source of care or, if they did, were more likely to use clinics or hospital outpatient departments or emergency rooms (NCHS 2003c).

Around 15 percent of Americans (or an estimated 43.6 million) lacked public or private insurance in 2002. The number of those uninsured for some period of time in the last two years is even higher. Those most likely to be uninsured at some point during the year are young adults and children under 18, Hispanics and blacks, and the poor. The percentage of those uninsured is higher for blacks and Hispanics than for other racial groups (U.S. Census Bureau 2003).

Most of the uninsured are workers or the dependents of workers who do not receive health insurance through their jobs. A large majority are in families headed by a full-time worker who has been employed

for at least a year. The uninsured are more likely to work in small firms or industries such as service or agricultural jobs that do not provide coverage. Even when coverage is offered, the high costs of the plans available in many firms have inhibited low-wage workers in particular from purchasing coverage. Among those who are insured at a given point in time, some may be uninsured or inadequately protected against the possibility of large medical bills. Compared to those who are continuously insured, those who experienced a period of being uninsured are at higher risk of going without needed medical care (Gabel et al. 2002; Kaiser Commission on Medicaid and the Uninsured 2002b, 2003; Marquis and Long 2001; Schoen and DesRoches 2000; The Access Project 2003).

Having insurance coverage and a usual medical care provider are important predictors of whether care is sought—either for preventive or illness-related reasons.

Need. Assessments of need may be based on patients' self-perceptions of their health, as well as on medical professionals' clinical diagnoses and evaluations. Providers' and patients' evaluations of needs may not always agree. Nonetheless, need—however measured—is consistently borne out to be an important predictor of the use of services and, in particular, of the volume of services consumed. Need is, for example, generally the most important predictor of the number of physician visits for those with at least one visit and of the number of days of care once a patient is admitted to the hospital. For more prevention-oriented or discretionary services, such as dental care, need has been and continues to be less important than other factors, particularly enabling factors such as income or insurance coverage. The utilization of services may be deemed equitable to the extent that services are distributed on the basis of need (Aday, Andersen, and Fleming 1980; Andersen 1995; Andersen and Davidson 2001; Andersen et al. 1987).

Realized Access

Utilization. Race, income, having a regular source of care, and insurance coverage are important policy-relevant predictors of the utilization of medical care services.

Despite improvements in the levels of access to medical care among Hispanics and other minorities, these groups are still less likely to use

certain types of services than are whites. Mexican Americans in particular are less likely to have seen a physician or dentist or to have been hospitalized than are whites, blacks, or other categories of Hispanics. Hispanic and Native American women are less likely to have sought care during the first trimester, or in some cases at all, during their pregnancy. The lack of insurance coverage appears to be a particularly important contributor to Hispanics' lower use of medical and dental services (NCHS 2003b).

The proportion of women seeking care in the first trimester of their pregnancy, of preschool children who are immunized, and of adults or children who have been to a dentist is much lower among blacks than among whites. The incidence of congenital syphilis and late-stage cancer, both of which are preventable through early intervention, is also higher among blacks (NCHS 2003b).

In the past, people with higher incomes used more medical care services than those with lower incomes. With the enactment of Medicare and Medicaid, the rates of utilization increased greatly among the poor. Nonetheless, income-related use differentials remain. The rates of use of physician and hospital services in general relative to need are lower for the poor—particularly the poor or working poor who have no insurance (NCHS 2003b).

In 2001, the percentage of people who saw a doctor during the year was lower for people from families with incomes below the poverty level (78.3 percent) than for those with incomes above the poverty level (86.0 percent). People with lower incomes were nonetheless in much poorer health than people with higher incomes based on subjective perceptions of health, reported days of limited activity due to illness, and limitations in major activity due to the presence of a chronic condition. Since the introduction of Medicaid and Medicare, the rates of hospital discharges; days of care; length of stay; and the mean number of visits to a physician, once seen, have tended to be higher for those with lower rather than higher incomes—reflecting perhaps their greater need, as well as their greater tendency to delay seeking care until the health problem has worsened (NCHS 2003b).

Having a regular source of medical care is a strong and consistent predictor of medical care utilization, particularly of the initial decision to seek care. Having an identifiable provider may be particularly important in motivating the use of routine preventive care. Once entry to the system is gained, having an established provider is a less significant

predictor of the subsequent number of visits to a physician or length of hospital stay. The accuracy of self-reports of a usual source of care as well as whether a regular source of care is a determinant or a result of using services are some methodological issues that affect the interpretation of these effects. Causal models testing the direction of this relationship have confirmed that having an identifiable medical provider does directly influence the decision of whether or not to seek care, although unidirectional models may tend to overestimate this effect. With the advent of managed care, the identification of a regular provider is increasingly linked to enrolling in a particular health plan. Uninsured individuals are more likely to lack a usual source of care, experience more access barriers, and have lower rates of preventive services use (Ettner 1996; Kuder and Levitz 1985; Lambrew et al. 1996; Merzel and Moon-Howard 2002; Perloff and Morris 1989; Robert Wood Johnson Foundation 2002; Williams, Flocke, and Stange 2001; Xu 2002).

The presence and extent of insurance coverage have been demonstrated to be important predictors of the utilization of medical care services in numerous national and local studies of access. In addition, there is evidence that patients with private, third-party coverage are more likely to receive more intensive and technology-oriented care and to experience better outcomes than those with public coverage or no insurance. The lack of insurance and related barriers effectively diminish minorities' use of preventive services and medical treatments that could improve health and reduce the associated burden of illness (Kaiser Commission on Medicaid and the Uninsured 2002a; Mueller, Patil, and Boilesen 1998; UCLA Center for Health Policy Research 2000).

Insurance coverage and usual source of care have increasingly been combined in various types of managed care arrangements. Managed care enrollees have lower hospital admission rates and shorter lengths of stay than those covered under fee-for-service arrangements. Research suggests that they have comparable or somewhat higher physician office visit rates, lower use of expensive tests and procedures, and greater use of preventive services. Racial/ethnic disparities in access continue to persist in managed care. Expanded coverage is important for addressing disparities overall, but barriers may still exist (Haas et al. 2002; Hargraves, Cunningham, and Hughes 2001; Miller and Luft 2002; Phillips, Mayer, and Aday 2000; Reschovsky, Kemper, and Tu 2000; UCLA Center for Health Policy Research 2001; Virnig, et al. 2002; Zuvekas and Weineck 1999).

Satisfaction. Surveys of public and patient opinion regarding the performance of the medical care system in different countries confirm that U.S. residents are more critical of the system as a whole and much less satisfied with their own particular experiences in getting care than are people in other countries, including Australia, Canada, New Zealand, and the United Kingdom, for example (see Appendix 7.1). In 2001, 28 percent of U.S. residents thought the system had so much wrong with it that it should be completely rebuilt, compared to around 18 to 20 percent of people in the other countries. U.S. residents also tended to report more problems with paying medical bills or not being able to get selected services. Minorities in the United States especially indicate more barriers and rate the healthcare system less highly than do white Americans (Blendon et al. 2002).

Satisfaction surveys of managed care enrollees and people in fee-for-service arrangements have tended to document that managed care patients have lower satisfaction with appointment waiting times, quality, and patient-physician interaction but have greater satisfaction with costs. In particular, managed care enrollees are often dissatisfied with the choices of plans and providers that are available to them. Variability in levels of satisfaction within plans also exists by age, gender, and race/ethnicity (Hellinger 1998; Miller and Luft 2002; Taira et al. 2001; Weech-Maldonado et al. 2001; Weinick, Zuvekas, and Cohen 2000).

Deliberative Justice

As indicated in the previous chapter, individual and community empowerment and participation have been important components of many international and national health initiatives. There are, however, no standardized or widely applied indicators and scales for measuring this key dimension of deliberative justice. Voter turnout rates and public opinion polls regarding levels of perceived confidence in or ability to influence public officials provide macro-level evidence of the presence and magnitude of civic participation. The failure of the Clinton administration's healthcare reform initiatives in the early 1990s was attributed to the dominance of technical-rational experts in the policy formulation process and the lack of a clear public consensus in support of comprehensive reform (Hacker 1996; Skocpol 1996).

Effectively implementing models of deliberative democracy in which vulnerable populations like African Americans have full and fair representation in bodies deciding what to do in addressing racial/ethnic

disparities has been suggested as an innovative means to effectively address these persistent disparities. A number of different methods have been utilized to measure participation at the micro and macro levels. Attitudinal scales can be used to assess the extent to which organization or community members feel a sense of control or influence over the decisions that most directly affect their health and well-being. Surveys are useful tools for assessing the capacity of health departments to engage in community-based, participatory public health or the magnitude of de facto community mobilization around a public health intervention. Key informant interviews and social network analysis also yield useful data for mapping the extent of community activation and involvement in health program design (Cheadle et al. 2001; Hendryx et al. 2002; Israel et al. 1994; Parker et al. 2003; Stone 2002; Wickizer et al. 1993).

A number of practices on the part of MCOs that limit the involvement of both patients and providers in decision making represent sentinel indicators of likely deliberative justice concerns. These include "gag rules" that inhibit providers from discussing selected treatment options with patients; "cram-down rules" that compel providers to participate in a state-mandated managed care program to receive benefits through other payer arrangements; selective or misleading plan marketing to potential enrollees; time constraints on patient-provider visits or failure to provide cultural-competency training that could affect patient-provider communication; and adversarial or obstructionist consumer grievance and dispute-resolution procedures. While some of these practices or failures may have also been present in fee-for-service Medicaid provider arrangements, Medicaid eligibles' options are likely to be constrained as a function of mandated enrollment in what might be a limited number of competing managed care plans in a given area (Daniels 2001).

Although community or consumer participation has been an explicit component of health policy design, particularly in the context of public health–oriented community empowerment initiatives, it has not always been effectively realized in practice. Further, participation also has not been fully developed and considered as a criterion of the fairness of health policy or program design. The future directions for research in this area would be to extend the conceptual and methodological development of indicators of deliberative justice; to use them in evaluating the performance of policies and programs at the national, community, system, institutional, and patient levels; and to examine

their importance for ultimately influencing access to health and healthcare on the part of the individuals and of the communities they were intended to serve.

The weight of the evidence regarding the extent to which equity has been achieved may be summarized as follows: not to a substantial extent. The evidence of the successes of the broad policy strategies for enhancing equity outlined earlier may be viewed at best as mixed and at worst as falling far short of desired equity objectives. The bulk of the evidence regarding the goal of enhancing access to medical care is rooted in the distributive justice paradigm of individual rights to medical care. Although substantial investments in both the organization and financing of medical care services have been made at federal, state, and local levels, wide variations in access to care and coverage persist across regions and subgroups of the U.S. population, and both the costs and effectiveness of the care provided continue to present challenges to policymakers in deciding what rights should be ensured, and at what cost to whom, within this framework.

The U.S. Public Health Service Year 2010 objectives provide a template for examining the extent to which the social justice goals of minimizing health risks and health disparities have been achieved, based on indicators and evidence of subgroup variations in achieving desired health promotion, health protection, and preventive services goals. The data routinely gathered to monitor progress toward these objectives show progress on some, and persistent or widening disparities on many others.

Although evidence is emerging of the importance of participation by affected parties in health policy and program design, the deliberative justice paradigm has been largely unexamined as a component of the fairness of the policy formulation and implementation process. The challenge to the public health and health services research community is to determine how best to conceptualize and measure norms of deliberative justice, so that both the presence and impact of this innovative benchmark of fairness can be more explicitly assessed.

The evidence available to date suggests that the major health policy strategies directed at achieving both procedural and substantive equity have, as a whole, fallen short of doing so. The next section reviews the specific criteria for evaluating equity in the context of breast cancer prevention and treatment, to set the stage for the policy example in Chapter 9 focusing on evaluation of a specific policy alternative—increasing mammography screening rates for older, Medicare-eligible women.

CRITERIA FOR ASSESSING POLICY ALTERNATIVES IN TERMS OF EQUITY

Table 7.1 summarizes the criteria for assessing health policies in terms of equity and provides illustrative examples of the application of these criteria in terms of breast cancer screening and/or treatment.

The deliberative justice norm of participation focuses on the extent to which affected groups participate in formulating and implementing policies and programs. This may be viewed at the macro level in terms of affected populations' role in shaping national or state policy and at the micro or clinical level in terms of the extent of patients' engagement in clinical decision making related to their care. For example, Medicare and Medicaid breast and cervical cancer screening policies have been influenced but not driven by women's health advocacy interests. Clinical guidelines do not provide explicit guidance for interactive and effective patient-provider decision making related to the role of risk factors and/or age in deciding on whether or how often to screen. Research has, however, suggested that women's participation in the decision to be screened leads to higher mammography adherence rates (Phillips et al. 1998; Platner et al. 2002).

The norm of freedom of choice, grounded in the distributive justice paradigm, argues for maximizing the availability and minimizing the constraints on patients' choice of providers and services. This criterion is documented most directly through evidence on the availability of providers and services, the accessibility of services within specific organizational and service-delivery contexts, and the affordability of services as a function of the cost and extent of third-party financing.

Substantial barriers impede the availability of breast cancer screening and related treatment services. Mammography screening facilities are less available in rural and inner-city areas, which have a disproportionate representation of older and/or minority women. Selected types of MCOs may be more likely to recommend screening than fee-for-service providers, but substantial variability exists across provider settings. Although private and public insurers may cover mammography screening, other factors—disruptions in eligibility, out-of-pocket costs related to deductibles, copayments, or resulting treatment costs—are burdensome to many (especially low-income) women (Lee-Feldstein et al. 2000; O'Malley, Forrest, and Mandelblatt 2002; Perkins et al. 2001).

The cost-effectiveness norm focuses on enhancing access to prevention and treatment benefits and services that are most likely to be cost-

Table 7.1 Criteria for Assessing Health Policies in Terms of Equity

Dimensions	*Criteria*	*Indicators*	*Examples*
		Procedural Equity	
Deliberative Justice			
Health policy	Participation	Ensure that affected groups participate in formulating and implementing policies and programs.	*Population:* Medicare and Medicaid screening policies have been influenced but not driven by women's health advocacy interests. *Clinical:* Clinical guidelines do not provide explicit guidance for interactive patient-provider decision making related to the role of risk factors and/or age in deciding on whether or how often to screen.
Distributive Justice			
Delivery system • Availability • Organization • Financing	Freedom of choice	Maximize the availability and minimize the constraints on patients' choice of providers and services.	*Availability:* Mammography screening facilities are less available in rural and inner-city areas, which have a disproportionate representation of older and/or minority women. *Organization:* Selected types of managed care organizations may be more likely to recommend screening than fee-for-service providers, but substantial variability exists across provider settings. *Financing:* Although private and public insurers may cover mammography screening, disruptions in eligibility, out-of-pocket costs related to deductibles, copays, or resulting treatment costs are burdensome to many (especially low-income) women.

Table 7.1 Criteria for Assessing Health Policies in Terms of Equity *(continued)*

Dimensions	*Criteria*	*Indicators*	*Examples*
Realized access • Utilization • Satisfaction	Cost-effectiveness	Enhance access to prevention and treatment benefits and services that are most likely to be cost-effective.	*Utilization:* The use and adherence rates for mammography, one of the most effective breast cancer screening procedures, still fall far short of the Year 2010 Healthy People objectives. *Satisfaction:* The quality of the doctor-patient relationship, including patient-centeredness and empathy, as well as cultural barriers to care, affect satisfaction with medical care in general, and particularly the satisfaction of women with their well-woman and related women's healthcare use.
Distributive and Social Justice			
Population at risk • Predisposing • Enabling • Need	Similar treatment	Minimize disparities in access to benefits and services across subgroups, particularly among those most at risk.	*Predisposing:* Mammography utilization and adherence rates decline with age and are lower for minority women and women in rural compared to urban areas. *Enabling:* Having a regular source of care enhances mammography screening rates, although other barriers (e.g., lack of transportation, limited access to screening services) may influence screening rates. *Need:* Death rates and rates of late stage of diagnosis are higher in elderly and black women.

