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Prologue: As health care costs continue their upward spiral, insurers attempt to rein in the amounts they cover. One way of doing this is to cut spending for procedures, devices, and drugs that are considered “experimental.” In this paper the authors argue that had insurers, including Medicare, not paid the costs associated with “unproven” technologies in the past, many of the innovations for which American medicine is lauded might not have come to pass. Insurers’ reluctance to foot the bill could curtail the development of new technologies and threaten our health care system’s ability to keep up the pace of helpful innovations. This paper contains a specific policy proposal for all stakeholders—payers, providers, government, and patients—to bear some responsibility to ensure that this does not occur. Earl Steinberg is vice-president of Health Technology Associates in Washington, D.C., and is codirector of its Outcomes Studies Group. He is a professor of medicine at the Johns Hopkins School of Medicine and holds a joint faculty appointment in the Department of Health Policy and Management, Johns Hopkins School of Hygiene and Public Health. He received his medical degree and a master of public policy degree from Harvard. Sean Tunis was director of the health program at the congressional Office of Technology Assessment (OTA) until that agency was closed at the end of September 1995. He joined the staff of OTA in 1992. Tunis holds a medical degree and a master’s degree in health services research from Stanford University. David Shapiro is a senior analyst at the Physician Payment Review Commission. He also is an attending physician at San Francisco General Hospital and assistant clinical professor of medicine at the University of California, San Francisco. He holds a medical degree from the University of California, Los Angeles, and a law degree from Yale University.
Abstract: As the number and cost of new technologies grow, it is increasingly important that we develop sound policies for payment for those technologies while their clinical impacts are being defined. Such policies need to balance social interests in promotion of innovation, early access to promising technology, patient safety, control of health care costs, and return on investment. We present a rationale, policy options, and a proposal for insurance coverage of experimental technology.

Americans are enamored with medical technology. At the same time, Americans are increasingly concerned about the cost of health care and whether some of what is spent goes for services that provide little or no value or that actually produce more harm than good.¹

Little attention has been given to the implications of insurance policies that would cover experimental technologies, or how such coverage should be structured. In this paper we address whether medical services whose benefits have not yet been demonstrated (to which we refer as unproven or experimental) ought to be covered by health insurance policies and, if so, under what circumstances and in what way.

When Is A New Technology No Longer Experimental?

Judgments about which technologies are safe and effective, and which remain of uncertain value, must be made by all health care payers (and providers). In recent years the financial implications of these decisions have increased. Both indemnity insurers and managed care plans are faced with strong incentives to be more selective regarding services they provide or pay for.

Historically, most indemnity insurers have included language in their contracts indicating that experimental or investigational services are not covered. Such contractual language has generated considerable controversy and litigation, largely related to what these terms mean and how the plan has determined whether a particular technology is experimental.²

Medicare’s approach. The way Medicare decides what is experimental, and hence not covered, illustrates the problem with existing approaches to defining experimental technology. Medicare continues to base coverage decisions on criteria established in the Social Security Act of 1965, which prohibits Medicare from paying for items or services that are not “reasonable and necessary.”³ “Reasonable and necessary” services, in turn, are defined as those that are “safe and effective, not experimental, and appropriate.” Determinations of which technologies are “safe and effective” are made by the U.S. Food and Drug Administration (FDA) using criteria delineated in the federal Food, Drug, and Cosmetic Act of 1938.

Blue Cross and Blue Shield approach. The national Blue Cross and Blue Shield Association (BCBSA) has developed an explicit, evidence-
based process for determining whether a technology is experimental. This process employs five criteria: (1) The technology must have final approval from the appropriate government regulatory bodies; (2) scientific evidence must permit conclusions concerning its effect on health outcomes; (3) it must improve net health outcomes; (4) it must be as beneficial as any established alternative; and (5) the improvement must be attainable outside the investigational setting. The results of these assessments are made available to the sixty-seven individual Blue Cross/Blue Shield plans, as well as other insurer and health maintenance organization (HMO) subscribers, each of whom makes an independent decision with regard to coverage of a particular technology.4

