7.

POLICY ALTERNATIVES

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This chapter outlines a number of policy alternatives intended to correct some of the shortcomings in the assessment process presented in earlier chapters. Many of these options do not require new legislation because sufficient authority already has been written into law. In certain cases, desired actions could be stimulated by congressional oversight. Alternatives are presented for each of the four phases of the assessment process: identification, testing, synthesis, and dissemination. Although not previously discussed, several policy alternatives which attempt to translate efficacy and safety information into improved management of the utilization of technologies also are presented. Many of the alternatives and all of the steps of the process are both relevant and applicable to other types of assessments of medical technologies. Cost-effectiveness assessments, for example, could follow the four-step process. In that context, the advantages and disadvantages presented in this chapter would have to be modified to reflect the expanded functions.

The first question to be addressed is to what extent, if any, should the Federal Government either change or expand its activities in the process of assessing efficacy and safety. As described in chapter 5, existing Federal and private mechanisms execute important parts of the task of assessing the efficacy and safety of medical technologies. The Food and Drug Administration (FDA) has the statutory responsibility for assuring safety and efficacy of drugs and devices. Other Federal agencies, such as the National Institutes of Health (NIH), fund clinical trials that produce information on safety and efficacy. The private sector supports a large number of clinical trials, some mandated by FDA legislation. If Federal action were desirable, the four functions described above could be assigned to one agency or divided among several agencies in the Federal Government, They could be developed in one or more existing agencies, or an entirely new agency could be developed. Alternatively, the private sector could be encouraged or provided incentives to expand its activities in these areas. Or, some combination of Federal and private strategies could be pursued. Again, the first question is whether the Federal Government should or should not act; that question must be decided by Congress.

SECTION ONE: CONGRESSIONAL ALTERNATIVES

Alternative A-1: Any change or expansion in the development of information on the safety and efficacy of medical technologies could be left to the private sector. This alternative does not imply that there are no problems in existing private sector activities. This alternative would give Government a twofold role: to stimulate the private sector and to monitor its activities.

Alternative A-2: The Federal Government could expand activities relating to the development of information on efficacy and safety of medical technologies. A series of possibilities is presented later in this chapter which could be followed if this alternative

were desirable. This alternative could include legislative mandates for the performance of certain activities.

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

Any agency or agencies involved in assessing efficacy and safety could complete this task better if certain criteria were met. As examples, such an agency (or agencies) might need:

- An explicit mission concerning efficacy and safety assessment, The agency must accept this role and be held accountable for its performance.
- Statutory or regulatory authority to accomplish its mission. For example, it should be able to gain access to information it needs, including access to FDA materials considered to be proprietary.
- Adequate funding for the assigned mission. This might require an existing agency to reorder its spending priorities. In addition, new funding would probably be necessary.
- A competent, multidisciplinary staff with expertise in technology development and technology evaluation. Statisticians, physicians, epidemiologists, sociologists, economists, and others would be essential.
- Credibility with the health professions, scientists, industry, and third-party payers. It would be desirable if the agency already had relationships with these groups. Relationships with practicing physicians are important, particularly because information dissemination to that group would be an important task. Working relationships with other Government agencies involved in technology development and use would also be necessary.

The following sections discuss a series of alternatives to current policy in each of the four areas mentioned earlier. The functions could be addressed in many ways. The alternatives given are not exhaustive, but rather illustrative. Nor are they mutually exclusive. Furthermore, any agency could use a variety of programmatic mechanisms for meeting its objective: grants, contracts, intramural research, and mandating or requesting assessment from those who are able to provide a service. Any or all of these mechanisms could be used by any one agency. The alternatives that follow do not discuss or compare these approaches. (Table 9 summarizes the possible responsible organizations for conducting the four basic functions in efficacy and safety assessment.)

SECTION TWO: IDENTIFYING TECHNOLOGIES THAT NEED ASSESSMENT

A system for identifying technologies that need assessment could be developed in a number of agencies at various levels.

Alternative B-1: A special commission could be established to identify technologies needing assessment. This task will be a lengthy one requiring a special commitment. Establishing a special commission for that purpose would have some advantages. It could include prestigious physicians as well as experts from other disciplines and lay “representatives. Its deliberations could be open to public scrutiny. The major disadvantage in choosing this alternative is that such a commission would be far removed from sources of

Table 9.—Possible Sites for Carrying Out Four Key Tasks in Efficacy and Safety Assessment

Identifying technologies that need assessment

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

Requiring, stimulating, conducting, or funding studies

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

Synthesizing information

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

Disseminating information

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

new technologies, including those that might prove to be problematic. Furthermore, new staff and multiple subcommittees would be necessary.