Table 7.1 Criteria for Assessing Health Policies in Terms of Equity *(continued)*

Dimensions	*Criteria*	*Indicators*	*Examples*
Social Justice			
Environment • Physical • Social • Economic	Common good	Emphasize primary prevention (disease prevention and health promotion), and related . . .	*Environment:* The social determinants research documents the role of social and economic context and related exposures to environmental risks in influencing the relative risks of breast cancer. Specific environmental risk factors have not yet been fully documented, although genetic risk factors do influence recommendations regarding mammography screening intervals.
Health risks • Environmental • Behavioral	Need	. . . environmental and behavioral risk reduction.	*Health risks:* Smoking and obesity have been found to be associated with higher relative risks of breast cancer, although mammography screening guidelines do not explicitly take these factors into account.
		Substantive Equity	
Health • Patients • Community	Need	Reduce morbidity and mortality overall, as well as disparities between subgroups.	*Health:* The percentage of breast cancers diagnosed at a late stage are much higher for black and Hispanic compared to white women. Rates of death due to breast cancer are much higher for blacks compared to other races.

effective. The cost-effectiveness criterion is discussed more fully in Chapter 5. Rates of utilization of services that have been documented to be effective in preventing or remedying health problems, as well as patients' preferences and levels of satisfaction with care, provide useful input for judgments of the extent to which cost-effective services are being provided. Effective access is defined to exist when the use of selected services improves health status. The definition of efficient access is linked to the relative improvement in health status compared to healthcare costs (Andersen 1995; Andersen and Davidson 2001; IOM 1993).

Mammography screening has been documented to be the most effective technology to date for detecting early-stage breast cancer (Feig 2002; Humphrey et al. 2002). Although the rates of mammography use and adherence have increased over the past decade, they remain short of the Year 2010 Healthy People objectives—especially for racial and ethnic minority women (see Appendix 1.1). The quality of the doctor-patient relationship, including patient-centeredness and empathy, as well as cultural barriers to care, affect satisfaction with medical care in general, and particularly the satisfaction of women with their well-woman and related women's healthcare services use (Bibb 2001; Foxall, Barron, and Houfek 2001; Ramirez et al. 2000; Valdez et al. 2001).

The similar-treatment norm argues for minimizing disparities in access to benefits and services across subgroups, particularly among those most at risk. Variations in rates of services utilization and health outcomes are sentinel indicators of likely problems with the equity of healthcare services delivery. The predisposing, enabling, and need dimensions of the equity framework (Appendix 7.1) provide guidance for identifying potential equity issues related to the similar-treatment norm. Equitable access is defined to exist when predisposing factors, such as age and gender and/or need, account for most of the variation in use. Inequitable access occurs when system factors and predisposing social characteristics, such as race/ethnicity, and enabling factors, such as income or insurance coverage, determine who gets care (Andersen 1995; Andersen and Davidson 2001). The similar-treatment norm and related evidence regarding subgroup variations in breast cancer prevalence and screening rates will be central in evaluating mammography screening policy for older women (discussed in Chapter 9).

Deaths rates and rates of late stage of diagnosis are higher in elderly and black women. Nonetheless, mammography utilization and adherence rates decline with age, are lower for minority women, and

are lower for women in rural compared to urban areas. Having a regular source of care enhances mammography screening rates, although other barriers, such as lack of transportation and limited access to screening services, may influence screening rates, especially for those women most at risk (Amey, Miller, and Albrecht 1997; Bloom et al. 2001; Coughlin and Uhler 2002; Coughlin et al. 2002; Legler et al. 2002; Lorant et al. 2002; Miller and Champion 1997; Qureshi et al. 2000; Selvin and Brett 2003).

The norms of common good and need, grounded in the social justice paradigm, emphasize primary prevention (disease prevention and health promotion) and related environmental and behavioral-risk reduction. Public health and clinical research on the risk factors for the development of breast cancer has not yet identified clear primary breast cancer prevention strategies. Social determinants research documents the role of social and economic context, and related exposures to environmental risks, in influencing the relative risks of breast cancer. Specific environmental risk factors have not yet been fully documented, although genetic risk factors do influence recommendations regarding mammography screening intervals. Smoking and obesity have been found to be associated with higher relative risks of breast cancer, although breast cancer clinical risk assessment protocols and related mammography screening guidelines do not explicitly take these factors into account (Anglin 1998; Caplan et al. 2000; Chlebowski 2000; Davis et al. 1997; Johnson-Thompson and Guthrie 2000; Link et al. 1998; Maskarinec 2000; McCaul et al. 1996; Program on Breast Cancer and Environmental Risk Factors in New York State 2003; Safe 2000).

The bottom line in terms of achieving substantive equity is evidenced by successes in reducing morbidity and mortality overall as well as disparities between subgroups. The Healthy People 2010 objectives provide explicit benchmarks of success in judging that substantive equity has been achieved. As documented in Chapter 1 (Appendix 1.1), in terms of breast cancer, the rates and distribution of breast cancer in the United States fall short of the Healthy People 2010 objectives. The percentage of breast cancers diagnosed at a late stage are much higher for black and Hispanic women compared to white women. Rates of death due to breast cancer are much higher for blacks compared to other races.

In summary, major procedural and substantive equity issues exist with respect to incidence, screening, and treatment for breast cancer. In Chapter 9, effectiveness, efficiency, and equity criteria will be inte-

grated and applied in evaluating mammography screening for a particularly vulnerable group of women—those 65 and older covered by Medicare—and particularly the oldest subgroup of the elderly—women 75 years of age and older.

SUMMARY AND CONCLUSIONS

The health policy goal of equity has not yet been achieved to a substantial extent in the United States. Significant health disparities persist between racial, ethnic, and socioeconomic groups. Vulnerable populations remain at risk of receiving less, or less than adequate, healthcare. Both public and private policymaking appears to eschew rather than elicit the views of affected stakeholders. A core argument of this book is that the ultimate measure of success of U.S. health policy is the level of improvement in the health of the population. This and the previous chapter have attempted to provide a conceptual blueprint and methodological tools for designing health policy directed toward more effectively achieving this objective.

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Appendix 7.1 Highlights of Selected Indicators of Equity of Access to Healthcare

Potential Access

Delivery System

Availability: Distribution of providers (Center for Studying Health System Change 2002; Health Resources and Services Administration, Bureau of Health Professions, Division of Nursing 2002; NCHS 2003b)

Active, nonfederal physicians in patient care per 10,000 civilian population (2001)

U.S. = 22.6	D.C. = 54.6	Texas = 18.2	Alaska = 17.0

Employed nurses per 10,000 resident population (2000)

U.S. = 78.2	D.C. = 167.5	Texas = 60.6	Alaska = 78.4

% nonfederal patient care physicians providing any charity care

1997	1999	2001
76.3	72.1	71.5

% nonfederal patient care physicians receiving any revenue from Medicaid

1997	1999	2001
87.1	86.8	85.4

% nonfederal patient care physicians not accepting new patients, by patient insurance status

	1997	1999	2001
Medicaid	19.4	19.1	20.9
Uninsured	NA	NA	16.2
Medicare	3.1	3.4	3.8
Private	3.6	3.6	4.9

Appendix 7.1 Highlights of Selected Indicators of Equity of Access to Healthcare *(continued)*

Organization: Types of facilities (NCHS 1997, 1998, 2003b)

Community hospital beds per 1,000 civilian population (U.S.)[1]

1960 = 3.6	1970 = 4.3	1980 = 4.5	1990 = 3.7	2001 = 2.9

Health maintenance organizations (all plans, U.S.)

1976 = 174	1980 = 235	1990 = 572	1995 = 562	1997 = 652
1998 = 651	1999 = 643	2000 = 568	2001 = 541	2002 = 500

Nursing home beds per 1,000 resident population 85 years of age and over (U.S.)[2]

1976 = 685.3	1986 = 542.1	1991 = 494.5	1995 = 482.7	2001 = 404.2

Inpatient and residential treatment beds in mental health organizations per 100,000 civilian population (U.S.)[1]

1970 = 263.6	1980 = 124.3	1986 = 111.7	1988 = 111.4	1990 = 111.6
1992 = 107.5	1994 = 112.1	1998 = 99.1		

Appendix 7.1 Highlights of Selected Indicators of Equity of Access to Healthcare *(continued)*

Financing: Sources of payment (NCHS 2003b)

% distribution of selected expenditures for health services and supplies (U.S.)[3]

	1987	1994	1995	1996	1997	1998	1999	2000
Public								
Federal government	15.7	20.4	20.5	21.2	20.8	19.3	19.0	18.9
State and local government	14.9	16.2	16.1	15.8	16.0	16.2	16.7	16.9
Private								
Employer contribution to private health insurance premiums	17.9	19.1	19.2	19.4	18.7	18.9	19.1	19.6
Employee contribution to private health insurance premiums and individual policy premiums	8.7	9.8	10.0	9.6	10.2	10.4	10.2	10.1
Out-of-pocket payments	22.8	15.9	15.3	15.1	15.4	15.7	15.7	15.5
Other private funds	4.7	4.2	4.4	4.4	4.6	4.7	4.5	4.2

Appendix 7.1 Highlights of Selected Indicators of Equity of Access to Healthcare *(continued)*

Population at Risk

Enabling: Regular source of care (NCHS 2003d, 2003e)

% distribution of regular care, by race, income (1999)

	Race			Income			
	White	Black	Hispanic	<$20,000	$20,000–$34,999	$55,000–$74,999	$75,000+
Children 0–17 years							
No regular source of care	4.5	6.1	13.6	12.4	9.4	2.9	1.7
Location of care for those with regular source							
Doctor's office	82.5	69.1	63.8	58.2	69.0	83.2	89.0
Clinic	15.9	26.3	31.9	36.6	27.1	15.0	10.2
Emergency room	0.2	0.9	1.1	1.4	*0.4	*0.3	*0.0
Hospital outpatient	0.8	3.3	2.5	2.9	2.8	*1.0	*0.5
Adults 18+ years							
No regular source of care	13.3	16.4	28.3	21.8	19.7	11.3	9.7
Location of care for those with regular source							
Doctor's office or HMO	82.0	72.0	70.5	66.3	75.9	83.5	86.6
Clinic or health center	15.5	20.9	24.0	26.5	19.8	14.5	11.8
Hospital emergency room or outpatient department	1.8	6.4	4.6	5.6	3.6	1.4	1.0

* Figure does not meet standard of reliability or precision.

Appendix 7.1 Highlights of Selected Indicators of Equity of Access to Healthcare *(continued)*

Enabling: Insurance coverage (Kaiser Commission on Medicaid and the Uninsured 2002b; U.S. Census Bureau 2003)

% U.S. population without insurance[1]

1987 = 12.9	1990 = 13.9	1992 = 15.0	1995 = 15.4	1998 = 16.3
1999 = 15.5	2000 = 14.2	2001 = 14.6	2002 = 15.2	

% uninsured by race/ethnicity, income (2002)

Race			Income			
White	Black	Hispanic	<$25,000	$25,000–$49,999	$50,000–$74,999	$75,000+
14.2	20.2	32.4	23.5	19.3	11.8	8.2

% insured individuals whose families in the past year (2001)

Postponed seeking medical care	18
Had problem paying medical bill	15
Needed prescription but did not get it	10
Were contacted by collection agency about a medical bill	8
Did not get the medical care they felt they needed	6

% insured, nonelderly adults who reported that they lacked (2001)

Prescription drug coverage	10
Dental coverage	29
Vision coverage	37

Appendix 7.1 Highlights of Selected Indicators of Equity of Access to Healthcare *(continued)*

Actual Access

Utilization

Type of use: Use of selected services (NCHS 2003a, 2003b)

% having had procedure or contact, by race/ethnicity or income

	Race			Income[4]		
	White	Black	Hispanic	Poor	Near Poor	Middle/High
Began prenatal care first trimester (2001)	89	75	76	—	—	—
Vaccinations, children (19–35 months) (2001)						
one dose MMR	92	89	92	89	90	91
four doses DtaP	84	77	82	76	79	84
three doses polio	90	85	91	87	89	90
Saw dentist, past year (age 2+ years) (2001)	69.6	56.9	51.2	47.0	49.7	72.0
Women who received a mammogram in past 2 years (age 40+) (2000)	72	68	62	55	56	76
Women who received a Pap smear in past 3 years (age 18+) (2000)	83	84	77	72	75	85

Purpose of use: Use of services relative to need (NCHS 2003b)

Physician visits relative to need, by race/income (2001)

	Race			Income		
Need	White	Black	Hispanic	Poor	Near Poor	Nonpoor
% in fair or poor health (2001)	7.9	15.5	12.7	21.0	15.5	6.2
% with limitation in usual activity due to chronic condition (2001)	12.1	15.5	10.6	24.1	18.8	9.9

— Data not available.

Appendix 7.1 Highlights of Selected Indicators of Equity of Access to Healthcare *(continued)*

Volume of use (NCHS 2003b)

	Race			Income		
	White	Black	Hispanic	Poor	Near Poor	Nonpoor
% with healthcare visit to doctor's office, emergency department, or home visit during the past year (2001)						
	85.7	83.6	73.0	78.3	79.6	86.0
% with 1–3, 4–9 or 10+ healthcare visits to doctor's office, emergency department, or home visit during the past year (2001)						
1–3	46.4	46.4	40.2	37.2	41.4	47.4
4–9	25.4	24.0	20.7	23.4	22.9	25.8
10+	13.9	13.1	12.0	17.7	15.3	12.8

Unmet need (NCHS 2003c)

% not receiving needed medical care in the past year due to cost, by race, income, insurance status, current health status (1999)[5]

Race	White	4.0
	Black	5.5
	Hispanic	4.6
Income	<$20,000	9.9
	$20,000–$34,999	6.9
	$35,000–$54,999	3.4
	$55,000–$74,999	1.7
	$75,000+	0.8
Insurance	Private	1.9
	Medicaid/other public	4.7
	Other coverage	7.2
	Uninsured	16.5
Health status	Excellent, very good, or good	3.4
	Fair or poor	12.9

Appendix 7.1 Highlights of Selected Indicators of Equity of Access to Healthcare *(continued)*

Satisfaction

General: Public opinion (Blendon et al. 2002)

	Australia	Canada	New Zealand	United Kingdom	United States
Citizens' overall views of their healthcare system (2001)					
% who reported					
Only minor changes needed	25	21	18	21	18
Fundamental changes needed	53	59	60	60	51
Complete rebuild needed	19	18	20	18	28
Citizens' views of access to and cost of care					
% who reported					
Very or extremely difficult to see a specialist	12	16	11	13	17
Access worse than two years ago	19	26	15	17	20
Often or sometimes unable to get care because it is not available where you live	17	21	18	13	20
Did not fill a prescription due to cost	19	13	15	7	26
Did not get medical care due to cost	11	5	20	3	24
Did not get test, treatment, or follow-up care due to cost	15	6	14	2	22
Problems paying medical bills	11	7	12	3	21

Notes:

[1] Changes in definitional and reporting procedures affect the comparability of data across the period.

[2] Estimates for 1995 and 2001 computed by author, based on estimates of resident population age 85+ in NCHS (1998) and NCHS (2003b) and nursing home beds in NCHS (2003b).

[3] Excludes research and construction.

[4] For dental visits, income categories are poor, near poor, and nonpoor.

[5] Health insurance status for persons under 65 years.