**FDA approval versus insurers’ coverage decisions.** Because the FDA does not regulate surgical or medical procedures, and because many diagnostic tests may be performed by clinical laboratories without ever having been reviewed by the FDA, health insurers must make decisions regarding coverage of some technologies in the absence of FDA approval. Also, medical devices may be approved by the FDA on the basis of a judgment that they are substantially equivalent to a technology that was marketed prior to the 1976 Medical Device Amendments to the Food, Drug, and Cosmetic Act. However, many of the devices marketed prior to 1976 have never been shown to satisfy current FDA approval criteria.5 Thus, a device approved under FDA’s 510(k) provision as being substantially equivalent to a pre-1976 device does not necessarily warrant coverage.

Differences between FDA and insurers’ decisions also may derive from differences in the clinical outcomes each considers when judging a technology’s effectiveness. For example, the FDA recently approved prostate-specific antigen (PSA) screening tests for men because the test has been judged by the FDA to be effective in early detection of prostate cancer. Some insurers, however, do not cover PSA screening because they have concluded that there is not sufficient evidence that it leads to a decrease in mortality. PSA screening is the subject of an ongoing clinical trial, sponsored by the National Institutes of Health (NIH), which is designed to determine whether such screening is effective in reducing mortality.

FDA marketing approval and insurers’ coverage decisions also could differ because each could make different judgments about the clinical significance of the benefits of a particular technology, or the net benefits of a technology. For example, one HMO decided that tacrine (a drug approved by the FDA for Alzheimer’s disease) would not be available on its formulary, despite the determination by the FDA that it was effective in improving cognitive functioning in patients with Alzheimer’s disease. The HMO’s decision was based on a judgment that the benefits of tacrine, while statistically significant, were not important enough clinically to warrant the
risks of liver injury associated with its use.\(^6\)

Finally, many insurers employ criteria in addition to proof of safety and effectiveness. For example, one of the BCBSA criteria is that the new technology must be at least as beneficial as an established alternative. The FDA, in contrast, does not require information on the effectiveness of new technologies compared with those already on the market. Many insurers also require evidence that the benefits of a technology are reproducible in settings outside of the ones in which the technology was developed. There is no similar FDA requirement, which creates the potential for discordance between the FDA's regulatory decisions and insurers' or HMOs' policies.

**Proposed definition of experimental.** In our view, a literal interpretation of the terms *experimental* and *investigational* is not appropriate. For example, if these terms are taken to mean that a technology is “under investigation,” then the requirement that a technology not be “investigational” is too stringent because many technologies undergo continued evaluation long after they have been shown to improve health outcomes.

The definition of the terms *investigational* and *experimental* that would make the most sense with regard to insurance coverage decisions is whether the impact(s) of a given technology are or are not known. If sufficient data are not available on its likely net health impacts, then the technology could reasonably be considered to be investigational or experimental.

### Coverage Of Experimental Technologies: Current Policy

There are a number of mechanisms through which payment is provided for experimental technologies. A review of the rationale for and experience with these approaches is useful in considering potential new policy options.

**Investigational drugs.** Drugs that have not been formally approved by the FDA can be used and paid for under certain circumstances. The FDA requires that all investigational new drugs (INDs) be evaluated in controlled studies to determine safety and efficacy. A subcategory, called a "treatment IND," is available to permit particular drugs to be used outside of the clinical trials being conducted under the IND designation. The FDA will grant a “treatment IND” when (1) the drug is intended to treat a serious or life-threatening disease; (2) there is no comparable or satisfactory alternative drug or other therapy to treat that stage of the disease in the intended patient population; (3) the drug is under investigation in a controlled clinical trial under an IND, or all clinical trials have been completed; and (4) the sponsor of the clinical trial is actively pursuing marketing approval of the investigational drug with due diligence.\(^7\)