Alternative B-2: The task could be assigned to the Institute of Medicine of the National Academy of Sciences, This is a desirable option because it chooses an extant, prestigious organization for the task. (The National Academy of Sciences previously carried out the task of evaluating evidence of the safety and efficacy of drugs on the market at the time of the passage of the 1962 Food and Drug Amendments.) The institute would probably have good sources of information about development of procedures in academic medical centers. As **a** quasi-governmental body, the institute could bridge the gap between Government and private sector medicine. The disadvantages of using the institute are the relatively small number of practitioners in its membership and the uncertainty as to whether it would perform such a task.

Alternative B-3: The task could be assigned to NIH. This arrangement is advantageous because NIH administers most of the Federal biomedical research support and a large percentage of the national expenditure. Staff at NIH could be expected to be cognizant of developments even in areas in which NIH has not committed funds. However, NIH has exhibited a stronger interest in developing medical technologies than in assessing them. To some extent, this potential problem could be ameliorated by placing

the function high in the administration of NIH, possibly in a new division or bureau. Such placement might avoid the parochial concerns of the various disease-oriented institutes. Nonetheless, if NIH were assigned this function, careful oversight by the higher echelons of the Department of Health, Education, and Welfare (HEW) and Congress would be essential to assure the effective completion of the task. Another potential problem in choosing this alternative is that d-e accomplishment of the basic mission of NIH could be hampered by such a new function.

Alternative B-4: Each agency (for example, the National Center for Health Services Research (NCHSR)) developing medical technologies could be asked to develop a list of its technologies that would need evaluation. This option would avoid the creation of another bureaucracy, It would also make an important function even more diffuse than it already is, and would lead to a great deal of overlap. In addition, it might **leave** many extant and new procedures unassessed. There are also potential, informal conflicts of interest associated with this alternative.

Alternative B-5: FDA could be assigned the task. FDA has experience in evaluating new technologies, and many of the same principles used in evaluations of drugs and devices could be applied to the area of procedures, with or without a regulatory program specifically concerned with procedures. The major disadvantage of using FDA is that it has had much more experience in working with private firms than in completing the type of function described here. Furthermore, FDA lacks technical resources and has image problems in the practicing community.

Alternative B-6: A new agency or office could be developed, possibly within HEW, that would be assigned the responsibility for efficacy and safety assessments. Its mission could include any combination of identifying technologies to be assessed, conducting and funding the studies, evaluating and synthesizing the information, and disseminating that information. The advantage in choosing this option is that no existing agency is deeply committed to assessing the efficacy and safety of medical and surgical procedures. Alternatively, it is difficult to establish a new agency, assign it a mission, document its need for a new budget, and recruit expert staff. Furthermore, it may not be desirable to develop a new bureaucracy that would handle all four functions when existent agencies and programs could do some, or most, of the job.

HEW has established an Office of Health Technology that would probably have the identification function within its mandate. The future structure and functions of that Office are unclear, however. If the Office of Health Technology begins functioning, it could engage in any or all of the activities specified in this report. Similarly, there are bills in Congress which would establish Federal agencies or offices that could be assigned many of the assessment functions, including identification.

SECTION THREE: REQUIRING, STIMULATING, CONDUCTING, OR FUNDING STUDIES

Expanded support for efficacy and safety testing could be developed in a variety of ways:

Alternative C-1: NIH could assume a larger role in testing both new and existing technologies for efficacy and safety. This option has the advantage of assigning the function to an agency that is already familiar with the field and, therefore, best equipped to

identify developing technologies. This alternative is disadvantageous because not only has NIH been reluctant to assume such an expanded role without new funding, but also NIH has resisted becoming deeply involved in existing medical practice. One method of realizing this option might be to develop a new program or bureau at NIH. The option would be most effective if new money were appropriated to NIH.

Alternative C-2: Other Federal agencies could be asked to expand their roles. The Veterans Administration (VA) is an obvious choice because it offers an excellent field for testing efficacy and safety due to its activities within a medical system that is quite practice oriented. However, VA's funds for medical research are limited, and most of its population is comprised of adult males. Furthermore, VA lacks connections both to HEW and the general community of practitioners. Nonetheless, VA and other agencies could make important contributions.