Chapter 8

Integrating Health Services Research and Policy Analysis

CHAPTER HIGHLIGHTS

1. The general objectives of policy analysis include the production and interpretation of descriptive, normative, and prescriptive information —or facts, values, and trade-offs, respectively—for understanding policy problems and identifying solutions.
2. The objectives of policy analysis may vary depending on the decisions faced by policymakers in a particular stage of policy development.
3. The health services research perspectives of effectiveness, efficiency, and equity offer conceptual frameworks, measures, and methods that can be applied in policy analysis.
4. The policy relevance of these perspectives can be enhanced by stronger health services research evidence on the relationship between health-care structures, processes, and outcomes and the impact of health-related environmental, economic, and social factors.

OVERVIEW

Health services researchers are routinely involved in producing and analyzing policy-relevant information. The objective of this chapter is to show how the two are related. Policy analysis is defined in greater detail (see Chapter 1 for an introduction) in the first section and related to the policy-development process. The second section explores standards in policy analysis revealing the multifaceted nature of the field. In the third section, different tasks in policy analysis are examined and related to the effectiveness, efficiency, and equity perspectives of health services research. The final section reviews some of the limitations in using health services research as a resource for policy analysis.

OBJECTIVES OF POLICY ANALYSIS

Whether public policies take the form of laws, programs, rules and regulations, or judicial decisions, they are made through a process of decisions or choices about what the objectives of government should be and the means of achieving them.[1] This general characterization of policymaking applies at any level of government—federal, state, or local—and in any policy area, including education, defense, welfare, or health. The focus of policy analysis is on determining the need for and supplying descriptive, normative, and prescriptive information to facilitate public debate (Dunn 2003).

Descriptive information is the factual material that documents social conditions and trends (e.g., a decrease in the number of uninsured, an increase in healthcare costs) or analyzes the potential or actual consequences of alternative policy actions (e.g., a forecast of the number of people who would be covered under a Medicare prescription drug program, an evaluation of the reduction in breast cancer mortality that occurred among women receiving Medicaid-covered mammography screening). The types of questions that descriptive information addresses are, Does a particular social problem exist? What are the consequences of past actions to solve the problem? What are the potential consequences of alternative actions? Such information is particularly relevant in the policy formulation stage of the policymaking process (Longest 2002).

Normative information, on the other hand, combines factual analysis with values to ask whether a particular social condition or trend deserves attention or whether a particular policy should be judged potentially valuable. For example, after providing factual information about the populations that would be covered under alternative Medicare prescription drug proposals, the analyst may attempt to determine the preferred alternative to achieve a particular equity objective such as equal treatment patterns across population groups. Note the introduction of values in the selection of the equity objective that may be based on the preferences of elected officials, surveys of the general public, the analyst's own professional training, interest groups, etc. Such values are debatable and sometimes conflicting, even when each may be justified in terms of some desirable principle for public action. Rational decisions require that such values be clear and quantitatively measurable, if possible, so that trade-offs can be identified (MacRae and Whittington 1997).

Prescriptive information goes further in supporting recommendations for specific action based on trade-offs of valued consequences of policy alternatives. Prescriptive information is important in moving a problem from the discussion agenda in policy development to the action agenda (Kingdon 2003). Assuming a rational policy process, it provides the basis for selection of a particular policy action. For example, after providing normative information about the potential of a particular Medicare prescription drug benefit proposal for achieving a particular equity objective, a policy analyst may be asked to develop prescriptive information of that proposal's relative worth compared to other similar proposals (i.e., different proposed drug benefit legislation with alternative patient copayment options) in assisting policymakers to arrive at a reasoned choice. Prescriptive information facilitates such choices by clarifying the potential consequences of alternatives, making trade-offs explicit, combining criteria in measuring consequences (i.e., using cost-benefit analysis), and determining priorities or decision rules (Weimer and Vining 1999).

Analysts interested in meeting these informational and analytic needs in the policy-development process are involved in (1) producing and/or interpreting descriptive, normative, or prescriptive information about social conditions and past or future alternatives for improving them; and (2) developing arguments translating such information into claims for government action (Dunn 2003). Findings from the first objective become the information used to develop or influence the arguments in objective number two, leading to the recognition of a specific problem or policy action. The second objective involves creating and critically assessing policy-relevant information by examining the validity of the data being brought forth, the values being explicitly or implicitly applied in interpreting that information, the logic of the claims being made, and the acceptability of the underlying assumptions.

Alternative views of the policy-development process lead to different definitions of the objectives of policy analysis. Awareness of these views facilitates a deeper understanding of the nature of policy analysis and the possible contribution of health services research.

The objectives discussed above are consistent with the *rational-comprehensive* view of policy development, which dates back to the philosophical writings of John Dewey (1927) and other American pragmatists.

According to this view, the policymaking process consists of the following series of logical, well-defined steps in problem solving:

1. Define the problem.
2. Identify a range of alternatives with the potential to resolve the problem.
3. Evaluate and select the alternative that best addresses the problem.
4. Describe and evaluate the consequences of the selected alternative after it has been implemented.
5. Evaluate and modify the alternative in light of its consequences.

This model idealizes the policymaker as an objective, well-informed individual serving the public interest. The model, as applied by one of the founders of modern policy analysis (Lasswell 1951), suggests a process of reasoning and comprehensive analyses to identify policies for resolving problems in a logical and orderly manner. It implies a major role for policy analysis as a "meta-discipline," providing information and clarifying values needed to pursue logical solutions to substantive problems through multidisciplinary research (Dunn 2003). The objectives of such analysis are to consider in a linear fashion all possible definitions of a problem in arriving at a correct definition, express government goals and values clearly and specifically, conduct a thorough examination of all possible alternatives to address the problem, and undertake an exhaustive assessment of effects of each alternative to arrive at an optimal choice. With this information, policy formulation becomes a logical exercise in social problem solving.

An alternative view recognizes that information needed for policymaking is often limited and difficult to interpret and that policymakers bring conflicting objectives and ideological perspectives to the policymaking process. The objectives of the *incremental* or *satisficing model* (Hayes 2001; Lindblom 1959) are less ambitious than the rational-comprehensive model: to find policies that are acceptable to a reasonable number of people and that alleviate some of the shortcomings in past policies. Whereas the rational-comprehensive model looks to correctly define the problem and select the optimal course of action, the incremental model selects the first choice that is minimally acceptable, and by way of a process of discovery through trial and error, strives to improve on the original choice. In this model, prospective policy analy-

sis, which relies on theory and analysis before policy actions are initiated and implemented, is less important than retrospective analysis, which provides feedback after policy actions have been taken.

For example, the rational-comprehensive approach to selecting an outreach policy to increase enrollment in the State Children's Health Insurance Program would involve an extensive review of existing theory and empirical research on the effectiveness of different marketing and educational strategies. The policy selected would be based on an ideal model of what would maximize the outreach objectives of the state at the least cost. Alternatively, an incremental approach would draw on research to some extent but, recognizing the limits of research and the problem of getting policymakers to agree, look for an approach that modified the current enrollment process to minimally satisfy policy objectives. Proponents of the incremental model believe that successive, limited changes ultimately lead to better definitions of problems, objectives, and consequences of various alternatives and eventually lead to the best policy. The major role of policy analysis is to supply feedback through evaluation of policy outcomes and to translate that information into recommendations for policy modification.

The rationalistic models—comprehensive and incremental—have both been criticized for their failure to recognize the political context of the policy process. As an alternative, a *political* view has been proposed that emphasizes the limitations of objective analysis to address policy questions; the multiplicity of stakeholders with different views, incentives, sources of power, and influence that are involved; and the complex and sometimes overlapping systems of responsibility between different branches of government (Stone 2002). This model challenges the characterization of policymaking as fundamentally a rational or even quasi-rational process.

According to the political view, policymaking is a messy, fragmented, discontinuous, and often seemingly random process of conflict resolution and consensus building among self-interested groups. Problems and solutions are addressed in this process only to the extent that they happen to reflect the individual goals of interest groups, not as a result of problem solvers attempting to make choices in the public interest. Outcomes of the policy process depend more on the ability of affected groups to organize and influence the political process than on the extent to which a policy achieves a given end. In a recent address, the chair

of AcademyHealth (the professional association for health services researchers in the United States) stated that "politics is not about truth; it's about values, and interests, and votes, and money" (Feder 2003, 3).

The symbolic analogy of the garbage can is used to depict the political view of policymaking as irrational and nonsystematic (March and Olsen 1979). Policy decisions reflect garbage cans whereby the mix of garbage in the can depends on the actors with influence at the moment, the number of cans available, and the speed with which garbage arrives and leaves the scene. In a classic study of the process of agenda setting and policy formulation, John Kingdon (2003) identified three parts of the process that come together when items get on the political agenda: identifying and agreeing on a well-defined and recognizable problem that needs attention, developing and diffusing solutions, and building interest in the general public and among political leaders in taking policy action. These three "streams" flow independently, creating a haphazard process of agenda setting and policy development. The convergence of all three streams creates opportunities for major policy change. Conducting research related to defining and understanding problems and determining effective proposals plays a role, but it is the political interactions of individuals and groups that determine whether a particular conceptualization of the problem, or proposed solution, makes it onto the decision agenda.

The *postpositivist* view, a variation of the political view with a particular perspective on interest group politics, also is critical of rationalism and its tendency toward a "tyranny of experts" in service of the status quo (Dryzek 1993; Habermas 1989). In its place, postpositivists embrace a bottom-up view of a policy process in which issues arise from affected populations who do not have the power to influence the process. Postpositivists maintain that the primary responsibility of the policy analyst is to attempt to offset the natural tendency for policy decisions to reflect the unequal distribution of power. The postpositivist model is lodged in the deliberative justice paradigm and related principles of the full participation of affected parties in policy formulation and analysis (discussed in Chapter 6). Practicing analysts are to rely on participatory modes of inquiry (e.g., focus groups or other group processes), collaborating with affected populations in the pursuit of reasonable assessment and debate in policy design (Durning 1999; Fischer and Forester 1993; Forester 1993; Friedmann 1987). The emphasis on reasonable discourse

shifts from policy elites to public opinion assessment and consensus formation. A particular analyst's success depends on eliciting public views free of the biases of the researcher and policymakers.

Notwithstanding alternative views of the policy process and the limitations of research and analysis, textbooks on policy analysis continue to adhere to the rational model as a framework for analysts to follow (Dunn 2003; MacRae and Whittington 1997; Patton and Sawicki 1993; Weimer and Vining 1999). It is widely recognized, however, that actual analysis may not follow the sequence of steps suggested by the model, may not be as thorough or rigorous as the model implies, and will be more politicized than the model suggests. The implication is that the policy analyst should generally follow the rational model (i.e., attempt to apply analytic skills and tools to clarify problems, systematically search for alternatives, and comprehensively evaluate alternatives) but he or she should also be aware of the subjectivity of analysis; recognize the multiplicity of legitimate value perspectives; and, to the extent possible, be involved in ensuring that affected parties are included in the policymaking process (Romero 2001). The limits of information, the subjective nature of analysis, and the political nature of the process in a given context must be considered in determining the type of analysis appropriate to a given question. Analyses should not be viewed as a substitute for the judgment, insight, and creativity of the policymaker. It is suggested, however, that systematic analysis at different stages of the policy process will enhance policymakers' decisions. Finally, the analyst should seek greater involvement in the process of analysis of the groups and individuals to be affected by the policy, encourage an open and visible process of decision making, emphasize negotiation, and recognize the role that values play in the entire policy process.

Standards in Policy Analysis

The broad objectives of policy analysis suggest general criteria for identifying sound policy analysis: relevance, validity, and reasonableness (Dunn 2003). *Relevance* refers to the extent that an analysis addresses actual policy issues of concern, that is, the specific questions about the social conditions, values, or alternatives being debated at a particular time in the policy-development process. For example, does the research address a specific bill before Congress or legislative proposal being considered in a state governor's office? It also refers to the extent that

an analysis reflects the constraints and opportunities of a particular policy context. For example, does the analysis reflect the appropriate degree of uncertainty about the nature of a problem, recognize the state of related policies and programs, reflect budgetary or administrative limitations, and adopt appropriate time frames?

Validity refers to the accuracy or precision of information being used to answer a particular question. The standards of validity in policy analysis are context specific (Durning 1999; Lynn 1999). The appropriate level of precision required is based on whether additional precision would add clarity to a choice (MacRae and Whittington 1997). For example, the desirability of a government-subsidized work-site childcare center may depend on information concerning the benefits and costs of establishing the center. If, upon preliminary examination, it becomes clear that the costs are such that the benefits required to favor the policy are beyond what might possibly occur under the most optimistic assumptions, then accurate estimates of the actual benefits may be unnecessary.

Establishing validity in policy analysis in terms of relevant cause-and-effect theories of institutional or individual behavior and carefully defined and empirically tested models of policy consequences based on these theories is beyond what the field has achieved and what most people in the field think possible (Hayes 2001). Establishing validity in policy development is difficult because of the unique context in which most decisions are made. Nevertheless, the pursuit of validity through the use of conceptual frameworks, theories of behavior, logical deduction, and empirical evidence is an important goal for policy analysis and often can illuminate meaningful policy choices (Lynn, Heinrich, and Hill 2001).

Recognizing the limits of validity and the important role of values in policy analysis leads to the importance of *reasonableness* as a standard. The same facts (e.g., data showing that disparities in health status are growing among socioeconomic groups) lead different analysts to different claims of the nature of policy problems (i.e., the need for reforms in healthcare coverage versus investments in improving social conditions). Such differences often stem from ideological or philosophical differences among stakeholders in the policy-development process, for example, different views among Democrats and Republicans over the basic role and responsibility of government in ensuring equality of health status. Sometimes the differences result from alternative frames

of reference of the analyst, such as economic benefit versus political feasibility, as the basis for an argument. The differences may also rest on the underlying assumptions used regarding individual, group, or institutional behavior. Recognizing these differences leads to the realization that all policy analysis is to some extent subjective and, therefore, reasonableness is a more realistic standard to use in evaluating policy analysis than the traditional scientific standard of empirical verification or replication.

Reasonableness in policy analysis has been defined as the extent to which a policy argument meets certain criteria of logical structure and completeness. It is assessed through examining a policy argument's empirical base, its underlying assumptions, and its internal logic. The following specific criteria for evaluating reasonableness in policy arguments have been put forth by Dunn (2003):

1. Completeness—does the argument include all appropriate, policy-relevant information and assumptions?
2. Consonance—are all the elements of the argument valid and internally consistent?
3. Cohesiveness—are all the elements of the argument operationally connected?
4. Functional regularity—are all the elements of the argument in accordance with expected methods, procedures, or patterns?
5. Functional simplicity—are all the elements of the argument arranged in a simple and understandable way contributing to the effective transfer of knowledge?

A recommendation that Medicare recipients be given incentives to enroll in private managed care plans illustrates the nature of each element. The basis of the argument may be information that managed care plans are more efficient than the traditional fee-for-service Medicare plan. Policy-relevant information on the relative efficiency of managed care plans serving the elderly may be referenced to support the recommendation. The analyst might provide further support by referring to healthcare costs in the federal budget or the prospects for insolvency in the Medicare program. Further assumptions, arguments, or principles for the recommendation may also be needed to complete the argument. Additional support for managed care in Medicare might include its functional regularity with the proposition that managed care in the

commercial insurance market and in the Medicaid program has been an effective cost-control strategy. Finally, the analyst should be able to explain the methods used in the analysis (i.e., using observational studies, experiments, etc.) in a manner that can be understood and evaluated by nonexperts in the field.

