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Investigational Treatments: Coverage, Controversy, and Consensus

*Mary Ader**

INTRODUCTION

It is becoming commonplace. Most readers of this article know someone, a friend or family member perhaps, who has been diagnosed with a terminal disease, who has been told that the only hope of survival is an innovative therapy still being conducted in clinical trials, and whose insurance company will not cover the treatment on the basis that it is still investigational¹ and therefore excluded under the terms of the insurance policy. It is hard to imagine a more distressing situation than having a life-threatening disease with a poor prognosis, yet with a chance of a cure but no chance of coverage. Since many of these innovative treatments are prohibitively expensive—high-dose chemotherapy with autologous bone marrow transplant (HDC-ABMT) or peripheral stem cell rescue, for example, might cost up to \$150,000 or more—many patients feel that they have no recourse but to sue their insurer.

Over the past few years, scores of such lawsuits have been filed, with varying and inconsistent results. Simultaneously, in an effort to provide a more definitive resolution, many state legislatures are debating or enacting legislation that mandates third-party payment for certain investigational therapies. These statutes vary widely in scope and effect.² Therefore, the likeli-

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1. The term “investigational,” for purposes of this article, means that the procedure is under investigation, and that the evidence on safety and effectiveness is still inconclusive. Different insurers use different terms to describe this concept, including “experimental” and “not medically accepted.”

2. Some state statutes mandate coverage of specific procedures for specific diagnoses. For example, Massachusetts, MASS. GEN. LAWS ANN. ch. 32A, § 17A (West Supp. 1996), Minnesota, MINN. STAT. ANN. § 62A.309 (West 1996), and New Hampshire, N.H. REV. STAT. ANN. § 415:18-c (Supp. 1995), mandate coverage of HDC-ABMT for breast cancer. Some states, such as Virginia, VA. CODE ANN. § 38.2-3418.1:1 (Michie 1994), mandate the offer of coverage. Other states, such as Florida,

hood of securing access to innovative treatment is subject to so many variables—benefit language, judicial interpretation, state law—that a patient's prospects of securing such treatment are, at best, unpredictable. This article outlines some of the legal and medical controversies surrounding payment for investigational treatments, and offers some thoughts on how these issues may be resolved more constructively and systematically. Traditionally, health plans have paid only for what works ("mainstream medicine") and have excluded coverage of what is yet unproven ("medical research"). However, in light of this current controversy, perhaps the time has come for health plans³ to reconsider their position and begin to support the quest for scientific evidence by contributing to clinical trials in certain appropriate circumstances. This article explores when appropriate circumstances might exist, and highlights the benefits that would flow from encouraging cooperation and developing consensus among all the parties to this debate.

I. DEVELOPING A RATIONAL OBJECTIVE

The parties to the debate over investigational treatments have many legitimate, and sometimes conflicting, perspectives. First, health plan beneficiaries, faced with life-threatening diseases, expect their health plans to cover anything that may save their lives, including treatment that is still under investigation in research programs. Second, health plans, by contrast, are reluctant to pay for investigational treatments. Rather, they prefer to purchase coverage of medically accepted care, to protect patients from unsafe and ineffective treatments, and to control premium costs for all plan participants. Third, researchers and providers of care, faced with dwindling research revenues from other sources, are keen to acquire third-party payment to help support their medical research. Fourth, courts and state legislatures, perceiving human and societal needs amidst these conflicts, proceed to address these needs in ways that are often inconsistent and at odds with medical science.

FLA. STAT. ANN. § 627.4236 (West Supp. 1996), have established an advisory panel to consider the various interests and make recommendations on coverage. Yet other states, for example, Rhode Island, R.I. GEN. LAWS § 27-18-36.2 (1989), require that all experimental cancer therapies be covered when certain criteria are met.

3. Throughout this article, the term "health plans" is used instead of the individual or group of individuals who maintain a particular position within the health plan. This generic term is used because of the wide range of individuals who may hold the relevant position in various insurance or managed care structures.

The way to accommodate these different but perfectly valid perspectives for the common good is to develop a rational approach to which all interested parties can subscribe. The parties include research institutions, providers, payers, patients, and lawmakers.

