INTRODUCTION AND SUMMARY

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The role of science in medicine has expanded rapidly in the past decades. As a result, the practice of medicine today is heavily, and increasingly, dependent on technology. Each year, hundreds, perhaps thousands, of new technologies enter the medical care system. New preventive, diagnostic, and therapeutic tools currently are available, and many infectious diseases can now be prevented. Innovations such as antibiotics have provided efficacious treatments for a number of conditions. Many of these technologies, and others, have undoubtedly contributed to the past century's substantial improvement in the health status of the American people. Additionally, relief of pain, amelioration of symptoms, and rehabilitation now have become possible for many patients with diseases that cannot be successfully prevented or treated.

However, concerns have arisen about the possible negative effects of the pervasive use of technology in medical care. The costs of medical care, which have escalated sharply, often are viewed as a significant societal problem. Currently, expenditures for medical care consume close to 9 percent of the gross national product (GNP); in 1960, health care costs represented 5.2 percent of the GNP. Third-party payers exacerbate the rise in health care cost because they put few constraints on expenditures. Prevailing methods of reimbursement encourage both inefficient utilization and increased provision of services, often without evidence of commensurate benefit to the patient.

Because of the lack of a direct and explicit relationship between the sharp cost increases of health care, the expanded use of medical technologies and improved health, questions have been raised about the efficiency of our health care delivery system. Additional concerns have been raised regarding the fact that many people or population groups have only limited access to medical care and its technologies. The increased role of science and technology in medicine also has led to ethical concerns regarding both the use of certain technologies, such as amniocentesis or renal dialysis, and the use of human subjects during research on medical technologies. Critics of the increased use of technology charge that medicine is being dehumanized by the use of machines and scientific methods. Some of the criticisms and concerns mentioned above may be unfair, some incorrect, and others fully accurate. Determining their validity is beyond the scope of this report. Health policy makers, though, must consider these and many more issues both comprehensively and individually. Consequently, the Office of Technology Assessment (OTA) has examined the individual issue of efficacy and safety because it is one of the prime keys to understanding many other health care concerns.

Efficacy and safety, or the direct medical benefit and risk of a technology, are the basic starting points in evaluating the overall utility of a technology. For example, ethical issues would not have been raised regarding amniocentesis if it had been demonstrated as inefficacious or clearly unsafe. In addition, efficacy and safety data are required in evaluations of the cost-effectiveness, cost-benefit, or social impacts of technologies. Well-informed decisions concerning modifications in the systems for reimbursement and the dif-

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fusion and use of medical technologies also require efficacy and safety information (153,196,204,226,254,299,340) .

Evidence indicates that many technologies are not adequately assessed before they enjoy widespread use (52,72,124,369). For example, the computed tomography (CT) scanner (355), the electronic fetal monitor (see chapter 3, case 7), and mammography (see chapter 3, case 4) are used frequently despite the lack of adequate information demonstrating their efficacy and safety. Many technologies which have been used extensively have later been shown to be of limited usefulness.

Information obtained from assessments of the efficacy and safety of new and existing medical technologies might serve three important purposes:

- To ensure that technologies demonstrated to have potential benefits with acceptable risks are made available rapidly in the private and public sectors; administrators of existing Government regulatory and financing programs could make sounder and faster decisions regarding the use of medical technologies with such information;
- To constrain the diffusion and use of technologies which either lack efficacy or cause excessive harm;
- To guide appropriate use of all technologies because they are rarely completely inefficacious or completely unsafe.

The Federal Government is concerned with questions of efficacy and safety because of its general role as protector of the public and its specific role as developer and user of medical technology. Because public funds pay more than 40 percent of the national health expenditure, concerns have naturally arisen about the benefits of medical care. Such questions seem certain to lead to increasing scrutiny of medical care expenditures and accelerated efforts to generate information on the benefits derived from the use of medical technologies. Indeed, a variety of Federal programs are hampered in carrying out their mandated tasks by lack of such information.

A state of total information on the efficacy and safety of medical technologies perhaps can never be attained because they are so numerous, complex, and varied. The task of evaluating all technologies would be overwhelming and, to OTA's knowledge, no health care expert has advocated such an undertaking. Therefore, the task of identifying and selecting technologies for assessment becomes critical.

SUMMARY

Efficacy and safety are complex measurements of actions or results that are best expressed in probabilistic terms. Efficacy is the probability of benefit from the use of a medical technology. When possible, this benefit should be expressed in terms of four factors: the type and probability of benefit, the medical problem giving rise to use of the technology, the population affected, and the conditions of use under which the technology is applied. Specifying the conditions of use serves to distinguish the terms efficacy and effectiveness. For efficacy, the conditions of use are considered to be ideal, or, as a substitute, experimental research settings. Effectiveness refers to average conditions of use.

