The “modest” proposal advanced by Friedman and McCabe in this issue of the Journal (1) that “All third-party payers of health care (private and public) should cover the clinical care costs (within the financial agreements of policy provisions) but not the research costs associated with patient participation in National Cancer Institute (NCI)-sponsored therapeutic clinical trials” can be an acceptable one. It can be acceptable because, as the authors point out, the involved constituents share one basic goal: to define the probabilities that a particular treatment will be safe and effective. At present, the nation’s health care community is failing to meet this objective for many new treatments critical to patients in need.

To become acceptable and to achieve the stated common goal, the modest proposal must at once be expanded and components within the proposal at hand must be more narrowly defined. This editorial briefly describes the needed elaboration and details specific modifications to the proposal that would permit it to be subsumed within a new, efficient, and expeditious system for the introduction and diffusion of health care technologies.

Proposal

A national advisory body should be established to oversee the conduct of evaluative outcomes research on important new technologies. The use and study of these new technologies would be restricted to a network of designated academic centers. Reimbursement for the use of new technologies (i.e., procedures, procedures involving Food and Drug Administration (FDA)-approved devices, drugs used beyond FDA-approved indications) would be provided but would be restricted to use under the study protocol and within the identified academic centers. In return, outcome data would be collected and analyzed under the auspices of the independent advisory body. When the outcome data collected from the study were judged to be sufficient, a comprehensive evaluation would be undertaken, culminating in definitive conclusions about the safety and effectiveness of the technology. Once the national advisory body concluded that a technology was safe and effective for the specified indication, it would be allowed to diffuse into practice. At this point, individual payers would decide whether they would pay for the specified use of the technology.

Rationale

The rationale for this extended modest proposal is that outcome data should demonstrate the safety and effectiveness of a particular technology (e.g., high-dose chemotherapy-autologous bone marrow transplantation) for its intended indication before diffusion of the technology occurs. Assurance of reimbursement would permit earlier access for use as “treatment” for patients in need. The “hassle factor,” which is a factor for payers, providers, and, unfortunately, sometimes for patients, would be mitigated, if not eliminated. The outcome data so desperately needed to make informed risk-benefit determinations integral to the formulation of sound coverage policy and of sound clinical decision-making would be generated, collected, and analyzed in a cooperative scientific environment. Inadequate investigator participation in clinical trials would be rectified by the offer of the use of the most advanced technology and the financial incentive of reimbursement early on. Inadequacies in patient accrual would be addressed by the concomitant expansion of the size (number of studies) and by restriction of general diffusion. The permissible designs used in studies also would be expanded. Studies and patient care would be conducted in institutions where rigid quality assurance such as that carried out by the NCI “assures an unmatched level of consistency, competence, and experience” (1). Society would achieve answers to important medical issues in a much more efficient and expeditious manner. Finally, as an added bonus, the sagging infrastructure of academic medicine would be buttressed by the restriction of payment for new technology to the setting where its initial diffusion and study belong, that is, in institutions dedicated to the deliberate evaluation of and advancement of patient care.

An Example

The introduction and diffusion of high-dose chemotherapy-autologous bone marrow transplantation (HDCT-ABMT) into the health care system can be characterized as disorganized, at times unscientific, confrontational, inefficient, and, in recent limited circumstances, avaricious. What saves this episode in health care from being described as an unmitigated disaster is the fact that many patients have received beneficial care and many dedicated investigators have persevered in their attempts to demonstrate the effectiveness and relative safety of HDCT-ABMT for certain indications. How much more rational, responsible, efficient, and valuable to patients, physicians, and payers it would have been if HDCT-ABMT were introduced into medical practice through a systematic approach as described above.

For example, a hypothesis would be generated to support the extension of HDCT-ABMT from application in established diagnoses such as non-Hodgkin’s lymphoma to application in the treatment of metastatic breast cancer. A network of centers...
under the auspices of the NCI would be established to evaluate the safety and effectiveness of the procedure.