To convert the current controversy into consensus, the respective parties will need to adopt the following fundamental principles. First, in the interest of evidence-based medical decision making, the parties must submit to the authority, indeed the supremacy, of scientific data over conflicting legislative, judicial, and journalistic pronouncements. Second, in the interest of quality care, the parties will have to agree that the clinical benefits of the treatment under consideration outweigh the harms. Third, in the interest of fiscal propriety, the parties will need to acknowledge that certain financial constraints attach to the setting of premiums for the benefit contracts issued, and that the terms of these contracts must not intentionally be contorted to cover clearly unintended treatments. For example, a contract covering Phase III clinical trials for diagnosis “X” and treatment “Y” means exactly that: it should not be stretched to include coverage of Phase II clinical trials for diagnosis “A” and treatment “B” as well.⁴ Therefore, developing a rational objective means developing a mutual one, grounded in the common assumptions of scientific integrity, clinical integrity, and fiscal integrity. Before exploring some specific suggestions on how to move the debate from controversy to consensus, it is helpful to understand the current context in which benefit contracts are administered.

II. UNDERSTANDING THE CONTEXT OF BENEFITS ADMINISTRATION

A. *The Need To Set Limits*

In any rational health care system, there are limitations on coverage and on affordability. Health plans are in the unenviable position of having to define and enforce these limits in the face of astonishing technological development, vigilante consumerism, and unprecedented judicial intervention. In determining coverage, health plans are caught in a crossfire: they

4. For example, in *Dodd v. Blue Cross & Blue Shield Ass'n*, 835 F. Supp. 888, 890 (E.D. Va. 1993), the plaintiffs alleged, in essence, that coverage of *certain* clinical trials obligated payment of *all* clinical trials. The court granted summary judgment to the defendant. *Id.* at 892.

must face an insatiable demand for new technology, assure safety and efficacy on a scientific basis prior to diffusion, and contain the costs of covered health care. In order to set reasonable and enforceable limits on coverage as well as capture the benefit of new technology, all parties to this debate must be willing to increase their level of communication and cooperation. Without this cooperative spirit, health plans will continue to define coverage limits through benefit contract exclusions and through their reliance on technology assessments, as described below.

B. *Setting Limits Through Benefit Contract Exclusions*

To assure uniformity, health plans set forth in their plan contract that which is covered and that which is excluded. Two exclusions are relevant to setting limits: one based upon medical necessity and the other addressing investigational treatment. The two exclusions, and the concepts they represent, are sometimes confused and sometimes used interchangeably. However, they serve different, but equally valid, purposes.

Medical necessity exclusions are designed to address the concept of efficiency. They speak to the appropriateness of care and the corresponding level of care for a given diagnosis. For example, the plan administrator may question whether a diagnosis requires six months of acute hospital care. A number of medical necessity lawsuits are in fact length-of-stay disputes. (Many of these cases involve mentally ill patients,⁵ which may suggest that there is more uncertainty over the diagnosis and treatment of mental illness than there is over the concept of medical necessity; the problem may be defining mental illness, rather than defining what constitutes a medical necessity.) Although medical necessity exclusions have been challenged in the courts, it is important that health plans are not intimidated

5. See, e.g., *Salley v. E.I. DuPont & Co.*, 966 F.2d 1011 (5th Cir. 1992); *Hughes v. Blue Cross of N. Cal.*, 245 Cal. Rptr. 273 (Cal. Ct. App.), *review granted*, 755 P.2d 355 (Cal. 1988), *cause transferred by* 768 P.2d 32 (Cal.), *aff'd*, 263 Cal. Rptr. 850 (Cal. Ct. App. 1989), *cert. dismissed*, 495 U.S. 944 (1990). A significant medical necessity case that did not involve psychiatric treatment is *Katskee v. Blue Cross/Blue Shield of Neb.*, 515 N.W.2d 645 (Neb. 1994). The plaintiff had a genetic predisposition to cancer—breast-ovarian carcinoma syndrome. *Id.* at 647. The court found that the plaintiff's condition constituted an "illness" within the meaning of the contract, and, thus, the recommended treatment—radical prophylactic surgery to remove the uterus, ovaries, and fallopian tubes—should not have been denied on medical necessity grounds. *Id.* at 652.

into abandoning the exclusion or relinquishing the concept of efficiency that the exclusion represents.