HEALTH SERVICES RESEARCH IN POLICY ANALYSIS

The policy analyst works with a variety of value frameworks, research methods, and analytic procedures to address policy questions. The appropriateness of a particular approach depends on the kind of question being asked and on the current stage of the policy process. The health services research field offers concepts, measures, and procedures for assessing effectiveness, efficiency, and equity in health services and systems that can be used for examining policy questions. Table 8.1 provides a summary list of policy decisions associated with different stages of policy development, information that may be relevant at each stage, and the various types of research and analysis that may be relevant.

Selecting Normative Criteria

Definitions of policy problems and solutions are guided by the values that underlie concern about social conditions. Such values may come from policymakers; from the community in terms of individual testimony, surveys, or focus groups; from expert or stakeholder groups; from policymakers themselves; or from the analyst's own conscience or professional training. To obtain information on values and criteria, the analyst may ask the relevant policymaking body or affected populations or rely on some kind of observational analysis of past decisions, legislation, testimony, or other written material to infer what the norms might be (MacRae and Whittington 1997; Patton and Sawicki 1993). Expert panels can sometimes provide a standard. For instance, the Agency for Healthcare Research and Quality clinical guidelines described in Chapter 3 offer a standard for identifying a problem in breast cancer policy: lack of access to mammography screening and treatment in uninsured populations, an effective screening procedure shown to reduce mortality in women age 40 to 70 years old. There are occasions when government officials have defined a normative standard in specific terms, such as the Healthy People 2010 Health Objectives of the U.S. Public Health Service regarding mammography screening rates for all

Table 8.1 Stages of Policymaking, Relevant Information, and Type of Research

Stages of Policymaking	*Relevant Information*	*Type of Research*
1. Define problems	Scope, severity, causes, importance of the problem	Conceptual analyses or descriptive studies of the problems and causes
2. Identify alternatives	Forecasts of likely consequences of alternatives	Conceptual or empirical projections of the consequences of alternatives
3. Evaluate alternatives	Normative evaluations prior to action	Applications of normative frameworks for prescription
4. Describe consequences	Implementation and impact of policies and programs	Descriptive studies of program and policy effects
5. Evaluate consequences	Normative evaluation of consequences	Normative studies of program and policy effects

women age 40 and older (Office of Disease Prevention and Health Promotion 2003).

As indicated in Chapter 2, the health services research literature offers two possible ways of defining effectiveness criteria in health policy analysis: the population perspective, focusing broadly on the importance of social, behavioral, environmental, and medical care factors to the health of the population; and the clinical perspective, focusing more narrowly on the clinical effectiveness of medical care for the individual patient. From the population perspective, effectiveness is defined in terms of the proportion of the population with a health problem who benefit from a healthcare intervention or from changes in social, behavioral, or environmental circumstances. The clinical perspective on effectiveness focuses more narrowly on the benefits achieved by individuals or groups of patients receiving medical care under conditions of actual practice. Although often confused with the population perspective, this perspective leads to an evaluation standard in terms

of actual benefits in medical practice compared to maximum achievable benefits—that is, compared to efficacy.

As described in Chapter 4, defining policy goals of efficiency in healthcare may also be approached in two ways: at the macro level, by encouraging the right mix of medical and nonmedical, health-related investments to maximize social welfare (i.e., allocative efficiency); or at the micro level, by encouraging the right mix of inputs and production methods to maximize the productivity of targeted services and systems (i.e., production efficiency). Criteria for analysis in both cases include production and cost standards deduced from microeconomic theory and measures derived from applying cost-effectiveness or cost-benefit frameworks.

As discussed in Chapter 6, equity values in healthcare policy have traditionally derived from ethical principles of distributive justice involving the fair distribution of the benefits and burdens of medical care. Public health policy has been primarily governed by the social justice notion of promoting the health of the community as a whole. The deliberative justice paradigm is proposed as a guide in policy development, bridging competing values by suggesting a process for policymaking in which affected parties participate and contribute. While debate continues over specific criteria that should serve as the basis for defining equity in healthcare delivery and policy, such criteria can be derived for a number of alternative principles (Table 6.3). Each can, in turn, be translated into quantitative or qualitative indicators, as explained in Chapters 6 and 7, to evaluate the extent to which equity has been achieved.

Table 8.2 provides a summary list of criteria and possible indicators that can be drawn from the three perspectives of health services research.

Defining Problems and Their Causes

The objective in the problem-definition stage is to clarify what the problem is about, why it exists, whom it affects, and possible solutions. Analysis is needed because most problems appear as vaguely defined concerns expressed by some interest group. There are three distinct aspects of problem definition and clarification for which policy analysis may be useful. One is to determine what policy objectives or criteria should be used in defining a particular social condition as a problem. The second is to evaluate the scope and magnitude of the problem, and the third is to determine possible causes.

Table 8.2 Criteria for Assessing Health Policies in Terms of Effectiveness, Efficiency, and Equity

Dimensions	*Criteria*
Effectiveness	
Population perspective	Maximize population health.
Clinical perspective	Maximize actual health benefits of healthcare services compared to potential benefits.
Efficiency	
Allocative efficiency	Ensure a mix of healthcare services that maximizes a combination of health outcomes and consumer satisfaction at least cost.
Production efficiency	Produce healthcare services that maximize output at least cost.
Equity	
Distributive justice	Maximize freedom of choice of plans/providers/services, cost-effectiveness, similar treatment.
Social justice	Maximize common good. Meet basic needs.
Deliberative justice	Maximize participation of affected parties.

As noted previously, the framework for defining problems of clinical effectiveness may involve the system, institution, or patient level (see Table 2.2). Policy analysis aimed at improving effectiveness in this way would compare the relative contributions of medical care and other population-oriented factors to the quality and length of life. The health problems associated with poverty, inadequate housing, smoking, or drug abuse might be contrasted with those resulting from poor access to medical care. Explicit analyses of the health effects of patient behavior and environmental conditions as well as the quality of medical care are relevant in this perspective.

The microeconomic model of healthcare provider performance analyzes the relationship between different levels and mixes of inputs, input

prices, and technology that minimize the cost of services. It can be used in policy analysis when the concern is the production of a specific service or mix of services. For example, each setting for healthcare—such as a community health center, hospital, or nursing home—uses a particular combination of health personnel, supported by other inputs, to produce services. The microeconomic model suggests criteria that can be used to empirically identify the most efficient combination of personnel, supplies, and other inputs to support a particular level of healthcare service.

The cost-effectiveness framework, on the other hand, may be used when the concern is the comparison of the relative efficiency of policies or programs that try to improve health through alternative methods of production. A cost-effectiveness ratio (e.g., cost per encounter, per case found, or per quality-adjusted life year) is computed for each alternative and compared to that of other alternatives. It is important to note that production efficiency also requires that services be effective. Efficiency analysis must be preceded by the technical appraisal of effectiveness. Once a policy or program is shown to be effective, either in clinical- or population-oriented terms, cost-effectiveness analysis compares its relative effectiveness and costs to other effective options.

The broader goal of allocative efficiency is assessed using the cost-benefit framework. The analyst calculates and compares the costs and benefits of a policy, program, or service to determine if it adds to social welfare, that is, if the social benefits exceed the social costs. All relevant social costs—including cost savings that may be associated with prevention services—and benefits must be identified and measured in dollars, if possible, so that comparisons of costs versus benefits can be made across all possible actions. Future costs and benefits must be discounted to reflect their present value. Subtracting costs from benefits yields net benefits, the criterion indicating increased social welfare. Allocative inefficiencies are indicated when the aggregate costs of a policy or program exceed its aggregate benefits.

Equity criteria related to healthcare delivery are based on the characteristics of the delivery system (e.g., the availability and distribution of services), the characteristics of the population (e.g., ethnicity, gender, insurance coverage, the availability of a regular source of care), the use of services, and satisfaction with services (Table 6.3). Equity-of-access objectives may be evaluated at the institutional, system, or population level by applying these criteria. Equity analysis in the context

of the social justice paradigm may be applied to the distribution of health and health risks and to the relationship of health risks to the physical, social, and economic environment. Deliberative justice norms would assay the extent to which individuals and groups affected by policies at the micro or macro level participate in the formulation and implementation of these policies.

Successful completion of the tasks involved in problem definition provides necessary information for moving to the next stage of policy-making—suggesting solutions through an understanding of the problem and of the policy objectives at stake. For example, critics of traditional fee-for-service Medicaid cite the following deficiencies in the program: inappropriate, expensive services are often provided (e.g., primary care is obtained in hospital emergency rooms); no accountability for outcomes exists (e.g., information is rarely collected at the provider level on measures such as childhood immunization rates or pregnancy outcomes); and access to care is lacking (e.g., many providers do not accept Medicaid reimbursement). Many states have attempted to remedy these shortcomings by implementing managed care in their Medicaid programs (Freund and Hurley 1995).

Table 8.3 provides a summary list of the problem analyses that can be drawn from the three perspectives of health services research.

Identifying and Evaluating Policy Proposals

In this stage of policy development, the objective is to identify policy alternatives that have the potential to correct, compensate for, or counteract policy problems and to project their consequences in terms of defined values and objectives. The generation of policy options for consideration in the policy-development process combines methods for searching among existing strategies and conceiving, or creating, entirely new ideas (Alexander 1982). Analytical tasks in evaluating alternatives are the identification of the mix of goals and objectives that are to be used to evaluate different alternatives, the translation of these goals and objectives into specific quantitative or qualitative criteria, and their application to the projected effects of alternatives (Dunn 2003; Patton and Sawicki 1993).

Facilitating the creation of new solutions involves a variety of tasks ranging from designing group processes that strive to be nonjudgmental or to enhance participants' ability to retrieve unrelated ideas or information from memory, to methods for an analyst to develop new

Table 8.3 Problem Analyses in Terms of Effectiveness, Efficiency, and Equity

Dimensions	*Analyses*
Effectiveness	
Population perspective	Compare the relative contributions of healthcare and other population-oriented factors to the quality and quantity of life.
Clinical perspective	Compare actual health benefits of individuals or groups receiving a healthcare service to potential benefits.
Efficiency	
Allocative efficiency	Identify services, systems, organizational arrangements, and financial mechanisms that are not cost-beneficial.
Production efficiency	Identify services and systems with similar objectives that are not cost-effective.
Equity	
Distributive justice	Apply equity-of-access model to estimate disparities in healthcare services and systems.
Social justice	Estimate disparities in health and health risks.
Deliberative justice	Estimate lack of participation of affected parties in policy development.

solutions by modifying existing solutions in light of a given problem. A variety of more or less systematic search techniques may be employed to identify alternatives ranging from in-depth research and experimentation to quick surveys and literature reviews. The best approach will vary with the policy context and the resource and time limitations of the analysis.

Forecasting the potential consequences of alternatives provides useful information in this phase of formulation. Statistical models and simulation techniques may aid the analyst in generating projections of policy consequences. For example, during the 1992–94 national health reform debate, U.S. Congressional Budget Office forecasts of the budget effects of the Clinton Health Security Act and other reform plans played

a particularly important role (Peterson 1995). The Centers for Medicare & Medicaid Services developed a ten-year projection of health spending by category of service for the first decade of the twenty-first century based on a variety of time series and behavioral modeling techniques (Heffler et al. 2002). More commonly, the analyst must rely on simpler techniques such as theoretical inference or subjective opinion to project consequences.

The structure-process-outcomes framework developed by Donabedian (1966, 2003) and Kane (1997) as the conceptual guide for clinical effectiveness research is useful in the policy-analytic task of a priori identification and evaluation of policy alternatives. This framework can be applied at the system, institution, or patient level to evaluate possible ways to improve the effectiveness of medical care through manipulation of structure and process variables. The framework suggests the kind of data needed to identify possible solutions to an effectiveness problem. Evidence linking the elements of the framework to outcomes suggests targets for interventions. For example, in clarifying a policymaker's concern about the quality of care in nursing homes, the structure-process-outcomes framework suggests that the quality of nursing home care is influenced by structural factors such as the quantity of staff and their qualifications. Quality, in turn, has an influence on outcomes, including mortality, morbidity, functional status, and client satisfaction. The framework indicates the structure and process factors that are subject to policy manipulation to improve the effectiveness of care.

Research concerned with allocative and production efficiency informs policymakers about what alternatives tend to result in the provision of effective services that are relatively inexpensive to deliver. Numerous empirical studies show, for example, that HMO patients' use of hospitals is much less than that of fee-for-service patients with no corresponding reduction in the effectiveness of care (Miller and Luft 1994, 1997, 2002; Rosenthal and Newhouse 2002) and that cost sharing results in lower use and lower cost of medical care with little or no decline in health status for the average patient (Newhouse et al. 1987). Researchers are attempting to provide better information on the efficiency of a variety of specific medical care services aimed at common medical problems and on the resources, organizational arrangements, and financing mechanisms involved in their provision.

Solutions to efficiency problems may also be identified and evaluated through analysis of medical care market conditions (see Chapter

4). Microeconomic theory identifies market conditions that lead to inefficiencies in production or allocation if not corrected. Many of these conditions have been shown to be present in medical care markets. For example, the uncertain consequences of some types of medical care make it difficult for patients to judge what care is in their best interest. The gap in knowledge between patients and providers leaves patients vulnerable to inappropriate care or care they would not choose for themselves if they were well informed. The external benefits and costs of some types of medical care (e.g., immunization to prevent infectious disease, which benefits populations as well as individuals), as well as investments in education, housing, and the environment, may not be appropriately valued by private markets, leading to inefficient allocation. Documenting the presence of such adverse conditions is another method used by analysts to suggest government interventions designed to improve efficiency.

It should be noted that applying the competitive economic model to enhance efficiency in healthcare assumes that maximizing satisfaction of consumer preferences is an appropriate policy goal. This is a value judgment that should be clearly stated when applying the model. An alternative model that emphasizes maximizing the population's health status, or meeting healthcare needs, is a substitute for consumer satisfaction in efficiency analysis. Both models are discussed in Chapter 4. Criteria for judging the determinants of allocative efficiency in the needs-based model are not as well developed as those in the competitive economic model.

The three primary policy strategies for enhancing equity, lodged in the distributive, social, and deliberative justice paradigms, were identified as (1) enhancing access to medical care, (2) reducing health disparities, and (3) ensuring affected parties' participation in policy and program design. Empirical analyses of the relative importance of various factors presumed to influence whether or not people receive care, experience social and behavioral risk factors and poor health, and participate in the health policy process point to possible areas of intervention for health policy to enhance equity. Potential access indicators discussed earlier may be used to identify potential solutions to an equity problem by examining the correlation of these indicators with realized access measures—utilization and satisfaction. The factors that most directly influence access to needed services, such as insurance coverage

or a regular source of medical care, then become the focus of the development of programs and services to enhance access.

The access model is typically used to examine equitable access to healthcare. Equitable access is defined as when demographic and need variables account for most of the variance in use. Inequitable access occurs when social characteristics (e.g., race/ethnicity) and enabling factors (e.g., income, insurance coverage) determine who gets healthcare. Effective access is defined when use improves health status. Efficient access is defined as the relative improvement in health status compared to healthcare costs (Andersen 1995; Andersen and Aday 1978).

Like efficiency analysis, equity research is ultimately concerned with those medical and nonmedical services that are effective in the clinical or population sense—that is, in improving health and healthcare access and reducing health and healthcare disparities. Equity criteria incorporating distributive justice norms regarding the distribution of medical care can help to identify equity solutions from this perspective (Gelberg, Andersen, and Leake 2000). Criteria incorporating norms regarding the health and health risks related to medical and nonmedical (e.g., social-structural, cultural, environmental) factors embody the population effectiveness perspective. The extent to which norms of democratic participation are involved in policy formulation or implementation is a criterion of equity based on the deliberative justice paradigm.

Table 8.4 provides a summary list of the solution analyses that can be drawn from the three perspectives of health services research.