Manufacturers generally pay for the cost of the trials needed to gain FDA approval for an IND, including the cost of the investigational drug itself.
For treatment INDs, however, manufacturers are permitted to charge patients for the cost of the drug, but they are not permitted to make a profit. Insurers vary in their willingness to pay for treatment INDs. Medicare considers them to be experimental and so covers neither the cost of the drug nor the associated costs of care, with the exception of Group C cancer drugs, which are treatment INDs sponsored by the National Cancer Institute (NCI). Group C drugs are paid for by the NCI, and Medicare covers the cost of care surrounding their use. The Civilian Health and Medical Program of the Uniformed Services (CHAMPUS) does not cover the cost of the treatment INDs themselves but does cover the related medical care “when the patient’s condition warrants their administration and the care is provided in accordance with generally accepted standards of medical practice.” Blue Shield of Northern California, by contrast, covers the use of treatment INDs (both the drugs and the associated treatment costs).

**Off-label uses of approved drugs.** The FDA permits an approved drug to be marketed only for its approved indications. Once a drug is approved for one indication, however, physicians can legally prescribe the drug for other indications as well. For various reasons, a manufacturer may decide not to seek FDA approval for the additional indications, even though it cannot promote the drug for such off-label use. Of the accepted indications listed in a major drug compendium published by the United States Pharmacopoeia, approximately 20 percent are not approved by the FDA. Off-label use is especially common in treatment of cancer.

Insurers vary in their policies regarding payment for off-label uses of drugs. A 1992 U.S. General Accounting Office (GAO) study found that a majority of oncologists had been denied reimbursement for an off-label use, and that oncologists believed that the rate of such denials was increasing. In response to perceived difficulties in obtaining coverage for “accepted,” off-label drug uses, seventeen states have passed legislation that requires private insurers to cover indications included in three major compendia. These drug compendia are a compilation of published evidence and expert opinion regarding the benefits of particular drugs that the compendium considers of proven value, whether or not the FDA has formally approved the drug for a given use. A federal law passed in 1990 cites these compendia as a standard reference for Medicaid reimbursement. Medicare coverage for care associated with off-label uses had been largely at the carriers’ discretion in the past, but since 1994 Medicare has been required to cover the use of FDA-approved drugs for indications listed in any of the three compendia or for any indication supported by the peer-reviewed literature.

**Devices.** The FDA divides medical devices into those that pose “significant risk,” to patients and those that do not. Any manufacturer of a device that is determined to pose a significant risk to health must be granted
permission by the FDA to gather clinical data under an Investigational Device Exemption (IDE). Many devices being tested under an IDE represent incremental improvements over approved devices that have been demonstrated to be safe and effective. Thus, there may be little reason to believe that such incrementally improved devices are not safe and effective.

The cost to device manufacturers of supporting clinical trials of each new generation of a device could pose a significant barrier to the development and evaluation of these incremental improvements. Some private insurers have covered the costs for use of a device under an IDE, especially when the new device differs only incrementally from an existing one.

Medicare coverage for IDE devices has been in flux over the past decade. Before 1986 hospitals and physicians often were paid by Medicare for the care associated with use of investigational devices, as local Medicare carriers were given the flexibility to provide coverage under most circumstances. In 1986 Medicare informed its carriers that they should not pay for procedures performed using devices that did not have marketing approval from the FDA. In June 1994 the Office of Inspector General of the Department of Health and Human Services (OIG/HHS) announced a nationwide investigation into billing practices of hospitals and physicians for non-FDA-approved devices over the past ten years. The OIG/HHS issued subpoenas to 135 hospitals for information regarding Medicare payment for medical devices not approved for marketing by the FDA. The subpoenas subsequently were narrowed to focus on selected cardiac devices that were not FDA-approved. In 1994 Medicare reasserted that it does not cover IDE devices or care associated with the use of IDE devices. The OIG/HHS investigation is still ongoing.

Prompted by concern over the impact of this investigation on the availability of potentially useful new medical devices to Medicare patients, the Physician Payment Review Commission (PPRC) recommended that Congress provide Medicare coverage for experimental devices under some circumstances. Legislation consistent with the PPRC recommendation was introduced in both houses of Congress in June 1995. Rep. William M. Thomas (R-CA) introduced H.R. 1744, which would require that Medicare pay for the use of any IDE device used in place of an FDA-approved device. Payment would not be permitted above the cost of the approved device. A slightly different proposal introduced by Sen. Orrin G. Hatch (R-UT), S. 955, would provide payment for investigational devices that were used instead of a covered device or procedure.