Investigational exclusions, by contrast, are designed to address the safety and efficacy of emerging technologies. A proper analysis of these issues begins with a review of the status of a particular investigation. The language of the exclusion should contain objective criteria that correlate to the continuing investigation of the technology under consideration. The criteria should answer the following question: Given this patient and this diagnosis, is there enough evidence to establish that this treatment will work? If available, technology assessments, described below, should determine the outcome of this inquiry.

In terms of coverage, many accounts do not want to pay for new technologies that have not yet proven their value. Payers (and their accounts) recognize, however, that even where technology assessments have not been available, certain therapies have slipped into common use because their benefits outweighed their potential to cause harm. This may, on occasion, result in a double standard—some therapies may have been rigorously assessed while others have not. It is clear that there exists the need for both continuing and comprehensive assessment and for adequate financing of promising medical research.

C. Setting Limits Through Technology Assessment

The other method of defining coverage limits is the use of technology assessments to determine what medical care is necessary, safe, and effective. Payers and their accounts want value for their money—they will pay for what works. Technology assessment is one means to this end.

Approximately seventy organizations nationwide perform technology assessments, including such groups as major insurers, medical societies, the federal government, and various research organizations. The key product is a document that summarizes the current state of knowledge concerning the technology in question, including divergent evidence, areas of controversy, and gaps in the knowledge base. It describes safety, efficacy, and appropriate use based on the available evidence. If there is insufficient scientific evidence from which to draw conclusions as to safety, efficacy, and improved health outcomes—such as length of life, ability to function, and quality of life—then payers and their accounts are likely to require the development of such evidence before they will regularly pay for the technology.

In this way, technology assessment addresses not the *cost* of emerging technologies, but, more significantly, *value* for money. The vital role that technology assessment plays in determining sound medical policy has generally been underrated and sometimes even ignored or flouted by courts and legislatures. The goal is to educate the populace about this pivotal means of delivering quality health care. With this in mind, making the process of technology assessment understandable to a wider audience will require a greater effort.

III. THE CONSEQUENCES OF ASSESSMENTS AND EXCLUSIONS

A. *The Impact on the Quality of Care*

When third-party payment is not forthcoming by virtue of an assessment or exclusion, what impact does this have on the technology and on the quality of care? First, payment decisions based on technology assessments and/or benefit exclusions may in fact delay the diffusion of technologies eventually found to be medically appropriate.⁶ Sometimes there are prolonged periods of uncertainty for technologies eventually proven to be beneficial, for example, cochlear implants for children. This occurs even though payers are committed to paying quickly to please their customers, and committed to improving quality. Second, and conversely, the benefits of new technologies may ultimately prove to be small or nonexistent. Here, the decision to pay may promote ineffective or even harmful technologies, for example, thalidomide therapy, bowel removal for epilepsy, the freezing of gastric ulcers, and the Garren gastric bubble. Safety should be the first order of the day.

B. *The Explosion of Litigation and the Lessons Learned*

Finding the right balance is not always straightforward and can be controversial. Currently, a number of technologies seem to fall into a "grey" area of payment confusion and litigation, such as high-dose chemotherapy with autologous bone marrow transplant (HDC-ABMT) for breast cancer, growth hormones for short-statured children, home uterine monitoring to detect early labor, Positron Emissions Test (PET) scans, and radial keratotomy. It is regrettable that every technological advance

6. Given a choice of possible treatments, providers generally opt for the one that the third-party payer reimburses. Therefore, until the newer technologies are proven safe and effective, and hence reimbursable, providers are reluctant to prescribe them.

seems to be accompanied by payment battles, which are often played out in the courts and the media. Surely these are inappropriate forums for determining access to investigational treatments given the nature of the scientific issues that such treatments present. The volume of this litigation clearly evidences the need to develop more collaborative, systematic ways of addressing the payment of investigational treatments.

During the past few years, innumerable patients have sought access to investigational treatments through the courts.⁷ Many of these patients have been successful for a variety of reasons, including the sympathy factor, inadequate and ambiguous contract language, and alleged negligent medical review on behalf of the benefit plan.⁸ As a result of this litigation compelling access to investigational treatments, drafters of benefit plans have tightened their exclusionary language and strengthened their medical review processes.