Evaluating and Modifying Past Actions

With a shift in focus from ex ante to retrospective analysis, the objectives become (1) to determine the degree to which a new policy or program was implemented as intended and (2) to measure its anticipated and unanticipated effects. When monitoring implementation, the analyst asks if certain standards are being followed or if the policy or program reflects the intended use of resources. Specific indicators used include measures of inputs (e.g., personnel, facilities, equipment, supplies), processes (e.g., administrative, organizational, clinical, behavioral, political, attitudinal), and outputs (i.e., the goods and services provided). In measuring effects, the analyst attempts to determine whether a policy has brought about change, for example, in the behavior, attitudes, or health status of targeted individuals, groups, organizations, or

Table 8.4 Solution Analyses in Terms of Effectiveness, Efficiency, and Equity

Dimensions	*Analyses*
Effectiveness	
Population perspective	Apply the structure-process-outcomes framework to identify policies associated with improvements in health.
Clinical perspective	Apply the structure-process-outcomes framework to identify policies associated with improvements in healthcare services and systems.
Efficiency	
Allocative efficiency	Conduct cost-benefit analysis of proposed medical and nonmedical services, organizational arrangements, and financing mechanisms.
Production efficiency	Conduct cost-effectiveness analysis of proposed services, organizational arrangements, and financing mechanisms.
Equity	
Distributive justice	Apply equity-of-access model to evaluate impact of proposed healthcare services and systems.
Social justice	Analyze impact on disparities in health and health risks.
Deliberative justice	Analyze impact on participation of affected parties in policy development.

communities. Approaches in determining effects range from social-systems accounting, in which the analyst monitors overall changes in health or other social status indicators (e.g., infant mortality rates) over time and attempts to relate the changes logically to past policies (prenatal care access interventions), to experimental and quasi-experimental evaluations of specific policies, and to programs to isolate their effects from other factors (overall downward trends in infant mortality) (Dunn 2003).

This stage involves collecting and analyzing performance information to help decide to continue, modify, or terminate existing policies.

In some cases, this stage leads to a redefinition of the original problem. To assess performance, the consequences of a policy are evaluated normatively in light of designated objectives and criteria. The menu of analytic tasks and methods used in the ex ante evaluation of alternatives (see Table 8.4) is also relevant to this stage, but the focus is on evaluating actual rather than potential consequences. To evaluate performance, the analyst must define policy objectives, transform them into specific criteria that can be used in evaluation, and evaluate the consequences of a policy or program in terms of the criteria.

One of the major contributions made by health services researchers is informing policymakers about what does and does not work. The health system performance perspective of much of this research provides evidence that analysts can use to show the effects of past policies. Effectiveness research supplies a conceptual framework, methods, and evidence to describe and evaluate the technical effectiveness of existing health policies. Research linking structural factors—the quantity and efficacy of medical and nonmedical inputs—to health outcomes can be conducted to assess the impact of a particular intervention on desired policy outcomes. In the same way, studies on the effects of process—the quantity, quality, and appropriateness of services delivered or of investments made—on health outcomes guide evaluations of the success of actions to change the process of medical and social service delivery. Analysts may use this information in evaluating the consequences (e.g., lessening health disparities) of any given solution (investments in public housing) that can then be related to desired policy objectives (to improve the health of the population).

The concepts, definitions, and methods that health economists have developed to examine the allocative and production efficiency of healthcare serve as important resources for describing and assessing the consequences of policy actions. There are numerous studies of production efficiency, as outlined in Chapters 4 and 5, to guide evaluations of the organization and production of health services. The RAND Health Insurance Experiment, discussed in prior chapters, is a good example of this kind of research carried out with a rigorous, large-scale, experimental design. Findings from these studies generally document the costs and effects of alternative insurance strategies that range from first-dollar coverage to catastrophic plans. Estimates were made of the excess spending that occurred under first-dollar coverage given the low marginal value of the added medical care services consumed. Studies of the

efficiency of prepaid group practice are another important example. Many well-conducted cost-effectiveness studies have provided useful information on the relative efficiency of alternative services and technologies (see Chapter 5).

Both analytic research and evaluative research are relevant to the task of describing and assessing the equity consequences of health policy and programs. Analytic research suggests causes of equity problems that are likely to be altered by private or government interventions. Empirical measurement of the effects of specific factors (e.g., social support available to high-risk mothers) form the primary basis for evaluating the equity consequences (prenatal care utilization rates) of alternative service delivery options (case management services). Evaluative research on access (reviewed in Chapter 7) is useful in actually informing policy analysts of the success of specific programs or policies (e.g., Healthy Cities and Community-Oriented Primary Care, aimed at community health) in enhancing procedural and distributive equity.

LIMITATIONS OF HEALTH SERVICES RESEARCH IN POLICY ANALYSIS

To the extent that the conceptual theories and empirical studies from effectiveness, efficiency, and equity research are neither well developed nor clear, the research is limited as a source of information and argument in policy analysis. The prior sections of this chapter reviewed the potential contributions of health services research to policy analysis, whereas the discussion that follows highlights some of the limitations.

Effectiveness

No policy or professional consensus exists on how to apply the population perspective in defining effectiveness in healthcare delivery. The clinical perspective leaves out important factors that contribute to the health of the population. The population perspective requires that health policy research address the impact on health of factors beyond the medical care system (e.g., housing and jobs) for which information is more limited. As indicated in Chapter 2, the clinical perspective has become more prominent of late in the United States in giving emphasis to research evaluating the outcomes of specific clinical practices. Related to the debate over perspectives is the question of defining policy objectives in health. From the population perspective, community

health indicators are important. From the clinical perspective, individual patient health status is emphasized.

The imprecision of measures of effective medical practice is a critical weakness in applying effectiveness analysis at both the clinical and population levels. Only rough estimates can be made of the direction and strength of the relationships between structure and outcomes and between processes and outcomes of care. Studies of variations in practice indicate an extremely wide range of acceptable practice patterns (Kane 1997). However, the efforts by the federal government to invest in this type of research notwithstanding, it is difficult to determine precisely how much of the variation can be attributed to the provision of ineffective services.

Another limitation is that the extensive research on the medical and nonmedical determinants of health has not often been well linked across the levels of analysis defined in Chapter 2. Approaches that appear to be beneficial at one level may not be effective at the next level of analysis. For example, improving the quality of care of individual patients may not be effective at the community level because of the limited potency of medical care interventions. In deciding how to invest societal resources in improving the health of the population, policymakers must take into account not only what works for the individual patient but how these resources are best used for the population as a whole. Without information across all levels of analysis, ineffective decisions can and will be made.

Efficiency

Efficiency research provides useful but limited information on the optimal allocation of resources and on optimal production methods. We are only beginning to understand the effects of healthcare and other important medical and nonmedical investments on health and well-being. Without this information, the social value of resource-allocation decisions cannot be determined with precision. The relative efficiency of different organizational models and resource mixes for producing cost-effective medical care are not clear, despite the extensive research in some areas—for example, comparing managed care versus consumer choice models of financing or comparing the costs and effectiveness of hospital inpatient versus outpatient provision of various procedures and services (Altman and Levitt 2002). A conceptual

difficulty in applying allocative efficiency criteria to the evaluation of policy alternatives is that the distributional consequences of alternatives (i.e., some win and some lose as a result of each alternative) cannot be assessed. Pareto optimum criteria, by which the beneficiaries compensate the payers, can be used (Chapter 4), but this may not be ethically acceptable if there are no mechanisms for ensuring that winners compensate losers.

Another important limitation is that different methods are applied by researchers doing efficiency research (e.g., cost-effectiveness, cost-benefit, cost-utility, and cost-of-illness), thereby limiting the ability to make comparisons across projects. Guidelines have been developed, however, for researchers to follow (Gold et al. 1996). There are also limitations associated with macrointernational comparisons of efficiency—the lack of standard definitions of health services, differences in national accounting practices, and difficulties in adjusting for currency differences.

Equity

The focus of equity research would be enhanced if there were greater clarity and consensus on equity objectives. Chapter 6 proposes multiple paradigms that provide the basis for alternative principles, criteria, and indicators of equity. Some of the frameworks potentially conflict, making it difficult to follow this perspective in policy analysis. A conceptual framework of equity (Figure 6.1) has been presented that integrates these criteria, considering procedural and substantive equity and their interrelationship. The causal relationships between procedural and substantive indicators of equity have not been thoroughly and uniformly documented. The challenge to health services research and policy analysis is to more accurately and fully document the contribution of medical and nonmedical factors to reducing healthcare and health inequalities—the ultimate criterion of distributive and social equity—across social and economic groups.

SUMMARY AND CONCLUSIONS

This chapter discusses the objectives of policy analysis that reflect different views of the policy-development process, standards of policy analysis, and the usefulness of health services research in performing the tasks of policy analysis. The rational model is described as a guide that policy analysts and health services researchers generally use to iden-

tify the types of research most relevant to health policy questions. This model identifies the sequential stages of policy development, the relevant policy analysis that is most appropriate, and the information and types of research needed to assist decision making at each stage. The objectives of the rational model of policy analysis, however, have been augmented by adding an awareness of, and attempting to take into account, the critiques of this model offered by the political and radical models. Health services research that meets the standards of scientific integrity and is concerned broadly with both medical and nonmedical determinants of health may be used in the policy-analytic tasks of defining values, clarifying problems, identifying and evaluating policy options, and evaluating and modifying past actions. Offering specific criteria and analytic frameworks for effectiveness, efficiency, and equity, the health services research literature provides a rich resource for policy development and assessment.

NOTE

1. Our discussion focuses on public policy development. However, the concepts, terms, and methods presented are generally applicable to what is referred to as policy development or strategic planning in the private sector as well.

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Chapter 9

Applying Health Services Research to Policy Analysis

CHAPTER HIGHLIGHTS

1. Relatively low mammography screening rates in older women have persisted despite Medicare coverage of this preventive procedure, stimulating a debate over whether the federal government should be doing more to encourage screening in this population.
2. In this chapter, normative criteria, measures, methods, and evidence based on the effectiveness, efficiency, and equity perspectives are applied in evaluating the policy importance of this problem to demonstrate the application of health services research to health policy analysis.
3. This multiple-perspective policy analysis supports the concern over low rates of mammography screening in older women and points to the need for cost-effective strategies to increase screening rates.

OVERVIEW

This chapter concludes the book by illustrating how the health services research perspectives of effectiveness, efficiency, and equity can be applied in policy analysis. A case study is presented that examines a policy question related to mammography screening of older women in the U.S. Medicare program. We chose this case because of its focus on an important population-based, preventive health service, the central role that the Medicare program plays in U.S. healthcare policy, and the availability of recent health services research evidence regarding various aspects of this question. The case illustrates concepts, criteria, and methods that have general relevancy in the analysis of health policy

questions at the national, state, or local level of government (MacRae and Whittington 1997).

We begin with background information on the problem and pose a specific policy question. We then define normative criteria for addressing the question based on the assumption that effectiveness, efficiency, and equity are important goals for healthcare policy. Relevant measures are defined to operationalize the criteria, and related data are reviewed to assess the consequences of policy alternatives in terms of the valued criteria. A summary analysis justifies the recommendation in terms of achieving specified goals reflecting the three perspectives and considering possible trade-offs among them.

BREAST CANCER SCREENING IN OLDER WOMEN: ARE LOWER RATES A PROBLEM?

Breast cancer is the most common type of cancer among U.S. women and the second leading cause of cancer-related death. In 2001, an estimated 192,000 new cases were diagnosed and 40,000 women died of this disease (ACS 2002). The incidence and mortality of this disease increases with old age (NCI 2002). By age 30, the odds of a woman having breast cancer are one out of 2,500; by age 50, the odds increase to one out of 50; and by age 70, they are one out of 14. In 2001, the 13 percent of women age 65 or older accounted for two-thirds of new breast cancer cases and 52 percent of disease-related deaths.

Mammography screening for breast cancer saves lives by identifying disease in an earlier stage (Moss 1996). There is an inverse relationship between the stage of breast cancer diagnosis and survival. Therefore, women who are screened have a better chance for early breast cancer detection and, in turn, increased survival than do women who do not get screened. Several randomized clinical trials conducted during the 1970s and 1980s in women age 40 to 69 have reported that regular screening results in earlier diagnosis and reduced breast cancer mortality. The mortality benefit in the clinical trials ranges from 14 percent to 32 percent beginning about four years after widespread screening (Feig 2002). Computer simulations that have controlled for some of the design flaws in the trials indicate that the beneficial reduction in mortality could be even greater than that indicated by the trial data (Feig 2002).

Evidence of the benefits of screening in older women (age 70 or older) is not as conclusive as it is for younger women simply because

this age group has not been included in randomized trials. Professional guidelines for mammography screening in older women are also somewhat inconsistent (Table 9.1). The American Medical Association, National Cancer Institute, U.S. Preventive Services Task Force (USPSTF), and American Geriatrics Society (AGS) recommend mammography and clinical examination for women 65 or older every one to two years. The American Cancer Society's recommendation is once a year. All but the AGS have no upper age limit. The AGS guidelines change to every two to three years for women age 75 or older with life expectancy of at least four years. The ACS guidelines include language advising older women that screening decisions should be individualized by considering the potential benefits and risks in the context of current health status and estimated life expectancy. The USPSTF guidelines state that the precise age at which to discontinue screening mammography is uncertain and that older women with comorbid conditions limiting their life expectancy are unlikely to benefit from screening.

The U.S. Medicare program encourages mammography screening by providing annual financial coverage for most women age 65 and older and waiving copayment provisions that apply to other outpatient procedures. Despite Medicare coverage, older women use mammography services at a lower rate than other women in the general population. In a study based on Medicare claims data from the mid-1990s, 59 percent of women 75 years of age or older had been screened in the prior two years versus 67 percent of women 69 to 74 years of age (Randolph et al. 2002). According to the mammography indicator reported by the Centers for Medicare & Medicaid Services (2002), the national biennial mammography rate among non-HMO-enrolled women age 65 years and older was only 50.8 percent in 2000–2001, far short of the 70 percent screening rate goal set by Healthy People 2010.

Lower mammography screening rates for older women may or may not be considered a policy issue. It deserves attention if it can be shown that such rates lead to unnecessary loss of life, higher medical costs, and anxiety that could be avoided through more screening and earlier diagnosis. Older women are good candidates for breast conserving surgery, which is a local-anesthesia office procedure, and mastectomy has low operative mortality (2 percent) (Mandelblatt 2003). Tamoxifen is tolerated by most older women, but chemotherapy may be a problem because of its potential adverse effects for women with cardiac and/or renal function comorbidities. If the effectiveness of screening and related

Table 9.1 Recommended Mammography Screening Schedules for Older Women

Organization	*Age Groups*				
	65–69	**70–74**	**75–79**	**80–84**	**85+**
	Mammography Screening Guidelines				
American Cancer Society	Annual with no upper age limit but with consideration of current health status and life expectancy				
American Medical Association	Every 1–2 years with no upper age limit				
National Cancer Institute	Every 1–2 years with no upper age limit				
U.S. Preventive Services Task Force	Every 1–2 years with no upper age limit but with consideration that comorbid conditions may make screening less beneficial				
American Geriatrics Society	Every 1–2 years		Every 2–3 years for women with life expectancy of at least 4 years		

Note: These screening schedules are for women who are at "average" risk. The evidence of the effectiveness of mammography screening from clinical trials is scanty for women 74 and older.

Sources: ACS (2003); AGS (1999); AMA (2001); NCI (2002); USPSTF (2002).

treatment in older women is not as great as in younger women, little or no benefit may be gained from efforts to enhance screening. A number of factors, such as shorter life expectancy, lower yield of screening in terms of earlier diagnosis, or low tolerance for cancer therapy, may affect the potential effectiveness of breast cancer screening and treatment. If the benefits of screening in older women are not as great as in younger women, the lower use rates suggest a rational response to the possible adverse consequences of screening that can include anxiety and costs from false positives. Nonetheless, equity questions may arise if screening rates vary significantly by age and/or racial/ethnic groups.