In September 1995 the Health Care Financing Administration (HCFA) issued a new rule under which it would, for the first time, cover certain devices being evaluated under an IDE. In rather tortured reasoning, HCFA stated that although it would not cover “experimental/investigational”
devices being evaluated under an IDE, it would cover “nonexperimental/ noninvestigational” devices being evaluated under an IDE. “Experimental,” devices were defined as those for which absolute risk of use has not been established (novel, first-of-a-kind devices); “nonexperimental” devices were defined as those for which underlying questions of safety and effectiveness have been resolved (for example, newer generations of legally marketed devices and other types of “crossover technologies” described by Stanley Joel Reiser). The FDA was charged with responsibility for classifying IDE devices into one of these two categories.

Procedures. A 1994 survey of managed care health plans found that many provide coverage for investigational procedures if they are considered to be very promising in treating life-threatening disease. Payment for experimental therapy provided outside these HMOs is usually made only when the HMO is not able to provide it internally. Some HMOs cover experimental therapy only when it is provided in the context of an approved clinical trial, either inside or outside the HMO.

In other instances, insurers have provided coverage for some experimental procedures but not others. Coverage often has resulted from political pressure. For example, despite multiple technology assessments concluding that the benefits of high-dose chemotherapy with autologous bone marrow transplant (HDC/ABMT) for metastatic breast cancer have not yet been proven, the Federal Employees Health Benefits Program (FEHBP) has decided to require participating plans to cover the procedure. Several states also have passed laws requiring insurers to cover this procedure.

Social Objectives Concerning ‘Unproven’ Technology

Part of the difficulty in developing a satisfactory approach to paying for “unproven” medical technology arises from the need to balance several legitimate but conflicting social objectives. Policies regarding payment for experimental medical technology, for example, influence rates of innovation, timing of access to promising technology, patient safety, and health care costs. Stakeholders with an interest in these issues include the general public, patients and their families, insurers, health care providers, product manufacturers, and researchers. These stakeholders’ interests, which may conflict, also need to be considered in the design of payment policies.

Reasons to facilitate access to experimental technology. (1) Allow earliest possible use of promising new treatments. In circumstances of serious or life-threatening disease and a lack of effective alternatives, patients have an understandably powerful desire to have access to whatever treatments are under development, as long as there is some suggestion that they may work. As was made clear to the FDA by advocates for persons with acquired
immunodeficiency syndrome (AIDS), patients may feel strongly that they are in a better position than federal regulators to decide whether the potential benefits of a treatment are “worth,” the associated known or unknown risks of a treatment.

(2) Support high rates of technological innovation. The rate of development of new technologies is influenced by the strength of barriers to payment for and adoption of those technologies. Unless payment is available for a promising technology, its developer may not have the resources to pursue development, refinement, and marketing of it. As the required level of proof of safety and effectiveness of new technologies rises, the number of new technologies developed is likely to decrease. The availability of reimbursement for experimental technology, in contrast, likely would promote innovation. The desire to promote such innovation is shared by patients, physicians, medical product manufacturers, and society at large.

Reasons to constrain access to experimental technology. (1) Ensure patient safety. One reason to constrain access to new technology is to avoid harm to patients. When a new technology has been used on only a small number of patients, it is difficult to know what risks the new technology presents. A side effect can occur infrequently but can be serious or fatal. The avoidance of harm is important not only to patients, but also to payers and providers who may be subject to liability for illness or injury caused by new technologies.