Nevertheless, the litigation continues. The outcomes—both legal and clinical—are still unpredictable, leaving unanswered some fundamental questions: Should health plans always exclude investigational treatment? If so, how should health plans define “investigational”? How should health plans determine whether a particular treatment meets the definition? To health care payers, it seems that the answers to these questions have been misunderstood by those outside the health care payment business—patients, providers, the media, and the courts.

The cases are instructive on certain key points, sending a clear message to health plans that if they wish to exclude investigational treatments, their investigational exclusions need to contain, at a minimum, four elements: (i) sound criteria for making the decision; (ii) a description of the decisionmaking process; (iii) language that is not ambiguous; and (iv) language that is

7. See, e.g., *Cole v. Blue Cross & Blue Shield of Mass.*, 738 F. Supp. 42 (D. Mass. 1990); *Harris v. Blue Cross & Blue Shield of Tex.*, 729 F. Supp. 49 (N.D. Tex. 1990). For an extensive compilation of these cases, see MARY ADER & NELL LEWIS, INVESTIGATIONAL EXCLUSIONS: A SUMMARY OF THE CASELAW (October 1995) (on file with the *Annals of Health Law*).

8. Where the plaintiff is seeking treatment for a life-threatening disease and the defendant is a deep-pocket defendant such as an insurance company, these facts tend to influence the outcome of litigation, regardless of the contract language at issue. In this “David v. Goliath” context, courts have often found the contract language ambiguous, see, e.g., *Bailey v. Blue Cross/Blue Shield of Va.*, 866 F. Supp. 277, 282 (E.D. Va. 1994), or have concluded that the medical review process was deficient or inadequate in some way, see, e.g., *Bucci v. Blue Cross-Blue Shield of Conn.*, 764 F. Supp. 728, 733 (D. Conn. 1991). See also ADER & LEWIS, *supra* note 7.

sufficient to put the subscriber on notice of what is and is not covered.⁹ This is easier said than done! Courts called upon to review complex medical and methodological questions will construe ambiguity in contract language against payers. Therefore, what is meant by “investigational” needs to be precisely defined in the contract and specifically related to enforceable criteria.

C. *The Development of Criteria for Determining Investigational Status*

Health plans have adopted different approaches to developing standards or criteria for determining investigational status. Some are better than others, but all have fallen victim to judicial disapproval at one time or another. The first approach is to accept the standard of “professional consensus,” a concept capable of various definitions but generally left undefined in the benefit contract. This open-ended approach, however, runs the risk of becoming the consensus of the *relevant* professionals, which in turn may become the consensus of the treatment’s *proponents*.¹⁰ From the payer’s perspective, this is a slippery slope.

A second approach is to refer to and adopt the positions of other respected entities, such as the Federal Office of Technology Assessment, the National Institutes of Health, or the American Medical Association. This approach is also problematic: the entity selected may not have a position on a particular technology; the entity’s position may be unclear, dated, or in conflict with the position of other entities; and the subscriber still may not receive adequate notice of what is or is not covered.¹¹

The third approach is to use scientific criteria, such as those used in the technology assessment program of Blue Cross and Blue Shield Association.¹² This approach recognizes that there

9. See, e.g., *Dozsa v. Crum & Forster Ins. Co.*, 716 F. Supp. 131 (D.N.J. 1989); *Waldrip v. Connecticut Nat’l Life Ins. Co.*, 566 So. 2d 434 (La. Ct. App.), *cert. denied*, 571 So. 2d 650 (La. 1990), *aff’d as amended*, 573 So. 2d 1172 (La. Ct. App. 1991). See also ADER & LEWIS, *supra* note 7.

10. See, e.g., *Dozsa*, 716 F. Supp. 131.

11. See, e.g., *Waldrip*, 566 So. 2d 434. Cf. *Boland v. King County Medical Blue Shield*, 798 F. Supp. 638 (W.D. Wash. 1992) (a plan sponsor’s reliance on a classification produced by an independent third party did not create fiduciary duties in that third party).