Safety is a judgment of the acceptability of the risks posed by the use of a technology. Risk is parallel to efficacy in that it is a probabilistic measurement, and the four factors mentioned above are also part of its specification. Risk and safety can apply to either ideal or average conditions of use, but when the term "efficacy and safety" is used, it refers to safety and risk under ideal conditions.

Efficacy and safety should be considered together to be relevant to clinical or policy decisionmaking. Judgments must be made to determine whether the benefits justify the risks associated with the use of a technology in particular circumstances.

Case Studies: Issues Related to the Assessment of Efficacy and Safety

Seventeen short case histories illustrate the diverse nature of medical technologies, the difficulties in assessing their efficacy and safety, and Federal involvement in medical technology development, diffusion, and use. They also illustrate the fact that social impacts, such as economic and ethical problems, influence assessments of safety and efficacy. The cases do not exemplify all points concerning efficacy and safety; however, they do demonstrate many of the complexities that must be recognized and considered if medical technologies are to be evaluated for efficacy and safety.

Techniques for Estimating Efficacy and Safety

Techniques used in estimating efficacy and safety may take many forms. Traditionally, clinical experience, based on informal estimation techniques, has been the most important. Other techniques, such as epidemiological studies, formal consensus development, and randomized controlled clinical trials, however, are being used increasingly. The last technique, especially, has gained prominence (in the past 20 years) as a tool for assessing efficacy and safety.

No technique is universally applicable. Depending on the situation and technology, less complex methods may be more appropriate than the use of statistically sophisticated controlled trials. Frequently, combinations of various techniques are used because technology has its own strengths and weaknesses. For example, informal assessment techniques are based upon the valuable clinical experience of physicians; however, they are subject to strong biases and frequently are based on very small numbers of observations. Controlled clinical trials can draw upon larger numbers of observations and use complex statistical techniques to eliminate or reduce bias. Yet, difficulties also exist in conducting such trials. For example, trials often raise ethical concerns regarding the denial of a "promising" but unevaluated new technology to the control group members. Also, the design of the trial and the interpretation of the results are often subject both to value judgments and measurement problems. Nonetheless, all these techniques, especially controlled trials, remain powerful tools for gathering evidence on efficacy and safety.

Current Assessment Programs

Certain programs for evaluating efficacy and safety are required by Federal law. The Food and Drug Administration (FDA) administers regulatory programs which are

limited to medical products—drugs and devices. Manufacturers of these products are legally required to conduct efficacy and safety tests using the FDA guidelines. In addition, products must be licensed for marketing by FDA.

Other Federal agencies, such as the National Institutes of Health (NIH) and the Veterans Administration (VA), have no explicit mandate to assess efficacy or safety but do conduct clinical trials and other tests of efficacy and safety as part of their general mission. These trials test drugs, devices, and procedures.

The private sector supports numerous efforts designed to assess the efficacy and safety of medical technologies. In addition, the private sector often supplies the personnel and institutional resources for Federal assessment programs. However, private sector activities, particularly regarding medical and surgical procedures, are fragmented and uncoordinated. Individual physicians (either hospital or individual practice-based) are the source of many innovative procedures and much of the efficacy and safety testing done on procedures.

In summary, demonstrating the efficacy and safety of drugs and devices is required by Federal law prior to marketing. There is no corresponding requirement for procedures; however, some procedures are being tested by various Federal and private groups.

Implications and Status of Efficacy and Safety Information

Often, it is difficult or impossible to obtain information regarding the probable benefits and risks of technologies when used under actual or average conditions. Determining the efficacy and safety of a particular technology in controlled settings, therefore, represents the starting point in the effort to evaluate its potential benefit and risk. Consequently, efficacy and safety serve as the prime and critical criteria for judging the possible technical effects of medical technologies.

Any person or organization using or directly affecting the use of medical technology is a user of information on efficacy and safety. Patients, physicians, other health care professionals, biomedical researchers, and personnel in Government regulatory and reimbursement programs, public and private health planning agencies or quality assurance programs, other Federal and State health agencies, and medical schools are the prime examples of such users. Because of the large numbers of people who use efficacy and safety information, the development and dissemination of well-validated, timely, and relevant information is particularly critical.

Optimally, the processes of developing and disseminating safety and efficacy information should be coherent, coordinated, and the clear responsibility of one or several agencies or groups in the public or private sector. These processes, though very complex, can be perceived in terms of four basic elements: identification of technologies to be studied, testing through use of various techniques to generate information on efficacy and safety, synthesis of the finding of testing data and of any other relevant information—which often results in judgments or recommendations, and *dissemination* of the synthesized information to appropriate parties, including decisionmakers.

When current activities and programs for assessing efficacy and safety are compared to the optimal model described above, shortcomings are evident.