(2) Produce valid information on benefits and risks. To have sound data regarding the benefits and harms of a particular technology, one needs to conduct well-designed research studies. Such trials typically require that patients who enroll in them be willing to take the chance of being randomly assigned to the nonexperimental therapy. The ability to conduct such studies may require limiting access to the technology outside of the studies. For example, problems have arisen in efforts to recruit women for trials designed to assess the benefit of HDC/ABMT in the treatment of metastatic breast cancer. Because of the availability of HDC/ABMT outside of clinical trials, many women with metastatic breast cancer have not been willing to accept the chance of being randomized to a control group in a trial designed to evaluate the effectiveness of HDC/ABMT. As a result, it has taken much longer than expected to obtain an adequate number of participants in these studies to resolve the uncertainty over the value of this technology.

(3) Control health care costs. Controlled evaluation of new health technologies would help to prevent widespread diffusion of technologies that have no real benefit. The costs associated with widespread use of ineffective technologies that have been adopted without adequate evaluation are likely to far exceed the costs of conducting such evaluations.
Questions To Consider

Several issues need to be addressed in considering potential new policies for payment of experimental technologies.

**Which experimental technologies should be eligible for coverage?** Coverage of experimental technologies could be provided for all experimental technologies or for some subset of them. Many potential technology subsets that would be eligible for coverage could be defined. For example, insurers could provide coverage for an entire technology category (such as experimental drugs, devices, or procedures) or could limit coverage for experimental services based on the nature of the potential clinical benefit the service provides. For example, coverage could be limited based on whether the condition being treated is life-threatening or severely debilitating, whether the experimental therapy is potentially curative or only palliative, or whether the experimental technology is therapeutic or diagnostic. Coverage for experimental technologies also could be limited to circumstances in which all nonexperimental options have been exhausted, or in which nonexperimental options exist but are thought to be less beneficial than an experimental alternative. The main reasons to restrict the scope of coverage of experimental technology based on the nature of the potential clinical benefit provided relate to cost (coverage of a narrower set of services would be less costly) and the problem of moral hazard (persons tend to use more services when they are insured than when they are not insured). One way of cutting down on use of experimental services in situations in which treatment is discretionary would be to exclude such use from coverage. An alternative approach would be to employ a substantial deductible (such as $2,000) for experimental services. Although a high deductible would discourage “discretionary use,” it also could make expensive experimental services unaffordable.

If only a subset of experimental technologies is to be covered, then some entity will need to decide what criteria will be used to define that subset. Such decisions could be made by individual insurance companies and HMOs, or by state or national panels. The advantage of having these decisions made by individual insurers and HMOs is that it presumably would result in multiple coverage options from which persons could choose. On the other hand, many insurers and HMOs lack the resources to determine which experimental technologies might merit coverage. In addition, a decentralized system of decision making would spread the cost of coverage of experimental technologies across a smaller number of consumers, likely increasing the cost and therefore decreasing the accessibility of such coverage. Decisions made at a national level could ensure that all persons have access to promising technologies and would allow assessments to be per-
formed by clinical and methodologic experts. Historically, manufacturers have expressed concern that a centralized technology assessment authority would limit access to potentially beneficial technologies.

At what stage of development should a technology be considered eligible for coverage? One of the more problematic aspects of paying for experimental medical technology is deciding at what stage to provide coverage. The earlier in the development and evaluation process at which coverage is provided, the greater the likelihood of paying for services that ultimately will be found to be unsafe or ineffective, and the larger the share of research and development (R&D) costs that would be shifted from manufacturers to the public or insurers.

Very early in the life cycle of a technology, there is not enough information to judge a technology’s promise. On the other hand, the potential benefits of a technology may never be realized unless clinicians have an opportunity to use it and to suggest improvements. Such a “learning curve” phenomenon applies particularly to many devices and surgical procedures. Over time, safety and effectiveness may improve, and costs may decrease, as experience with the technology increases.

On the other hand, because many technologies undergo continuous evolution, evaluations of them would never be undertaken if one waited for the technology to have matured to a “stable state.” In addition, once a significant number of providers and patients have uncontrolled experience with a technology, they may form opinions about its safety and effectiveness that would make it difficult to recruit patients into a study in which they may be assigned to a group that does not get the treatment.