To assist policymakers facing the question of whether more should be done to encourage screening in the Medicare population, we exam-

ine the importance of mammography screening in older women from the perspectives of effectiveness, efficiency, and equity. We define effectiveness, efficiency, and equity criteria for assessment, review available data related to each criterion, and develop an evidence-based argument that lower mammography screening in older women should or should not be viewed as a policy problem.

CRITERIA FOR VALUING MAMMOGRAPHY SCREENING

The general types of information used by policy analysts to determine whether lower screening rates in the Medicare population constitute a policy problem were discussed in Chapter 8 and summarized in Table 8.1. Such information is needed on the scope, severity, causes, and importance of a problem. Relevant types of research include conceptual analyses and descriptive studies of problem conditions and their causes.

The general criteria of effectiveness, efficiency, and equity to be used to guide policy analysis in this stage and others were described throughout the earlier chapters, elaborated on in Chapter 8, and summarized in Table 8.2 for two levels of analysis: at the population level, where the focus is on evaluating policies that affect the various determinants of the health of the entire population (i.e., the community), and at the clinical level, where the focus is on policies targeting healthcare users (i.e., system enrollees, institutional users, or individual patients). In this case, we are concerned with analyzing the benefits of the use of mammography screening services. Therefore, the clinical-level criteria are applied.

Effectiveness

The criteria for assessing the effectiveness of healthcare programs and policies were delineated in Chapter 3 (Table 3.2) and are briefly revisited here. Two of the criteria relate to the population perspective, as defined in Chapter 2: (1) policy design guided by community or population needs assessment and (2) plan inclusion of options contributing to comprehensiveness through integration of services across the entire continuum of health services (see Figure 1.1). Other criteria relate to the clinical perspective derived from Shortell et al. (1995): the formulation of practice guidelines and performance monitoring of process and outcome indicators for selected conditions to ensure clinical accountability. The effectiveness analyses for mammography screening focuses on the latter two criteria—to enhance guidelines development and

ensure clinical accountability—especially among older, Medicare-eligible women.

A variety of patient factors, such as age, health status, and clinical disposition, may be related to the effectiveness of mammography screening. The benefit of mammography screening is expected to be lower in older women because they have lower life expectancy than their younger counterparts and may have multiple chronic diseases, thus making them more likely to die of another disease. Critics of large-scale screening programs in elderly women have called for judicious physician clinical judgment to carefully weigh the relative benefit and effectiveness for each individual patient (Gotzsche and Olsen 2000). As a counterpoint, although mammography screening may not reduce breast cancer mortality rates, there is evidence that many older women benefit as much as or more than younger women from mammography screening (Lee 2002). For example, screening in elderly women results in fewer false positive results, thus making this age group of women better candidates for screening (Costanza 1992).

An examination of the weight of the evidence on breast cancer screening effectiveness in the medical literature yields mixed results, adding to the ongoing debate on whether breast cancer screening is really effective in elderly populations. As reported earlier, clinical trials (Andersson and Janzon 1997) have shown that mammography screening significantly reduces breast cancer mortality rates in women age 50 to 69 years, with benefits possibly extending up to the age of 74 years. An observational study (Randolph et al. 2002) found that older women (75 years of age or older) had larger tumors at diagnosis and were less likely to have undergone screening mammography than younger women (69 to 74 years of age). The association between increased mammography use, smaller tumor size, and stage at diagnosis was significantly greater in older women than in younger women. Other literature clearly points to beneficial outcomes associated with mammography screening in women age 65 years and older, both in terms of early detection (McCarthy et al. 2000; Smith-Bindman et al. 2000) and increased life expectancy (Van Dijck et al. 1996). These findings thus appear to support the effectiveness of mammography screening in older women.

Mandelblatt et al. (1992) found that life expectancy increases due to screening mammograms were about two days on average for women age 65 to 74 years and one day for women age 75 to 85 years. For women who actually have breast cancer, appropriate screening and treatment

may result in a gain of a number of years of life. In a study comparing the effectiveness of mammography screening in women with high bone-mineral density (shown to increase the risk of breast cancer), continuing mammography screening after age 69 results in a small gain in life expectancy (about two days) in those with higher bone-mineral density (Kerlikowske et al. 1999). This finding suggests that targeted screening may be a particularly effective strategy in elderly women.

A conceptual framework that can be used to assess and evaluate the clinical effectiveness of mammography screening using these concepts draws on that proposed by Donabedian (1966, 2003) and Kane (1997) to evaluate quality of care and outcomes associated with medical care. The conceptual framework to analyze the effectiveness of mammography screening in elderly women can be divided into the three classic components of structure, process, and outcomes. Structure refers in this case to elements of medical care associated with the receipt of mammography screening, such as availability of insurance coverage and access to facilities for mammography screening. Process refers to the intervention being effectively utilized. In this case, the intervention being examined is mammography screening, and process includes the rates of use of the test and the effectiveness of the test in correctly diagnosing invasive cancer in older women. Doctor-patient communication is importantly related to whether women seek screening (Mandelblatt et al. 2003). Finally, both structure and process results in outcomes. In this case, the intermediate outcome is the early detection of breast cancer, and the final outcome is improved life expectancy as a result of early detection. This approach to determining a policy problem in terms of mammography screening effectiveness is illustrated in Figure 9.1.

All components of medical care need to be constantly evaluated in terms of the three components of effectiveness, measuring, for example, the intensity of the intervention (in terms of rates of screening in different types of populations), improvement in quality (test sensitivity and specificity), and outcomes (earlier diagnosis of breast cancer and reduced mortality). Monitoring and benchmarking these measures for mammography screening in older women against extant standards of process and outcome effectiveness (such as Healthy People 2010 goals and objectives) may be used to determine policy problems in this area. One of the crucial aspects of evaluation of the effectiveness of any medical care policy is the comparison against established guidelines or

benchmarks to answer the questions, Where are we, and where do we want to be?

We therefore conduct an effectiveness benchmarking analysis by comparing screening rates and outcomes in each five-year category of age in women 65 or older against selected benchmarks for where we want to be under maximum possible effectiveness estimates reported in the literature for this population.

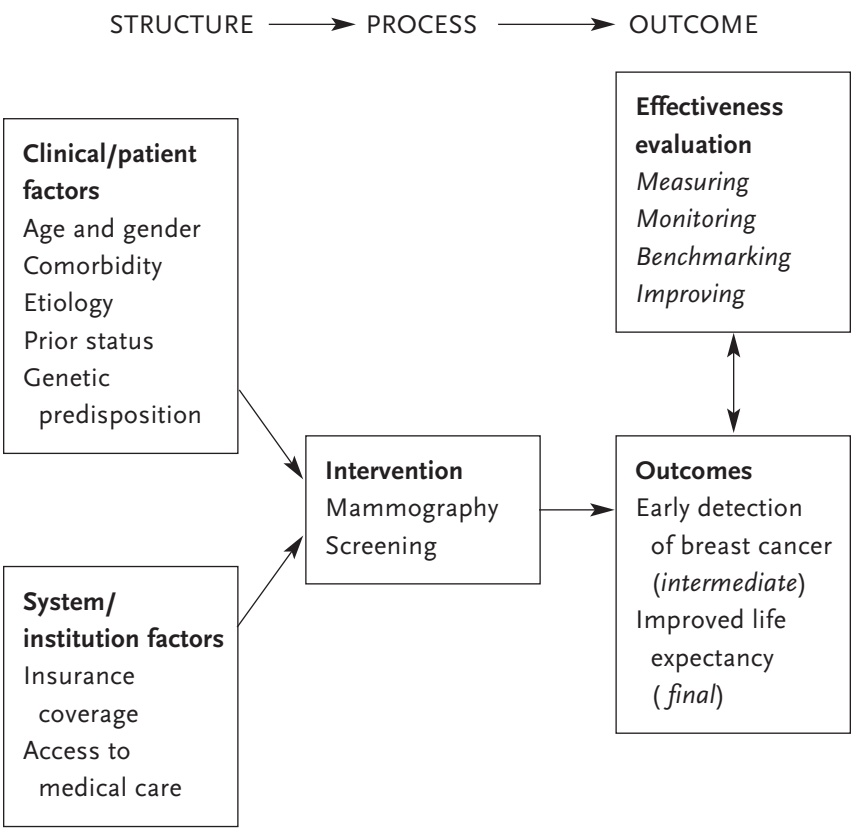
Efficiency

Assessing efficiency of health-related screening services at the clinical level involves the comparative assessment of their relative cost and effectiveness (Table 8.2). A cost-effectiveness ratio is calculated, where the denominator reflects the incremental gain in health and the numerator reflects the additional cost of screening. Economic assessment models differ in terms of the measures used to express the benefits of any health-related screening or treatment service (Drummond et al. 1997; Gold et al. 1996). All require that appropriate cost and saving of the service be taken into account. For a screening program, cost analyses usually consider the cost of the screening plus the cost of follow-up diagnostic services less cost-savings from earlier treatment. Effects are usually measured in terms of life years saved. The use of quality-adjusted life years or healthy-year equivalents is recommended but, because of lack of data, not often applied.

Application of the efficiency perspective involves conducting a cost-effectiveness analysis of mammography screening in which the health benefit of screening, expressed as the increase in life years gained due to early diagnosis, is compared to the cost of screening. The cost-effectiveness ratio is used to evaluate screening against the no-screening option in terms of the incremental cost of screening, follow-up, and treatment divided by the number of life years gained. Different amounts or rates of screening may also be compared. A number of possible perspectives may be taken in determining costs: the payer perspective, in which the payment for screening, follow-up, and treatment services is considered for different screening groups; the patient perspective, which focuses on patient out-of-pocket, time, and transportation costs; or the societal perspective, which attempts to capture all costs, no matter who bears the burden.

Application of the cost-effectiveness framework to evaluate screening can be facilitated by modeling the screening strategies and possi-

Figure 9.1 Framework for Analysis of Effectiveness



Sources: Adapted from Donabedian (2003, 46–47) and Kane (1997, Figure 1-1, 13).

ble subsequent events and outcomes as a simulation or a decision tree (Mandelblatt et al. 2003). In this case, we adapt a simple decision-tree model (Figure 9.2) of the problem drawn from the mammography literature (Mandelblatt et al. 1992). The perspective of the model is the average woman faced with a point-in-time choice between screening and not screening.

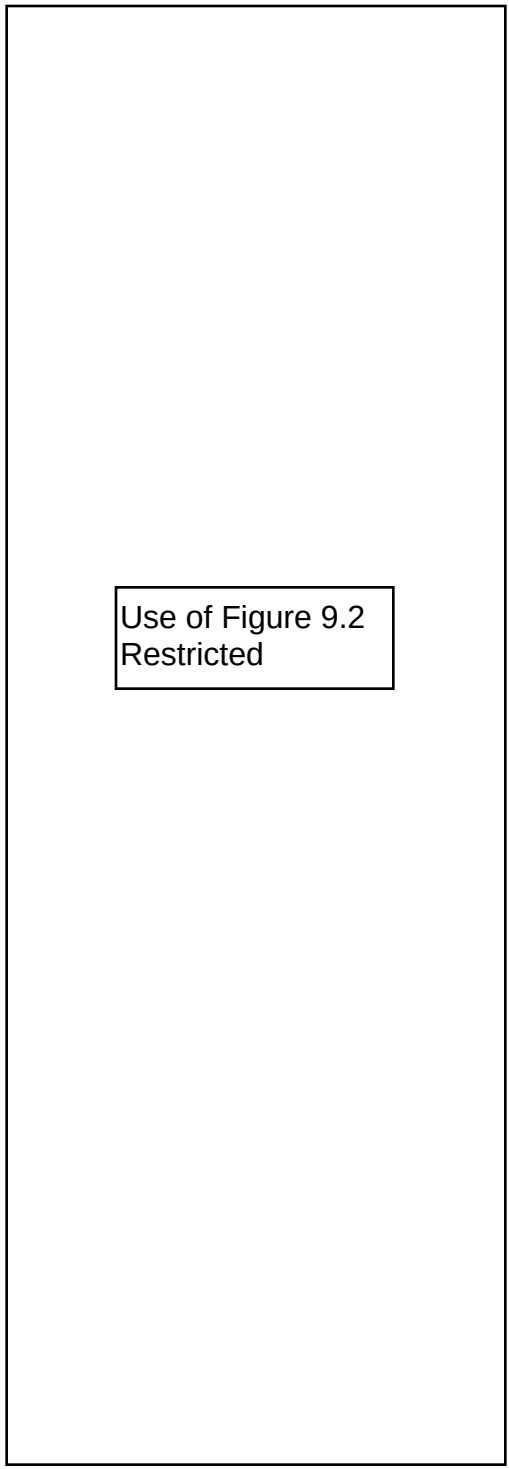
Starting from the decision node on the left of Figure 9.2 (indicated by a square box), the branches of the decision tree indicate the alternatives, and the subsequent possible events of screen results, follow-up results, presence or absence of cancer, stage at diagnosis, and survival

expressed in life years. The purpose of using the tree is to estimate the cost-effectiveness of screening in terms of the costs per life years saved for average asymptomatic women who are screened compared to those who are not. This is estimated using the tree by calculating the expected value of life years gained from screening and no screening, then dividing the difference by the average cost of screening and follow-up to derive the net cost of screening per life year gained.

The upper branch of the decision node leads to the "screen" option and then to a chance node (indicated by a circle); at that node, the possibilities are "test positive" and "test negative," with the respective probabilities (TP, TN = 1 – TP). The proportion of those screened who will have positive or negative results depends on the characteristics of the mammography test and the proportion of the population with breast cancer. We can best express the mammography test characteristics in a 2×2 table (Table 9.2) that reflects the test result probabilities for women with and without cancer. The proportion TP in the screening population who are positive include true positives, who are women with cancer identified through screening (incidence * sensitivity); and false positives, who are women with cancer who mistakenly receive a positive test (no disease * (1 – specificity)). The proportion TN in the screening population who are negative are composed of true negatives—women without cancer identified through screening (no disease * specificity)—and false negatives—women with cancer who mistakenly receive a negative test (incidence * (1 – sensitivity)).

The decision model assumes that all women with abnormal screens will have repeat mammography and/or ultrasound or biopsy follow-up services. The follow-up results for these screens are assumed to be negative for people with no cancer (FP/TP + FP) and positive for cancer in diagnosed cases (TP/TP + FP) ("early diagnosis"). Women with negative results at screening may either be disease free (TN/TN + FN) or have cancer (FN/TN + FN). For those with invasive cancer, it is assumed that a correct diagnosis will be made via clinical examination, diagnostic mammography, and biopsy about six months later ("interval diagnosis") than screen-detected cases.

Unscreened women may or may not have cancer. For women with cancer, we assume the disease will progress and be diagnosed about one year later ("late diagnosis") than screen-diagnosed cases through clinical examination, diagnostic mammography, and biopsy. The model further assumes that all women diagnosed with cancer will receive the same


Use of Figure 9.2
Restricted

treatment based on stage at diagnosis. Women with local and regional cancer will have surgery with a small risk of operative death (2 percent) (Mandelblatt et al. 1992). Women with distant cancer will receive nonsurgical treatment. The outcomes of women with cancer are measured by years of survival and vary based on age and stage at diagnosis.

In summary, the criteria for determining whether low screening rates in older women should be considered a policy problem involves estimating a decision-tree model. The model, which reflects the normative value of clinical efficiency, is used to determine if the additional years of expected survival for a screened woman of a given age is large enough to justify the additional costs of screening. An average cost-effectiveness (CE) ratio for all women is calculated to compare screening to no screening:

Average CE ratio = (screening and follow-up cost)/ life years gained
Screening cost = cost mammograms + cost follow-up services
Life years gained = expected years of life1 – expected years of life0, where 1 represents screening and 0 represents no screening.