12. The five Blue Cross and Blue Shield Association Technology Evaluation Criteria are:

- (1) The technology must have final approval from the appropriate government regulatory bodies.
 - This criterion applies to drugs, biological products, devices and diagnostics.

is a difference between the systematic generation of objective data and authoritative opinion. The latter may prevail in lawsuits in the short run, but it must inevitably yield to the former in the long run.¹³

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- A drug or biological product must have final approval from the Food and Drug Administration.
 - A device must have final approval from the Food and Drug Administration for those specific indications and methods of use that Blue Cross and Blue Shield Association is evaluating.
 - Any approval that is granted as an interim step in the FDA regulatory process is not sufficient.
- (2) The scientific evidence must permit conclusions concerning the effect of the technology on health outcomes.
 - The evidence should consist of well-designed and well-conducted investigations published in peer-reviewed journals. The quality of the body of studies and the consistency of the results are considered in evaluating the evidence.
 - The evidence should demonstrate that the technology can measure or alter the physiological changes related to a disease, injury, illness or condition. In addition, there should be evidence or a convincing argument based on established medical facts that such measurement or alteration affects the health outcomes.
 - Opinions and evaluations by national medical associations, consensus or other technology evaluation bodies are evaluated according to the quality of the supporting evidence and rationale.
 - (3) The technology must improve the net health outcome.
 - The technology's beneficial effects on health outcomes should outweigh any harmful effects on health outcomes.
 - (4) The technology must be as beneficial as any established alternatives.
 - The technology should improve the net health outcome as much as or more than established alternatives.
 - (5) The improvement must be attainable outside the investigational settings.

When used under the usual conditions of medical practice, the technology should be reasonably expected to satisfy criteria 3 and 4.

13. Some courts have decried the health plans' failure to specify the *quantum* of evidence required and have called for specific thresholds of statistical success in terms of cure or survival rates. *See, e.g.,* Bucci v. Blue Cross-Blue Shield of Conn., 764 F. Supp. 728 (D. Conn. 1991); Reiff v. Blue Cross & Blue Shield of Okla., No. 90-C-1030-E (N.D. Okla. Feb. 11, 1991) (on file with the *Annals of Health Law*); Pirozzi v. Blue Cross-Blue Shield of Va., 741 F. Supp. 586 (E.D. Va. 1990). With all due respect to these courts, such a standard—for all diseases, all technologies, and all times—simply could not be met; it would be utterly impracticable even to attempt to embrace this standard in a single health benefit contract. Although these particular courts condemned the lack of standards for determining statistical success, at least one other court, looking at the numbers, took a different view. *Evans v. HMO Colo., Inc.*, No. 91CV3797 (Colo. Dist. Ct., Denver County June 14, 1991) (on file with the *Annals of Health Law*). Here, the plaintiff sought benefits for HDC-ABMT for the treatment of cervical cancer. The court found significant that the procedure had been performed on only six patients at one research facility. It said: “[T]he University of Nebraska is the only one that’s been trying to find out if this is going to work on this kind of solid cancerous form [N]obody knows yet. Everybody agrees that six is too few to make a judgment on.” *Id.* at 11.

For health plans that want to cover only those treatments proven to work, the scientific criteria approach, geared to the systematic generation of objective data, appears to be the superior one. Such plans would specifically exclude treatments performed in Phase I, II, or III clinical trials¹⁴ under protocols that seek to determine safety, efficacy, and toxicity, and seek to compare the investigational treatment with conventional alternatives. These plans would also exclude treatments that call for institutional review board approval and consent to investigational treatment.¹⁵

Regardless of a health plan's approach and objective, its contract language will always be subject to legal challenge on the grounds of ambiguity. Moreover, its medical review process will be scrutinized for timeliness, comprehensiveness, and currency. The burden of vigilance here, the courts say, falls on the health plan.¹⁶ Another problem is the practice of lawyers who focus on how to interpret benefit language, rather than on how to benefit a patient or advance medical science. As a result of this "loop-hole lawyering," clinical issues of safety and efficacy are lost.