The same logical NCI system for advancement from phases I through III would persist. At some point (phase III or late phase II), when it was determined that sufficient antitumor activity had been shown for the particular HDCT-ABMT regimen to justify its study as a treatment, expanded study and payment for patient care costs would begin.

The study would have to take place simultaneously on two levels. First, the NCI would sponsor a national randomized, controlled clinical trial comparing the "investigational therapy" with the best available standard. Obviously, important health outcome (e.g., survival and quality of life) would be discussed and a specific outcome or outcomes would be selected as the variable(s) for study. Patients willing to be randomly assigned to and to participate in such a trial would be enrolled. The study on metastatic breast cancer would be carried out within a network of academic centers approximately as large as the current NCI study and with a geographic distribution similar to that in the NCI study.

Restriction of the study to the use of the recognized gold standard, the randomized, controlled clinical trial, will not suffice. Patients will persist in their desire to receive the "new, advanced" treatment and will not agree to random assignment. The same will also hold true for a large percentage of physicians who refer these patients. An alternative mechanism (e.g., case series) would have to be used for the collection, synthesis, and analysis of outcome data from patients who insist on receiving the new treatment, in this case HDCT-ABMT. These studies would be conducted in parallel at the same academic centers.

During the conduct of these studies, the patient care costs would be covered by the payers. The research costs, as defined in Friedman and McCabe's commentary (1), would be paid by the sponsoring not-for-profit research institute. Patients would be enrolled in either type of study only if they met selection criteria established for such a study. Payers would abide by the clinical judgment of the participating institutions. Thus, there would be no individual case management regarding the appropriateness of the provision of the study treatment to patients.

Data collection and analysis would be conducted by an objective panel, with representation given to practicing physicians and also to payer medical directors. When sufficient data were available, this independent panel would reach a definitive conclusion about the safety and effectiveness and would recommend whether diffusion should occur. Then individual payers would formulate their specific coverage policies.

Discussion

This proposal represents a general concept that needs to be explored by all constituents of the health care community. It builds on the statement made by Friedman and McCabe (1) that "all interest groups are assumed to have an investment in identifying or preventing the application of ineffective, unnecessary, or harmful clinical interventions." It is entirely consistent with their call to establish a mechanism that "would permit careful evaluation of a new costly therapy, such as autologous bone marrow transplantation, rather than permit its uncritical diffusion into routine care."

The Friedman and McCabe proposal and this expanded version seek to establish a logical and orderly mechanism for the diffusion and utilization of important new technologies. This mechanism would be based on solid outcome data and expert consensus. Payers would cover the cost of important new technologies early on within a designated network of academic medical centers in exchange for data collection and analysis and definitive conclusions about the safety and effectiveness of the technology. Implementation of this proposal would contribute to the enhancement of societal good by 1) supporting the use and study of technologies that will lead to either definitive support for widespread use or agreement that the technology should be discarded, 2) enhancing the availability of important new technologies to needy and appropriate patients early on, and 3) shoring up the sagging infrastructure of the academic medical center.

Critics will be quick to pounce and claim that great harm will be done both to innovation in technology and access to needed care by patients. The proposals at hand will, in the long run, facilitate innovation, introduction, and diffusion of new technologies by establishing a broader base of financial and scientific support early on and by financing a rapid general diffusion once the value of a technology has been established. Indeed, this extended proposal recognizes and affirms the import of a technological imperative in medicine. However, it seeks to avoid technological Armageddon.