Equity

As discussed in Chapter 7 and summarized in Table 7.1, a number of possible criteria may be applied in the equity evaluation of health-related screening and treatment services based on distributive, social, and deliberative justice principles. The similar-treatment norm and related evidence regarding the need for screening based on subgroup variations in breast cancer incidence and mortality are the primary criteria of equity applied in assessing mammography screening among older women.

The framework for the analysis of the distribution of the benefits and costs of screening in terms of similar treatment and/or need is based on the Aday and Andersen model of healthcare utilization (Aday and Andersen 1981; Aday, Andersen, and Fleming 1980; Andersen 1995) (Figure 9.3). The model posits that the use of health services is a function of the characteristics of the healthcare delivery system and the population at risk. The availability, organization, and financing features of the system, as well as the predisposing, enabling, and need characteristics of the population to be served, all contribute to accounting for variations in their use of and satisfaction with care.

Table 9.2 Screening Test Probabilities

	Disease Present	*Disease Absent*	*Total*
Test+	**TP** (incidence * sensitivity)	**FP** (no disease * (1 – specificity))	**TP + FP**
Test–	**FN** (incidence * (1 – sensitivity))	**TN** (no disease * specificity)	**TN + FN**

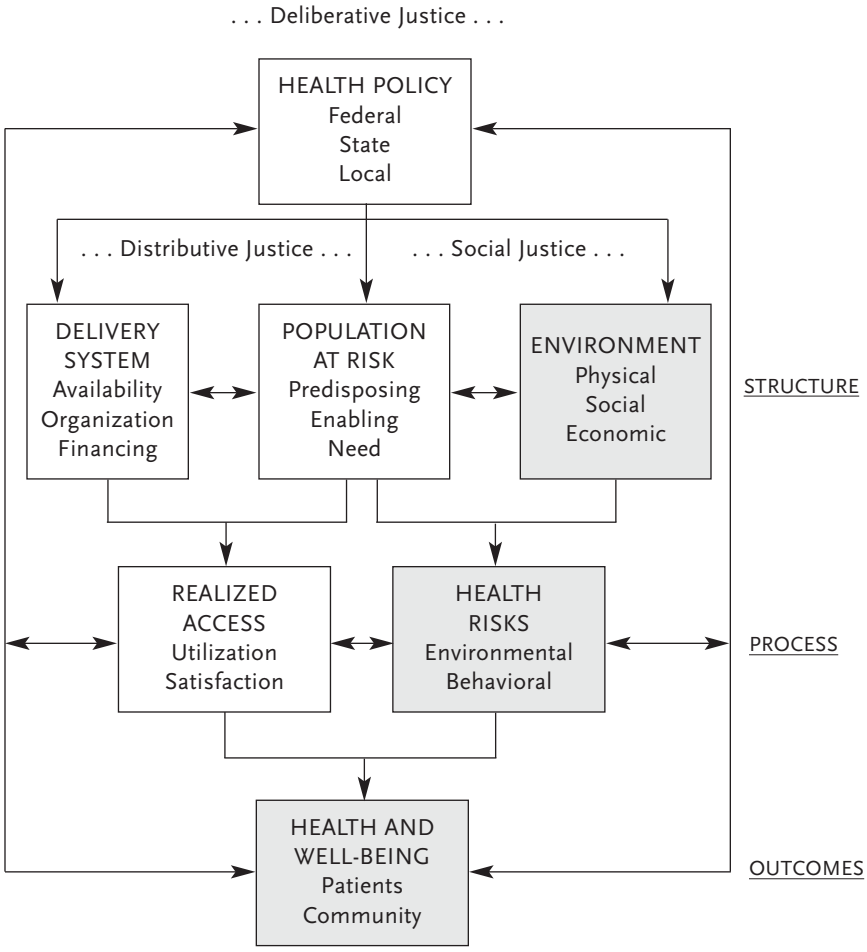
Predisposing characteristics of the population include variables such as age, gender, race/ethnicity, marital status, education, occupation, and health beliefs. Enabling characteristics include the availability of health services and the means to get to them, such as health insurance coverage, transportation, and having a regular source of care. Need factors are related to the severity of an illness or general state of health and may be either clinically defined or based on an individual's perception.

The final stage of the original access model was health service use, but in the current version (Figure 9.3), realized access, defined as health service use and related satisfaction with care, is linked to ultimate health outcomes. Environmental factors (e.g., physical, social, and economic components) are also acknowledged as contributing to both environmental and behavioral health risks and the associated health and well-being of patients and the community. In addition, feedback loops demonstrate how resulting access and health outcomes can, in turn, inform and influence the subsequent development of health policy.

Based on the model and related principles of similar treatment and need, equitable access is defined when demographic and need variables account for most of the variance in use. Inequitable access occurs when system factors and social characteristics (e.g., race/ethnicity) and enabling factors (e.g., income, insurance coverage) determine who gets healthcare.

Therefore, the major criteria that are applied in evaluating the equity of lower screening rates among older women are the similar-treatment norm (minimize disparities in mammography screening rates across subgroups of women) and need-related norm (minimize

Figure 9.3 Framework for Analysis of Equity



disparities in late-stage breast cancer rates and breast cancer death rates across subgroups of women). Evidence is presented regarding variations in breast cancer incidence, stage of cancer, death rates, and the utilization of mammography screening services by selected subgroups of women—notably by age and race. Stratification by age and race helps to identify those groups who are most at risk of not receiving the benefits of screening.

EVALUATION OF MAMMOGRAPHY SCREENING IN OLDER WOMEN

Effectiveness

The summary measures of effectiveness benchmarking are provided as indices in Table 9.3. The benchmarks for each measure were selected to reflect the ideal condition so that the effectiveness index provides an answer to the question, Where are we in terms of effectiveness relative to where we want to be? The definition and source of the benchmark for each process and outcome measure are provided in the footnotes of Table 9.3. The effectiveness indices for the different age groups are computed by dividing the rates for each age group by the benchmark if a higher value of the benchmark indicates a more positive outcome (a) and dividing the benchmarks by the actual rates in each group if a lower value of the benchmark indicates a more positive outcome (b). A value of 1.00 on the effectiveness index means that the rate is the same as the benchmark. A value of less than 1.00 means that the rate compares unfavorably, and a value greater than 1.00 means that it compares favorably with the benchmark.

With regards to the process measure of mammography use, the proportion of women age 65 or older receiving a mammogram in the preceding two years is 0.73 of the Healthy People 2010 goal for the entire U.S. population of women in all age groups (including those under 65). The gap increases for older age groups with the effectiveness index dropping to 0.28 for women age 85+.

The process measure of test performance indicates a different result, as expected, because this measure depends on the biological characteristics of the cancer. Compared to the highest reported possible sensitivity and specificity for these tests (in women with almost entirely fatty breast density and no fibrous tissue or dense regions), the sensitivity effectiveness indices range from 0.83 to 0.98 across the age groups of older women. The specificity rates are even closer to the benchmark, ranging from 0.96 to 0.98 across the age groups.

The proportion of breast cancers diagnosed at a late stage is examined to assess where we are regarding stage at diagnosis. Compared to the benchmark percentage based on 100 percent screening, the effectiveness indices are between 0.45 and 0.57. Since mammography screening has been shown to be strongly associated with early diagnosis of

breast cancer in older age groups of women (McCarthy et al. 2000), these data raise concern about the low mammography screening rates in elderly women.

In the final effectiveness index, breast cancer survival rates are examined, comparing the increased life expectancy resulting from *actual* screening rates compared to a *100 percent* screening rate. There is a clear trend that screening effectiveness diminishes with age and associated lower rates of screening, as evidenced by the index declining from 0.63 for the 65 to 69 age group to a low of 0.19 in the age group of women 85 years or older.

The indices of effectiveness point to gaps in actual versus ideal screening rates and to gaps in gains we would expect to see in earlier stage at diagnosis and increased life expectancy with higher mammography screening in women age 65 or older. This is reinforced by the small gap between the sensitivity and specificity of the screening tests in these women and the ideal. The gap between where we are and where we want to be increases with age, supporting an argument from the effectiveness perspective that the low rate of screening in this population is a problem.

Efficiency

The probability values and outcomes for the decision tree are summarized in Table 9.4. Breast cancer incidence data are taken from the Surveillance, Epidemiology and End-Results program (SEER) for women of all races (NCI 2003). Based on data from the National Cancer Institute's Breast Cancer Surveillance Consortium (Carney et al. 2003), we assume an age-specific sensitivity (the proportion of positive tests expected among truly positive cases) ranging from 73.3 percent to 86.1 percent, and we assume an age-specific specificity (the proportion of negative tests among truly negative cases) ranging from 93.0 percent to 94.3 percent for screening using mammography. The follow-up biopsy is assumed to have a sensitivity and specificity of 100 percent.

The proportions of cases in each stage of breast cancer at diagnosis among screened and nonscreened women were based on estimates for each five-year age group derived by Mandelblatt (2003) based on National Cancer Institute SEER program data (NCI 2003). The proportions of cases in each stage of breast cancer among screened women ("early diagnosis") were assumed to fall within the following ranges:

Table 9.3 Effectiveness Indicators of Mammography Screening by Age for Women 65+

Indicators	*Age Groups*					*Total*	*Benchmarks*
	65–69	**70–74**	**75–79**	**80–84**	**85+**		
Process Measures							
% women 65+ with mammogram in preceding 2 years 2000–2001 (a)	63.4	60.2	52.5	39.2	19.3	50.8	70[1]
Effectiveness index	*0.91*	*0.86*	*0.75*	*0.56*	*0.28*	*0.73*	—
Sensitivity of screening test (a)	73.3	81.4	81.4	86.1	86.1	75.0	88.2[2]
Effectiveness index	*0.83*	*0.92*	*0.92*	*0.98*	*0.98*	*0.85*	—
Specificity of screening test (a)	93.0	94.1	94.1	94.3	94.3	92.3	96.5[2]
Effectiveness index	*0.96*	*0.97*	*0.97*	*0.98*	*0.98*	*0.96*	—
Outcome Measures							
% breast cancers diagnosed at late stage, 2000 (b)	26.8	23.8	26.2	24.0	30.5	25.9	13.6[3]
Effectiveness index	*0.51*	*0.57*	*0.52*	*0.57*	*0.45*	*0.53*	—
Life years saved based on screening, considering actual rates of screening for each age group (a)	2.85 days	1.86 days	1.31 days	0.69 days	0.26 days	varies with age group	varies with age group[4]
Effectiveness index	*0.63*	*0.60*	*0.53*	*0.38*	*0.19*	—	—

Note: The effectiveness indices for the different age groups are computed by dividing the rates for each age group by the figures reported in the benchmarks if higher value of benchmark indicates more positive outcome (a) and by dividing the figures reported in the benchmarks by the rates in each group if lower value of benchmark indicates a more positive outcome (b). A value of 1.00 on the effectiveness index means that the rate is the same as the benchmark. A value of less than 1.00 means that the rate compares unfavorably, and a value greater than 1.00 means that it compares favorably with the benchmark.

Sources:
[1] Age group estimates are from CMS (2002). Source tables for biennial mammography services paid by Medicare among non-HMO women 65+ based on HEDIS 2002 criteria: Age Groups 2001 HD02; Race 65+ 2000–01 HD02 (CMS 2002). The Healthy People 2010 objective (all age groups including under the age of 65 combined) is used as benchmark (Office of Disease Prevention and Health Promotion 2003).
[2] All estimates are from Carney et al. (2003). The rate in the total column is for all women age 40 years or older. The benchmarks are the highest sensitivity and specificity rates reported for screening (obtained in women with almost entirely fatty breast density).
[3] Age group estimates are computed from the SEER*Stat database: Incidence—SEER 12 Regs Public-Use, Nov 2002 Sub (1973–2000) (NCI 2003). Late stage includes "regional" and "distant" cancers. Unstaged cancers are excluded from the denominator. The figure in the Total column is for all women age 65 years or older. The benchmark is based on a reported relative risk of 0.57 for metastatic cancer for all women 65 or older (66–79) that were screened compared to those who were not in a study conducted by Smith-Bindman et al. (2000, Table 2, 115). The benchmark represents the percentage rate that could be achieved contingent on all women age 65 years or older getting screened. The calculation of the benchmark is as follows: (0.57 × best outcome in any age category [in this case, it is 23.8 percent in the 70–74 category that presented with late stage cancer in 2000]) = 13.6 percent.
[4] Age group estimates of life expectancy are obtained from the decision-tree model assuming the actual screening rate. The benchmarks for each age group (the increase in life expectancy due to screening) are obtained from the decision-tree model based on 100 percent screening for each age group: 4.49 days (65–69), 3.10 days (70–74), 2.48 days (75–79), 1.83 days (80–84), 1.35 days (85+).

- local, 74.0 percent to 78.0 percent
- regional, 14.5 percent to 21.0 percent
- distant, 4.5 percent to 7.5 percent

The proportions among nonscreened women ("late diagnosis") were assumed to fall within the following ranges:

- local, 43.9 percent to 52.5 percent
- regional, 36.8 percent to 39.7 percent
- distant, 10.7 percent to 16.4 percent

The stage distribution at diagnosis among women with breast cancer detected after a false negative mammographic result ("interval diagno-

sis") was assumed to be halfway between that of screened cases and those detected with no screening, as follows:

- local, 62.6 percent to 64.25 percent
- regional, 27.1 percent to 29.65 percent
- distant, 7.85 percent to 11.95 percent

We assumed no occurrence of biopsy-associated mortality, but the risk for perioperative death for women having mastectomy was estimated to be 2 percent (Mandelblatt et al. 1992).

Costs of Screening

The costs associated with screening were estimated using the same items associated with regular screening and follow-up services reported by Mandelblatt et al. (1992) (Table 9.5). The costs of screening and follow-up services are based on the national Medicare payment rates for 2003 (CMS 2003). Screening costs include the cost of routine screening mammography based on an extended office visit to discuss mammography plus the costs of the procedure.

Calculation of Incremental Cost-Effectiveness Ratio. The results of the decision analysis indicate that screening saves lives for all ages of elderly women, but the magnitude of the savings decreases with increasing age (Table 9.6). For women age 65 to 69, the life expectancy of a screened woman is 19.18 years, compared with 19.17 years for an unscreened woman, a net savings on average of 4.49 days. For a woman over 85, screening results in savings of 1.35 days. Although the average impact seems small, when multiplied by all women being screened it is a substantial savings that accrues to women with cancer detected through screening.

The marginal costs of screening, including the clinical breast examination, mammography, and diagnostic workup of true- and false-positive screening results, were $219. Thus, with 100 percent screening of 65- to 69-year-old women, the cost-effectiveness of the screening strategy, compared with a strategy of no screening, is $17,812 per year of life saved ($219/[4.49 days]). The cost per year of life saved increases with increasing age. For example, the cost-effectiveness increases from $25,785 for a 70- to 74-year-old woman to $59,211 for women age 85 years or older.