Alternative **C-3:** FDA could be given a larger role. However, FDA's experience is in administering a regulatory program, and it is not clear that procedures could be studied in a way analogous to regulation of drugs and devices. In addition, FDA has limited contacts with clinical researchers who could conduct the requisite studies.

Alternative C-4: A new agency could be developed in HEW to fund and conduct efficacy and safety testing, This option incorporates recognition of the fact that the function requires new staff and funds and an organizational focus, and that it would be difficult to change dramatically the mission of an extant agency. The major problem associated with this alternative is that of developing an entirely new agency, This problem could be partially overcome by assigning experts from existing agencies to the new agency. If a new agency were developed, it also might be an appropriate site for identifying technologies that need assessment. An agency with a vested interest in evaluating efficacy and safety could be expected to be active in identifying candidates for evaluation.

Studies would not have to be federally funded. Under FDA statutes, for example, the greatest expense of testing is borne by the manufacturers. If proof of the efficacy and safety of procedures were required by private and public third-party payers, private funding could support more of this testing. Third-party payers also could fund studies directly; National Blue Cross, for example, has funded a study by the Institute of Medicine on the efficacy of the computed tomography (CT) scanner. If successful, this model probably could be used more often. Much of the current testing of medical and surgical procedures is already supported by private funds, including service funds.

SECTION FOUR: SYNTHESIZING INFORMATION

Merely executing numerous research studies will not solve the problems of assessing the efficacy and safety of medical technologies. More data certainly will be helpful, but gaps in knowledge still will remain. Furthermore, value judgments are an integral part of making decisions of efficacy and safety. For example, the net benefit of a technology includes both efficacy and safety; yet, these two pints of the concept cannot be measured in fully comparable terms (see chapter 4). Value-based decisions must still be made regarding whether the positive benefit (efficacy) justifies the risk. Furthermore, study design and the general validity of research findings will need evaluation.

Many agencies and programs could synthesize information. Examining the literature available on a particular technology could highlight the need for further studies in certain areas. Thus, additional studies could appropriately be conducted by the same program

that identifies technologies needing assessment. Wherever this function is performed, it should be open to the public and other parties of interest; it also should have public and professional visibility.

Alternative D-1: The task of synthesizing information could be undertaken by the same commission that identifies technologies needing evaluation (Alternative B-l). The advantage associated with this option is that such a commission would be involved in developing information as a result of trials it stimulated. One disadvantage of this alternative is that such additional responsibility would necessitate the increased capability of staff and advisory committees. Also, such a commission might have little credibility with the practicing community.

Alternative D-2: The Institute of Medicine could be asked to undertake this task, in addition to identifying candidates for assessment. The same advantages found in Alternative B-2 would also apply here.

Alternative D-3: NIH could undertake the task of synthesizing safety and efficacy information. NIH already has the largest extant activity in this area and has begun to use the mechanism for developing consensus effectively in at least one area. However, NIH has shown little inclination to make judgments that could be used by regulatory agencies. * Perhaps NIH could continue to develop consensus in areas in which little controversy exists and in which consensus could have immediate benefits, such as that of diagnosis and treatment of hypertension.

Alternative D-4: Agencies involved in technology development could also synthesize the information derived from trials. One concomitant disadvantage with this option is the diffusion of the function among numerous agencies. The disadvantages mentioned directly above in Alternative D-3 also would apply.

Alternative D-5: FDA could undertake the performance of this task. It already has extensive experience synthesizing and evaluating information submitted both by drug and device manufacturers and physicians. It also has a mechanism for forming expert committees and using outside consultants which would be desirable and applicable to this alternative. However, FDA is basically a regulatory agency and may not be able to attract the scientists necessary for regulating procedures. Again, FDA's negative image with the practicing community would hamper its work.

Alternative D-6: The Office of Health Practice Assessment (OHPA) could undertake the task. OHPA already makes synthesis decisions for the Medicare program. Given adequate resources and access to appropriate experts, it could accomplish the task of synthesizing safety and efficacy information. However, OHPA currently lacks credibility with the practicing community and lacks expertise and access to the information required to complete the task.

Alternative D-7: A new Federal agency could undertake the entire task, including synthesis (see Alternative B-6).

*NIH does provide some information, in the form of judgments or recommendations, to agencies such as the Food and Drug Administration. However, the 1977 Department of Health, Education, and Welfare technology management study concludes that the needs of regulatory agencies remain generally unfulfilled.