The introduction and utilization of health care technology are at once recognized as the most responsible and most controllable factors contributing to the rate of rise of health care expenditures. Yet, as various solutions are proposed to mitigate this rate of rise, the issue of technology utilization has not been directly addressed. When rates of inappropriate utilization of 15%-30% are discussed, two other important factors are missing from the computation. First, there are technologies (e.g., external carotid–internal carotid artery bypass, Garren gastric bubble, gastric freezing) that should never have been introduced into general practice; thus, the rate of inappropriate utilization is 100%. Second, there are specific devices (e.g., magnetic resonance imaging) that may be applied for an approved indication; yet they are applied uselessly due to insufficient power of the individual device (e.g., magnet) or inadequate technical considerations. Thus, the cost of the inappropriate utilization of technology may well exceed present estimates.

In the near future, high-dose chemotherapy-autologous bone marrow transplantation (HDCT-ABMT) may achieve the distinction of becoming the technology that annually expends the greatest amount of health care dollars. What will happen to a new technology like HDCT-ABMT in the year 2000? Will it be guided expertly through an orderly scientific process with expanded patient access and, then, into full clinical practice? Will it practice as HDCT-ABMT is doing now with limited scientific study paralleled by rapid and somewhat chaotic diffusion resulting in irretrievable patient outcome data? Or, will HDCT-ABMT be prioritized as technology #798 with national health care funding available only through technology #612.

There are many important issues that have not been discussed here but which will be integral to any discussion and development of this proposal. They are

1) What is the source of authority for the national advisory board? How will it be constituted? How will it relate to the
A Swift Response to a "Modest" Proposal

John L. Cova*

Friedman and McCabe's proposal (/) that insurers cover the patient care costs associated with National Cancer Institute (NCI)-sponsored clinical trials fails to address several fundamental issues related to the appropriate role of private health insurance. Private insurers accept the argument that NCI-sponsored clinical trials contribute to the public good. But they do not believe that it is appropriate or feasible for them to cover the patient care costs of individuals who are hospitalized for the sole purpose of research.

Private health insurance pays for medical technologies and interventions that are proven to be safe, effective, and medically necessary. Asking private insurers to defray the costs of clinical trials that seek to answer safety and efficacy questions about emerging therapies is, therefore, inherently contradictory. If existing public agencies received adequate funds for clinical research, private insurers would not have to go beyond the current practice of paying the health care costs of patients who enter a clinical trial while hospitalized for covered therapies.

Who should pay for clinical research? Who should cover the financial shortfall that results from inadequate government funding? It is highly unlikely that the federal government will cover its own shortfall in the near future. Elected officials are acutely aware of the public's aversion to taxation as well as of the exigencies of the deficit and are reluctant to allocate additional resources for clinical research. Because of this situation, Friedman and McCabe advocate that responsibility for clinical research be shifted from the public to the private sector. From their perspective, this is a "modest" solution. However, it is at best only a short-term fix with undesirable consequences. A closer integration of private insurance and clinical research will, in the long-term, force insurers and employers to impose "taxes" in the form of higher premiums, co-pays, and deductibles. The net effect will be an increase in the cost of private health insurance.

It is unrealistic to expect private insurers to cover unproven technologies that may not improve patient outcomes. An insurer formulates rational coverage policies by balancing what is normative with what is equitable. All health insurance programs, regardless of the funding mechanism, are ultimately constrained by finite financial resources. Thus, extending coverage policies to include expensive unproven technologies (ones that cannot be expected to cure but may offer some marginal clinical benefits) increases the cost of covering other treatments that are known to be 100% effective (i.e., appendectomies). The unintended effect of this kind of coverage expansion is an increase in health insurance premiums. The adverse consequences of this expansion are greatest for individuals at the lower end of the income scale.

Patient care costs should be included in the total cost of performing clinical research, just as the cost of reagents, equipment, and animal care is factored into the total cost of laboratory experiments. The fact that insurers occasionally pay for all or part of the cost of clinical research does not imply that these practices are always appropriate or in the long-term interests of those engaged in clinical research. One could argue that such practices encourage continued compartmentalization of total research costs into research and patient care costs. Further, it seems inherently unfair for institutions that sponsor clinical research projects to expect volunteers to pay for their...