Clearly, there is no “right” answer regarding the stage of development at which coverage for experimental technologies should begin to apply. Conceptually, however, it seems reasonable for such coverage to be activated when there is evidence that the technology is safe in humans and there is a strong rationale for conducting large, controlled studies of it. Examples of circumstances in which this construct would apply are new drugs and devices that are being evaluated in FDA- or institutional review board (IRB)-approved Phase III clinical trials and new procedures being evaluated in IRB-approved studies after there has been experience with at least ten patients that suggests that the procedure is safe and effective.

Should experimental technology be paid for whether or not the patient is enrolled in a study? To receive coverage for some or all experimental technology, patients could be required to enter a study in which the technology is being evaluated. The benefit of such a restriction is that the impacts and benefits of experimental technology will be known earlier than if data were not collected via an experimental design.

There is a limited window of opportunity in which one can obtain
well-controlled experimental information on such technologies, and that is when legitimate uncertainty exists. During that time it is critical that some mechanism be found to collect data from a representative sample of persons who will be treated with the technology. As evidence about the value of the technology becomes available, it becomes more difficult to convince people to subject themselves to randomization, and hence to risk not getting access to the experimental technology. The result is a delay (or a complete failure) in acquisition of data regarding the benefits of a technology.

**Which costs associated with provision of an experimental drug, device, or procedure will be eligible for coverage?** Costs associated with coverage of experimental services would vary considerably depending on whether the costs of an entire hospitalization are covered or whether only the costs of the procedure (or device) itself are covered. A related issue is whether payment should be made for the actual cost of providing an experimental service, or whether payment should be limited to the lesser of the cost of the experimental service or the cost of some “accepted,” intervention for which the experimental service is substituting.

**Should coverage be restricted to services provided by certain providers?** Coverage of experimental services also could be restricted to selected providers (such as those with the most experience or best outcomes with use of the experimental service). Such a restriction could produce a quality-control benefit and keep experimental technologies from diffusing widely before their benefits have been established. This approach is already employed by some insurers with regard to coverage of highly technical procedures (for example, heart transplants) whose safety and effectiveness in the hands of experienced clinicians have already been established.

**Who should pay for experimental treatment and research?** There are several potential entities who could pay for the cost of use and evaluation of experimental medical technology. Candidate payers for the cost of use include drug and device manufacturers, consumers through purchase of insurance that covers experimental technology, government, persons who use experimental technologies, and health care providers. With regard to potential payers for the evaluation cost of experimental technologies, we consider drug and device manufacturers, insurers, and government.

**Payment for use of experimental technology.** Drug and device manufacturers are logical candidates to pay for the cost of use of experimental products that they have produced, either within or outside the context of clinical trials. The rationale for manufacturers’ paying for such use is that they are the ones who will benefit financially from products that are successful in the marketplace. The cost of using technologies that are still experimental is, in a sense, just another R&D cost, and thus one that eventually will be passed on to the general public in the form of health care costs.
The Health Industry Manufacturers Association (HIMA) has argued that pharmaceutical firms are better able to pay for these costs than are device manufacturers, both because the cost of drugs tends to be lower than the cost of devices and because pharmaceutical firms tend to be larger than device manufacturers. Similar difficulties have been noted by biotechnology pharmaceutical developers, whose products also may be extremely expensive and pose a significant investment burden during R&D. In addition, HIMA has argued that devices tend to evolve incrementally and thus that it is not practical economically for device manufacturers to pay for each “next-generation” device that they develop. Support therefore exists for the FDA to make it less burdensome for device manufacturers to receive approval for slight modifications of approved products, and for HCFA’s new policy of coverage for “nonexperimental,” IDE devices.

Another important issue to consider is that for most procedures, there is no manufacturer. A new surgical technique, for example, may not require any new equipment. Moreover, even if a new device is involved, the cost of that device may be only a small fraction of the cost of the surgical procedure as a whole. This same problem applies to some nonsurgical procedures as well. HDC/ABMT for metastatic breast cancer, for example, employs approved drugs at near-lethal doses in combination with harvesting and administration of a patient’s bone marrow, a procedure to which no manufacturer is directly linked.