SECTION FIVE: DISSEMINATING INFORMATION

Synthesized information—regardless of how valid, understandable, or relevant—is of little value if it is not disseminated to those individuals and organizations which need it. This task is more complex than it seems. The agency responsible for such dissemination must not only have access to the synthesized efficacy and safety information, and any other relevant information, but also must develop, improve, or expand methods of communication to appropriate parties, identify those parties, evaluate the effects of its actions in terms of information conveyed, and perform other related tasks.

Alternative E-1: NIH could refine and expand its dissemination efforts. That agency is one of the most active in disseminating information; and in addition, it contains the National Library of Medicine. However, NIH is reluctant to expand its role in this area, particularly in regard to practicing physicians and health care delivery-related information, partly because of budgetary constraints.

Alternative E-2: This function could be assigned to the Federal agencies involved in testing or synthesis that already perform the dissemination task to a limited degree. The utility of increasing activities by all those agencies, however, would be qualified by at least three factors: parties in need would receive information from a multitude of sources; the function might require a degree of talent, skill, and technique development that many of the agencies could not attain; and, many of the agencies do not have the necessary contact or credibility with the parties who need the data.

Alternative E-3: A new Federal agency, as described in Alternative B-6, could be given the funds and personnel for this task. A close working relationship with NIH would have to be established,

Alternative E-4: Instead of assigning the task to a new agency, either one created to perform the dissemination task or one created to perform alternative tasks, a new office perhaps could be developed either at the level of the Assistant Secretary for Health, HEW, or within an existing Public Health Service (PHS) agency. Presently, there is no focus within HEW for health professional information dissemination as there is now for consumer information. Placing a new office at the Assistant Secretary level would have the advantage of proximity to the National Center for Health Statistics (NCHS). In addition, it would be at a level high enough for access to information and resources of PHS agencies, particularly NIH. It may also facilitate communication with the Health Care Financing Administration (HCFA). A disadvantage of a new office would be its having to start with little credibility or few contacts with many of the parties who need the information. Also, functional conflicts with NIH would have to be anticipated as in Alternative E-3.

USING INFORMATION

This report has primarily addressed a specific problem: the lack of accessible, reliable information on the safety and efficacy of medical technologies. The mere availability of such information, however, does not assure the efficacy and safety of medical technologies currently in use. The development and dissemination of efficacy and safety information leads to a fifth step, namely, the application of such knowledge.

As illustrated in chapters **3** and *6,* many Federal programs use, or could use, information regarding efficacy and safety, According to health planning legislation, ap-

proval of capital investments depends on establishing "need," and such establishment requires scientific information regarding the health benefit expected from application of a particular technology. Professional Standards Review Organizations (PSROs) that examine services for appropriateness, depend on such information. Federal programs that finance and provide medical care also must make some evaluation of efficacy and safety in determining reimbursement of a particular procedure. All these programs must make decisions based partially on efficacy and safety. These decisions often have been made passively or by default.

The following are intended to serve only as examples of possibilities for using information on efficacy and safety to assist providers and consumers in making informed decisions.

Example 1: Medical and surgical procedures could be subject to regulation. In this option, all procedures would be evaluated for safety and efficacy, and only those approved by an agency such as FDA could be used. Such an approach, while theoretically possible, would be difficult to enforce. Because procedures are developed in many sites and are not products, they cannot be regulated through such measures as controlling advertising and interstate transport. In addition, physicians would undoubtedly resist such regulation. The process would be expensive and could retard innovation.

Example 2: When a new technology shows promise, and when a group responsible for the identification task has judged it worthy of full-scale evaluation, medical centers that have the resources to conduct evaluation studies could be allowed to use the technology. Third-party payers would fund this evaluation on **a** prospective budget basis; they would not pay fee-for-service charges for use of the technology until its efficacy, safety, and indications for use were evaluated. No additional public funds would be required if this option were utilized; yet, private insurance companies would spend less on the testing than they would otherwise spend on reimbursement for unproven procedures. No legislation or regulations would be required, and any provider could offer the technology to anyone willing to pay for it out-of-pocket. To be successful, such a mechanism would need a panel of well-recognized professional experts whose plan for testing the technology would have credibility. The plan would include specified testing sites and conditions of use. A similar mechanism could be used for technologies already in use, but payment would not be withdrawn while they were being tested. Once testing was completed and the technology proved to be relatively unsafe or lacking efficacy, reimbursement for its use could be terminated, or specific conditions for reimbursement could be outlined by third-party payers.