- There is no formal or well-coordinated overall system,

- Identification of technologies to be studied is a very informal, usually agency-specific process.
- Existing technologies are identified much less frequently for study than are new and developing technologies; thus, they are studied much less frequently.
- Medical drugs and devices are subject to a more rigorous process of assessment than medical procedures.
- Preventive technologies receive far less attention than therapeutic ones.
- Serious questions have been raised concerning the adequacy of funding for clinical trials.
- Synthesis activities are still too modest despite their recent expansion.
- The quality and appropriateness of medical literature, the primary source of synthesized information, has been criticized.
- Synthesis activities cannot be adequate when there is a critical lack of information regarding efficacy and safety.
- Federal agencies have not assigned a high priority to disseminating information.

These and other shortcomings may have contributed to the status of information on efficacy and safety, which may be inadequate to allow the rational and objective utilization of medical technologies. It has been estimated that only 10 to 20 percent of all procedures currently used in medical practice have been shown to be efficacious by controlled trial. Given the shortcomings in current assessment systems, the examples of technologies that entered widespread use and were shown later to be inefficacious or unsafe, and the large numbers of inadequately assessed current and emerging technologies, improvements are critically needed in the information base regarding safety and efficacy and the processes for its generation.

Policy Alternatives

Policy alternatives presented in this report are grouped into five sections outlined in chapter 7. The first section discusses alternatives to current Federal assessment activities both in terms of their expansion or change and the extent of that potential expansion. The other four sections correspond to the four steps in the assessment model. Each of these sections presents a number of options concerning the organizational location of the four functions of assessment. Following is a brief outline of these options:

Section One: Congressional Alternatives

Alternative A-1: Changes or expansions in the development of information concerning the safety and efficacy of medical technologies could occur solely in the private sector. This alternative would give the Federal Government the role of stimulating the private sector and monitoring its activities.

A-2: The Federal Government could expand its activities relating to the development of information on efficacy and safety of medical technologies. In this alternative, legislation could mandate the performance of certain activities.

A-3: Some combination of Alternatives A-1 and A-2 could be pursued.

Section Two: Identifying Technologies That Need Assessment

Alternative B-1. A new commission
B-2. Institute of Medicine
B-3. National Institutes of Health
B-4. Agencies involved in technology development
B-5. Food and Drug Administration
B-6. A new Federal office or agency, or the Office of Health Technology

Section Three: Requiring, Stimulating, Conducting, or Funding Studies

Alternative C-1. National Institutes of Health
C-2. Other Federal agencies
C-3. Food and Drug Administration
C-4. A new Federal office or agency, or the Office of Health Technology

Section Four: Synthesizing Information

Alternative D-1. A new commission
D-2. Institute of Medicine
D-3. National Institutes of Health
D-4. Agencies involved in technology development
D-5. Food and Drug Administration
D-6. Office of Health Practice Assessment
D-7. A new Federal office or agency, or the Office of Health Technology

Section Five: Disseminating Information

Alternative E-1. National Institutes of Health
E-2. Other Federal agencies
E-3. A new Federal office or agency, or the Office of Health Technology
E-4. A new office in the Department of Health, Education, and Welfare

SCOPE OF THE REPORT

This report discusses various possibilities for assessing the efficacy and safety of medical technologies systematically, thoroughly, and scientifically. It therefore focuses on efficacy and safety. Although efficiency, effectiveness, ethical, and other social concerns are related to efficacy and are also very important to the medical care system, these are not discussed at length.

Medical technologies are used for six different purposes: prevention, diagnosis, treatment, rehabilitation, patient support, and administration. The latter two classes of technology are not discussed in this report. Similarly, the report does not preview the safety and efficacy of technologies used in the psychosocial medicines, such as psychotherapy, counseling, and behavior modification. Rather, this report considers only the products of traditional biomedical research.

This report highlights both the critical need for data pertaining to safety and efficacy and the current and potential systems for obtaining such information. Once developed, this information could affect many alternative variables not discussed in this report, such

as the organization of medical care. The report discusses biomedical research and technology development in broad terms and presents a general framework for assessment; however, the policy alternatives refer primarily to options which can be implemented by Federal agencies.

ORGANIZATION OF THE REPORT

The report is organized into six remaining chapters. Chapter 2 presents the concepts of efficacy and safety, describes their characteristics, and develops working definitions of both terms. Chapter 3 outlines a short history of interest in the assessment of efficacy and safety and includes 17 brief case studies of medical technologies. The case studies are designed to illustrate various aspects of efficacy, safety, and their assessment. Particular attention is paid to highlighting policy issues raised by the use of certain technologies. Analytical techniques used to assess efficacy and safety are described in chapter 4. Chapter 5 reviews both Federal and private sector agencies and programs that engage in assessment activities. Chapter 6 discusses various aspects and the implications of the current assessment systems and programs. Building upon those implications, the last chapter presents a range of policy alternatives resulting from the analysis.