A second option is to have the costs associated with use of experimental technologies paid for through health insurance. This option raises the additional question of whether coverage of experimental technologies would be provided through an expansion of the coverage provided under standard policies or through an optional, supplemental policy. There are potential advantages and disadvantages to each of these approaches. One advantage of expanding standard policies to include experimental services is that the actuarial risk would be spread across a large population that includes both high- and low-risk persons. In contrast, optional supplemental policies covering experimental technologies might preferentially be purchased by persons who have serious diseases (a form of adverse selection), leading to an increase in the cost of such supplemental coverage. On the other hand, individuals and employers likely vary in their interest in having (and willingness to pay for) coverage of experimental technologies. By making such coverage optional, insurers would be recognizing variation in purchasers’ preferences and would be able to keep the cost of standard coverage lower than they otherwise could. Mandatory coverage of patient care costs associated with use of experimental technologies in approved clinical trials would have significant cost implications for payers.

A third option would be for the persons who use experimental technolo-
gies to incur the associated costs. Such an approach would enable persons who do not want to use new technologies until their benefits have been established to avoid paying for technologies that ultimately do not prove to be beneficial. On the other hand, most consumers cannot afford to pay for medical services out of pocket, and such an approach fails to capitalize on the risk-spreading benefits of insurance.

Fourth, one could argue that it would be reasonable for government to pay for the cost of experimental medical technology. It is clearly in society’s interest to have new and improved technologies developed, and one could argue that a healthier population would increase economic productivity and tax revenues. One also could argue, however, that if government were to subsidize development of new technologies, then it should share in the profits derived from successful technologies, perhaps through royalties.

A fifth possibility is to have health care providers pay the cost of using experimental procedures. Such an approach assumes that providers earn a profit that can be used to cross-subsidize uncompensated care. While this has been true to some extent, it is becoming less viable as providers compete in the health care marketplace, in part on the basis of the cost of the care they provide.

Finally, multiple parties could share the cost of experimental technologies. For different technologies and different clinical conditions, the optimal formula for who pays may be different. Procedures might need to be supported more by government, while drugs and some devices could be paid for largely by manufacturers.

Payment for evaluation of experimental technology. To know which experimental technologies should be introduced into general medical practice, the safety and effectiveness of those technologies compared with existing alternatives need to be assessed in well-designed clinical studies. There is a cost associated with designing, conducting, and reporting the results of such studies that is quite separate from the cost of providing the experimental technology in such studies. These costs typically have been paid by manufacturers. Again, because there often is no manufacturer with an economic interest in a new procedure (as opposed to a new drug or device), some other entity must pay the cost associated with evaluation of new procedures. The two most logical candidates for paying such costs are government, through research grants, or insurers and HMOs. Some insurers, in fact, have come to view payment of such costs as being in their own economic interest. Their rationale is that it is better to pay for a study that will determine whether an expensive procedure is beneficial than to have that procedure diffuse into use without having its safety and effectiveness established, or to be pressured by the courts to pay the cost of such experimental procedures, with or without punitive damages.
A Proposal For Coverage Of Experimental Technologies

The following proposal provides a mechanism to evaluate quickly and concurrently pay for promising but expensive new technologies. It is based on two assumptions. The first is that providing access to experimental technologies may benefit many individuals and stakeholders; thus, the associated costs should not be borne by only one entity. Second, scientific evidence is necessary to determine technologies’ safety and effectiveness.

Criteria defining technologies meriting special attention. Given limited resources, it may not be possible or desirable to provide coverage for use of all experimental technologies. We therefore advocate coverage for certain “special-priority” technologies, in addition to the nonexperimental IDE devices recently covered by HCFA. A technology satisfying all of the following criteria could be considered “special priority”: (1) Scientific evidence or strong rationale suggests but does not prove that the technology provides substantial net clinical benefit; (2) the technology is used in the management of life-threatening or severely disabling conditions; and (3) no adequate alternative treatments are available. The FDA could be responsible for determining whether a technology satisfies these criteria.