IV. A RATIONAL ALTERNATIVE: ACCESS TO CLINICAL TRIALS

Parties to litigation over investigational exclusions often find themselves literally, as well as metaphorically, locked in mortal combat in the courts. When potentially life-saving treatment is at stake, trial courts are likely to rule in favor of the plaintiff/patient. Defendants rarely appeal.¹⁷ Health plans have responded to this in a variety of ways: they have strengthened the general investigational exclusion; they have developed proce-

14. Phase I clinical trials are designed to determine the reactions of the experimental drug or procedure in humans, including side effects, effectiveness, and toxicity. Phase II clinical trials are conducted to evaluate the effectiveness in humans of the experimental drug or procedure on a particular condition or disease, with the goal of gathering data on the effect/benefit that the drug provides. The goal of Phase III clinical trials is to gather large amounts of data on the effectiveness and safety of an experimental drug or procedure; this is usually accomplished by randomly providing either experimental or conventional treatment to human subjects and comparing their outcomes.

15. Institutional review board approval is required for all research that involves the use of human subjects. See 45 C.F.R. 46.109, 46.101 (1996).

16. Contract language does not always contemplate new medical developments. See, e.g., *Bailey v. Blue Cross/Blue Shield of Va.*, 866 F. Supp. 277 (E.D. Va. 1994); *Calhoun v. Complete Health, Inc.*, 860 F. Supp. 1494 (S.D. Ala. 1994); *McLaughlin v. Williams*, 801 F. Supp. 633 (S.D. Fla. 1992).

17. See generally *ADER & LEWIS*, *supra* note 7.

dure-specific exclusions for certain treatments; they have been more selective in those cases they choose to take to trial; and they have offered riders for investigational treatments. All of these responses have minimized, but not eliminated, the element of uncertainty inherent in benefit language interpretation.

What these responses fail to do is answer the fundamental question raised earlier: Should health plans be in the business of excluding all investigational treatment in the first place? Is this a defensible position? Expressed differently, is there a legitimate role for health plans to cover certain kinds of investigational treatment? If so, what might this role be? How should benefit language be drafted? Can health plans develop a "clinical trials benefit" provision?

With these questions in mind, it might be prudent for health plans to begin to think about two categories of investigational treatment, and to distinguish "bizarre" therapies from legitimate medical research. The case law shows that the investigational exclusion is an effective and appropriate barrier to paying for "zany" treatment, such as coffee enemas and tomato therapies.¹⁸ However, for potentially life-saving treatments, the case law suggests a trend that may render standard medical coverage language insufficient to protect against claims for investigational treatments. This trend is most notable when the plaintiff/patient is diagnosed with a terminal illness and, but for the investigational therapy, life expectancy is limited; the investigational therapy provides the only possibility to preserve life; the treatment is prescribed by legitimate providers and performed in leading institutions; and conventional therapy has failed.¹⁹ Even the finest of investigational exclusions and decisionmaking processes will face an uphill battle in court in life-threatening cases if the plan is characterized as asking the court for a death sentence for the subscriber. For these kinds of cases, health plans may wish to consider developing humane, worthwhile alternatives to claim denials. The funding of clinical trials would be a logical place to begin.

18. Requests for payment for these kinds of therapies are often denied by the plan's medical director, without recourse to the courts. For treatments involving apricot pits and vitamin therapy, see *Free v. Travellers Ins. Co.*, 551 F. Supp. 554 (D. Md. 1982) (laetrile treatments and vitamin regimens), and *Henne v. Mutual of Omaha*, No. 81-1359 (D.D.C. Jan. 10, 1983) (on file with the *Annals of Health Law*).

19. See, e.g., *Pirozzi v. Blue Cross-Blue Shield of Va.*, 741 F. Supp. 586 (E.D. Va. 1990) (Stage IV breast cancer).

The adverse publicity and continuing litigation undermine the efforts of many health plans, especially those that are not-for-profit. In addition, these negative forces frustrate all health plans that have in good faith set their premiums for their products based on the actuarial principles applied to the benefits in their written contracts. Moreover, despite misguided attempts by plaintiffs and the press to depict health plans as latter-day Scrooges, the reality is that health plans routinely pay enormous sums for appropriate noninvestigational treatment. For example, HDC-ABMT is routinely covered for those diagnoses for which it has been proven effective—at least six forms of cancer.