Table 9.4 Values Used in the Decision Tree To Assess Mammography Screening

Variable	*Age Groups*				
	65–69	**70–74**	**75–79**	**80–84**	**85+**
Breast cancer incidence rate per 100,000[1]	526.3	531.1	564.8	525.0	406.0
Sensitivity of screening test[2]	73.3	81.4	81.4	86.1	86.1
Specificity of screening test[2]	93.0	94.1	94.1	94.3	94.3
Breast cancer stage distribution[3]					
Early diagnosis					
Local	76.0	74.0	76.0	76.0	78.0
Regional	20.5	21.0	18.5	17.5	14.5
Distant	4.5	5.0	6.5	6.5	7.5
Interval diagnosis					
Local	62.5	62.6	64.25	63.4	60.95
Regional	29.65	29.4	27.15	27.3	27.1
Distant	7.85	8.0	8.6	9.3	11.95
Late diagnosis					
Local	50.0	51.2	52.5	50.8	43.9
Regional	38.8	37.8	36.8	37.1	39.7
Distant	11.2	11.0	10.7	12.1	16.4
Life expectancy with breast cancer[4]					
Local	18.8	15.40	12.1	8.87	6.26
Regional	10.05	9.23	7.44	5.46	3.67
Distant	3.08	2.66	2.39	1.94	2.21

Sources:

[1] Age group estimates are computed from the SEER*Stat Databases: Incidence—SEER 12 Regs Public-Use, Nov 2002 Sub for Expanded Races (1992–2000) (NCI 2003).

[2] Carney et al. (2003).

Table 9.4 Values Used In The Decision Tree To Assess Mammography Screening *(continued)*

[3] The distribution by stage at diagnosis for early diagnosis and late diagnosis cases are based on data from the National Cancer Institute SEER program (NCI 2003) as analyzed by Jeanne Mandelblatt, M.D. (Mandelblatt 2003). The percentage distribution of cases with an interval diagnosis is assumed to be halfway between the two extremes.

[4] Age- and stage-specific life expectancy of women with breast cancer was based on 1973–2000 breast cancer five-year survival rates (accounting for all causes of mortality) obtained from the National Cancer Institute SEER program (NCI 2003). These data were compared with age-specific survival and life expectancy information for the general population (accounting for all causes of mortality) to calculate age- and stage-specific life expectancy for women with breast cancer in the five-year age groups (NCHS 2002). (The procedures for computing life expectancies for women with breast cancer in general paralleled those used in Mandelblatt et al. (1992, Appendix Table 1, 729). All mortality rates were assumed to be constant, i.e., exponential decay.)

The results are insensitive to mammography screening rates. The second figure in each row for each age group (Table 9.6) is the life years saved and cost per life year saved based on actual screening rates in 2000–2001. Although screening continues to be effective at all ages, the magnitude of the savings generally decreases with lower screening rates. Similarly, lower rates of screening also lower costs, and the ratio between the two stays about the same for all age groups.

In conclusion, compared to no screening, increasing screening in women age 69 and older is well within the commonly applied threshold of cost-effectiveness of $50,000 to $60,000 for all age groups up to 85+ (Harvard Center for Risk Analysis 2003; Kerlikowske et al. 1999). The analysis shows that higher screening rates in each group would lead to higher costs of screening, but these costs would be offset by proportionate increases in average life expectancy.

Equity

Table 9.7 summarizes selected equity indicators related to breast cancer and mammography use by age and race for women 65 years of age and older. Disparity indices for the different age groups are the ratios between the rates for each of the oldest age groups (70–74, 75–79, 80–84, and 85+) and the youngest age group (65–69).

Table 9.5 Cost of Screening and Follow-up Services

Category	*Cost ($)*
Cost of screening	111.00
Marginal cost of clinical breast exam during routine visit for other conditions	28.00
Screening mammography (two-view)	83.00
Cost of diagnostic workup for abnormal screening mammogram	1,056.00
Incisional biopsy	309.00
Localization	79.00
Pathology reading	50.00
Two physician visits	102.00
Facility costs	440.00
Mammogram for diagnosis	76.00

Source: CMS (2003).

The numerator and denominator for the ratios differ for the different indices. For breast cancer incidence, deaths, and late-stage diagnoses, the numerator is the rate for women age 65 to 69, and the denominator is the rate for each of the older age groups. A disparity index value of 1.00 means that the rate for a given age group is essentially the same as for women 65 to 69 years of age. A value of greater than 1.00 means the rates are more favorable (lower) for the older groups of women compared to those 65 to 69, while a value of less than 1.00 means they are less favorable (higher). For the percentage having mammography, the numerator is the rate for each of the older age groups, and the denominator is the rate for women 65 to 69. Correspondingly, a value of greater than 1.00 for this index means that the screening rates are more favorable (higher) for the older groups of women compared to those 65 to 69, while a value of less than 1.00 means they are less favorable (lower). A comparable procedure is used for looking at disparities between races, with whites being the group to which the other

Table 9.6 Cost-Effectiveness Analysis by Age Group and Screening Rates

Age Group/ Screening Rates (%)	*Life Years Saved*	*Cost per Life Year Saved*
65–69		
100	4.49 days	$17,812
63.4	2.85 days	$17,782
70–74		
100	3.10 days	$25,785
60.2	1.86 days	$25,871
75–79		
100	2.48 days	$32,232
52.5	1.31 days	$32,035
80–84		
100	1.83 days	$43,680
39.2	0.69 days	$45,412
85+		
100	1.35 days	$59,211
19.3	0.26 days	$59,336

racial/ethnic groups (black, other race, and Hispanic) are compared.

The data in Table 9.7 document that the incidence of breast cancer in general is highest among women 75 to 79 years of age and lowest among women 85 years of age or older. Breast cancer death rates do, however, increase steadily with age, which may be associated with the overall greater vulnerability and higher death rates in general with advancing age. On the other hand, mammography screening rates decline steadily with age, with the rates being lowest for the oldest old (85+ years). Only one in five women 85 years of age or older had a mammography within the past two years. This oldest old group of women also have the highest rates of cancer diagnosed at late stages (regional or distant).

Although the incidence of breast cancer is higher among white compared to black and other minority women, the incidence of late-stage

cancer and breast cancer death rates are higher among black than white women. Unfortunately, there appears to be differential access to screening by race, with elderly black women much less likely than elderly white women to have had a mammogram within the past two years—41.0 percent versus 52.2 percent, respectively.

Mammography screening rates for around half of all elderly women fall short of the benchmark of having a mammography every one to two years (Table 9.7). Based on the evidence presented here regarding the risks and screening rates for women age 75 to 79 and black women, concern about the low rates of screening may be well founded. Only about four in ten black elderly women have had a mammography in the past two years—a proportion that should be increased, given their greater likelihood of presenting with late-stage cancer and associated higher breast cancer death rates. Socioeconomic factors such as poverty and inadequate access to healthcare remain important barriers to minority elderly women seeking mammography screening services (Mandelblatt et al. 2003; Smith-Bindman et al. 2000).

In summary, women age 75 to 79 and all black women age 65 or older appear to be most vulnerable in terms of breast cancer risks relative to rates of breast cancer screening. Women who live to age 85 or older are less likely to have breast cancer, but among those who do, a higher proportion are likely to present with late-stage cancer. Screening rates are also lowest among these oldest old women (85+).

Conclusion Based on Assessment of Mammography Screening

The analysis points to considerable convergence among the effectiveness, efficiency, and equity perspectives of lower mammography screening in older women (Table 9.8). The effectiveness analysis shows that the gap between where we are and where we want to be in terms of saving lives through screening is substantial and the gap increases with age. For women age 65 to 69, there is a 37 percent gap in the average number of life years saved with current screening rates as compared to what it could be with 100 percent screening. The gap increases to 81 percent for women in the 85+ age group. From an equity perspective, all women over 65 appear to be at considerable risk in terms of breast cancer risks relative to rates of breast cancer screening. The greatest risk is for the 75 to 79 age group and black women. Economic analysis suggests that mammography screening is cost-effective for older women into their 80s. It borders on being cost-effective for women through-

Table 9.7 Equity Indicators of Female Breast Cancer by Age and Race for Women 65+

Indicators	*Age Groups*					*Race Groups*				*Total*
	65–69	**70–74**	**75–79**	**80–84**	**85+**	**White**	**Black**	**Other**	**Hispanic**	
Breast cancer incidence rate per 100,000, Yr 2000[1]	526.3	531.1	564.8	525.0	406.0	553.7	446.6	297.2	333.9	520.8
Disparity index	*1.00*	*0.99*	*0.93*	*1.00*	*1.30*	*1.00*	*1.24*	*1.86*	*1.66*	—
Breast cancer deaths per 100,000, Yr 2000[2]	79.6	97.3	118.8	143.1	205.7	117.8	130.5	42.1	68.4	116.9
Disparity index	*1.00*	*0.82*	*0.67*	*0.56*	*0.39*	*1.00*	*0.90*	*2.80*	*1.72*	—
% breast cancers diagnosed at late stage, Yr 2000[3, 4]	26.8	23.8	26.2	24.0	30.5	25.3	33.5	25.6	24.6	25.9
Disparity index	*1.00*	*1.13*	*1.02*	*1.12*	*0.88*	*1.00*	*0.76*	*0.99*	*1.03*	—
% women 65+ with mammogram in preceding 2 years 2000–2001[5]	63.4	60.2	52.5	39.2	19.3	52.2	41.0	37.6	NA	50.8
Disparity index	*1.00*	*0.95*	*0.83*	*0.62*	*0.30*	*1.00*	*0.79*	*0.72*	—	—

Note:
The disparity indices for the different age groups are ratios between the rates for each of the oldest age groups (70–74, 75–79, 80–84, and 85+) and the youngest age group (65–69). The numerator and denominator for the ratios differ for the different indices. For breast cancer incidence, deaths, and late-stage diagnoses, the numerator is the rate for women 65–69, and the denominator is the rate for each of the older age groups. A disparity index value of 1.00 means that the rate for a given age group is essentially the same as for women 65–69 years of age. A value of greater than 1.00 means the rates are more favorable (lower) for the older groups of women compared to those 65–69, while a value of less than 1.00 means they are less favorable (higher). For the percentage having mammography, the numerator is the rate for each of the older age groups, and the denominator is the rate for women 65–69. Correspondingly, a value of greater than 1.00 for this index means that the screening rates are more favorable (higher) for the older groups of women compared to those 65–69, while a value of less than one means they are less favorable (lower). A comparable procedure is used for looking at disparities between races, with whites being the group to which the other racial/ethnic groups (black, other race, and Hispanic) are compared.
Sources:
[1] Estimates computed from the SEER*Stat Databases: *Age, Race (except Hispanic), Total:* Incidence—SEER 12 Regs Public-Use, Nov 2002 Sub for Expanded Races (1992–2000); *Race (Hispanic):* Incidence—SEER 11 Regs Public-Use, Nov 2002 Sub for Hispanics (1992–2000) (NCI 2003).
[2] Estimates computed from the SEER*Stat databases: *Age, Race (except Hispanic), Total:* Mortality—All COD, Public-Use With State, Total U.S. (1969–2000); *Race (Hispanic):* Mortality—All COD, Public-Use With State, Total U.S. for Hispanics (1990–2000) (NCI 2003).
[3] Estimates computed from the SEER*Stat databases: *Age, Race, Hispanic origin:* Incidence—SEER 12 Regs Public-Use, Nov 2002 Sub (1973–2000) (NCI 2003).
[4] Late stage includes "regional" and "distant." Unstaged cancers are excluded from the denominator.
[5] Source tables for biennial mammography services paid by Medicare among non-HMO women 65+ based on HEDIS 2002 criteria: Age Groups 2001 HD02; Race 65+ 2000–01 HD02 (CMS 2002).

out their mid-80s, and there is reason to be concerned about the decline in screening rates in these populations of older women.

The analysis also reveals points of possible divergence between the three perspectives. Based on the consideration of gaps between benchmarks and actual behavior, the effectiveness perspective leads to a focus on the oldest women with the lowest screening rates and the largest gap between outcomes under current screening rates and potential outcomes if everyone were to receive regular screens. The efficiency perspective, on the other hand, draws attention to women in their mid-70s for whom screening would be very cost-effective. The equity perspective focuses on women in their mid-70s and black women age 65 or older who appear to be the most vulnerable. Closing the gap between benchmarks and actual behavior to achieve effectiveness for women over the age of 80 presents particular challenges from the perspectives of efficiency and equity.

The analysis also reveals the difficulties encountered in bringing health services research to bear on evaluations of policy options because of gaps in knowledge of the consequences of selected provisions that must be addressed to make effective judgments of their probable impact. For example, focusing on age alone as is done in this analysis may be misleading, as there is a great deal of heterogeneity in health status and related comorbidities among the elderly (Mandelblatt et al. 2003). Because of the lack of information on selected data needed for the effectiveness and efficiency analysis by race and ethnicity, these analyses were not conducted by racial/ethnic subgroup, nor was any other subpopulation analysis conducted (e.g., considering subpopulations within each age group with and without comorbidities). Nor was the analysis accompanied by sensitivity analysis, which is needed both to validate the stability of the results and to identify which factors or parameters have the most influence on outcomes. Finally, the analyses did not take quality-of-life issues into consideration, therefore disregarding such factors as the anxiety of further testing or the psychological impact of living longer with cancer. Health policy decisions that fail to consider potential consequences must rely on political expediency. Therefore, even with the uncertainties and limitations, this rudimentary application of effectiveness, efficiency, and equity analysis of screening demonstrates how health services research can inform goal-oriented, evidence-based policy decision making.

Table 9.8 Effectiveness, Efficiency, and Equity Values of Mammography Screening[1]

		Age Groups					
Perspective	**Criteria**	**65–69**	**70–74**	**75–79**	**80–84**	**85+**	**Benchmarks**
Effectiveness	1. Screening rate as a proportion of benchmark	0.91	0.86	0.75	0.56	0.28	70
	2. Screen sensitivity as proportion of benchmark	0.83	0.92	0.92	0.98	0.98	88.2
	3. Screen specificity as proportion of benchmark	0.96	0.97	0.97	0.98	0.98	96.5
	4. Diagnosis at distant stage as proportion of benchmark	0.51	0.57	0.52	0.57	0.45	13.6
	5. Life years saved based on actual screening rates as proportion of benchmark	0.63	0.60	0.53	0.38	0.19	varies with group[2]
Efficiency	Incremental cost per life year saved, screening versus no screening	$17,812	$25,785	$32,232	$43,680	$59,211	$50,000 to $60,000

Equity	1. Incidence rate as proportion of benchmark	1.00	0.99	0.93	1.00	1.30	526.3
	2. Death rate as proportion of benchmark	1.00	0.82	0.67	0.56	0.39	79.6
	3. Diagnosis at distant stage as proportion of benchmark	1.00	1.13	1.02	1.12	0.88	26.8
	4. Screening rate as proportion of benchmark	1.00	0.95	0.83	0.62	0.30	63.4

Notes:

[1] A value of 1.00 means that the value on the criterion is equal to the benchmark value (effectiveness) or actually serves as the benchmark value (equity). A value of greater than 1.00 means that the value on the criterion compares favorably with the benchmark, and a value of less than 1.00 means that it compares unfavorably with the benchmark. See Tables 9.3, 9.6, and 9.7 for more detail on the criteria and related indicators on which they were based.

[2] Age group estimates of life expectancy are obtained from the decision-tree model assuming the actual screening rate. The benchmarks for each age group (the increase in life expectancy due to screening) are obtained from the decision-tree model based on 100% screening for each age group: 4.49 days (65–69), 3.10 days (70–74), 2.48 days (75–79), 1.83 days (80–84), 1.35 days (85+).

In summary, the discussion in this and previous chapters examines the conceptual and normative blueprints of the major healthcare system goals of effectiveness, efficiency, and equity. It analyzes the balances and trade-offs that influence policies and programs designed to realize these objectives. It reviews the methods used to measure the extent to which each of these goals has actually been achieved. And it encourages dialog among health services researchers, policy analysts, policymakers, and administrators who study, recommend, formulate, and implement health policy. Designing a healthcare system that optimizes the policy ideals of effectiveness, efficiency, and equity requires critical inquiry into the meaning of these goals and how best to apply them in gaining a better understanding of existing problems and pursuing unrealized opportunities. This book invites such inquiry.

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