Large, multicenter trials to evaluate “special-priority” technologies. A “special-priority” technology would be studied in clinical trials designed with adequate statistical power to assess its safety and effectiveness. The protocol for the studies would be approved by the FDA. It is assumed that randomized trials would almost always be performed; however, other quasi-experimental methodologies may be appropriate in some cases. Coverage would be provided for patients enrolling in the trials, and access to the technology outside the trial would not be available.

Payment for clinical care and research costs. All public and private insurers and HMOs should be required to cover clinical care costs related to use of a “special-priority” technology, but not the technology itself (Exhibit 1). For drugs, the product would be provided free by the manufacturer. For devices and expensive biotechnology products, payment policy would vary depending upon whether or not the “special-priority” technology is clearly substituting for an approved technology. When such substitution is clearly occurring, insurers (or HMOs) would cover the lesser of the cost of the “special-priority” technology and the approved technology for which it is substituting. (This is similar to Medicare’s revised policy for coverage for nonexperimental IDE devices.) If such substitution is not occurring, then the manufacturer could elect to provide the product at no charge, or payment for the cost of the device or biotech product would be provided from a trust fund established to support clinical research. In the latter case, a percentage of future profits from the product would be contributed to the
## Exhibit 1
Who Should Pay For Clinical Care Associated With Use Of “Special-Priority” Technologies: A Proposal

<table>
<thead>
<tr>
<th>Type of technology</th>
<th>Entity to pay for the technology itself</th>
<th>Entity to pay for care surrounding use of the technology</th>
</tr>
</thead>
<tbody>
<tr>
<td>Drug</td>
<td>Manufacturer</td>
<td>Insurers and HMOs</td>
</tr>
<tr>
<td>Device or expensive biotechnology product</td>
<td>Insurers and HMOs (for products substituting for covered products) Manufacturer or trust fund (for other products)</td>
<td>Insurers and HMOs</td>
</tr>
<tr>
<td>Procedure</td>
<td>Trust fund</td>
<td>Insurers and HMOs</td>
</tr>
</tbody>
</table>

**Source:** Authors' analysis.

**Note:** HMOs are health maintenance organizations.

* If a trust fund is used, the manufacturer would pay a percentage of future profits to the fund.

Clinical research trust fund. For procedures, all clinical care costs would be paid for from the clinical research trust fund.

Research costs would be the responsibility of the manufacturer if a product is involved or would be shared equally by public and private payers and HMOs when no other obvious source is identified. The maximum amount any insurer or HMO would be required to contribute to the cost of patient care and research costs related to these “special-priority” technologies would be capped at an agreed-upon percentage of collected premiums. Payers who do not reach their maximum cap each year would contribute the remainder to the clinical research trust fund.

## Conclusion

Whether intentionally or not, insurers, including Medicare, have paid for some experimental technologies for years. These innovations may never have come to market had payment for them not been provided while data regarding their efficacy and safety were being collected. Increased cost pressure on providers and payers, as well as the ongoing OIG/HHS investigation, threatens to curtail development of new medical devices. Controversy over how to pay for and study experimental technologies goes beyond devices, however, and reflects the absence of a broader policy for ensuring that potentially valuable new technologies can be introduced and tested in a rational, efficient manner, with all relevant stakeholders paying their fair share of related costs. We have articulated several issues to be addressed in development of such a policy and have offered a specific policy proposal. Further attention needs to be given to these issues if U.S. leadership in innovation and patient care is to be maintained.
The views expressed in this paper are those of the authors and do not necessarily represent the views of the organizations with which they are affiliated.

NOTES

4. S. Gleeson, “Blue Cross and Blue Shield Association Initiatives in Technology Assessment,” in Adopting New Medical Technology, 96-100.
7. Federal Register 52 (22 May 1987), 19467.
12. Ibid.
13. The three compendia are the U.S. Pharmacopoeia Dispensing Information (USPDI), the American Medical Association’s Drug Evaluations, and the American Society of Health-System Pharmacists’ AHFS Drug Information.
22. Ibid.