Accordingly, the conflict in the courts over contract interpretation is but a symptom of a different and more fundamental problem. The real issue to be addressed is not the enforceability of the investigational exclusion, nor its wordsmithing, but rather how to finance medical research. In earlier days, cancer and heart disease were first in line for relatively abundant research funds, and payers generally did not object to covering the patient care costs of this research. Today, however, the issues are more complex: government resources are subject to budget constraints; new diseases have unprecedented political clout; burgeoning techno-med (or high-technology medicine) consumes an increasing proportion of the health care dollar; and hospitals and physicians look to medical research programs to solve patient census problems and contribute to the bottom line. Health plans are now being tapped to fill the void and, as a result, frequently balk, particularly if the research is in the early stages.

If the cost of medical research is to be shifted to health plans, then two results would follow. First, health care premiums might increase, at least in the short run. Health plans would be covering *additional* care that would not necessarily be *substitutional* in nature. Second, health plans would begin to consider developing constructive payment mechanisms for investigational procedures, and would then steer resources into research studies in a timely manner. For example, they might rewrite their benefit contracts to cover certain forms of investigational treatment when provided in well-conceived clinical trials under specified terms and conditions. Health plans would then be able to pool the resulting data and learn something for the money they expended. This approach would stimulate valuable research and yield useful information for the care of future patients. It would represent a new era of collaboration and cooperation between

health care payers and health care providers. Above all, this way of promoting clinical trials would move the debate of whether a particular treatment works from the legal to the medical arena, where it belongs.

A logical way to begin this process would be for health plans to engage in selective contracting with specified institutions for promising treatments. For example, they might choose to pay for all health care provided in Phase III trials, representing a stage in the research that shows “substantial promise.” This option could permit coverage only when the procedure was performed at specified institutions or institutions that met specified criteria—an “exclusive provider organization.”

Variations on this “exclusive provider organization” theme, and suggested contractual components of it, might include: (i) contributing to controlled clinical trials, (ii) under research protocols (national if possible, to elicit comparable data), (iii) within a designated time frame, (iv) with data analysis, (v) allowing for cost sharing, and (vi) risk sharing. Each of these six components is designed to reinforce the *research and investigational* nature of the procedure at issue. Cost sharing and risk sharing might take a variety of forms: two way, between the health plan and the health care provider, or even three way, among the plan, the provider, and the patient/subscriber.

One example of such an approach is the Blue Cross and Blue Shield System’s Demonstration Project for HDC-ABMT treatment of breast cancer, currently sponsored by Blue Cross and Blue Shield Association (BCBSA) in conjunction with the National Cancer Institute (NCI). The Blue Cross and Blue Shield Plans expect to contribute approximately \$40 million to this randomized controlled clinical trial over the next several years. The demonstration project supports a series of four national Phase III trials. The NCI trials are intended for the eventual enrollment of 1500 women and are currently being conducted at eighty-six medical research institutions. The BCBSA has contracts with forty-two of these institutions. Clinical trial cooperative groups—voluntary associations of cancer research institutions that conduct clinical trials executed uniformly among member institutions—designed and approved the research protocols governing each of the NCI trials. The NCI approved the research protocols. In addition, the trials are

randomized²⁰ so that the outcomes of the treatments can be scientifically compared. This demonstration project should be completed in 1998.

Another example of this kind of contracting is the BCBSA multiple myeloma trial program. This program, modeled after the breast cancer demonstration project, includes unique contracts with a network of qualified institutions, access to trials that are approved by a national research organization, a fixed contribution rate negotiated with each institution, and a centralized patient management and tracking system.

A third example is BCBSA's response to the September 1994 federal government mandate that all carriers in the Federal Employee Health Benefits Program cover HDC-ABMT or peripheral stem cell rescue for three diagnoses: breast cancer, multiple myeloma, and epithelial ovarian cancer. The BCBSA developed a "Clinical Trials Benefit," which covers these services along with allogeneic bone marrow transplants for multiple myeloma when performed in a clinical trial.

Clinical trials are the only way to both resolve the relative efficacy of new technologies and ensure that the patient is being treated as safely as possible by the most competent physicians. If there *is* a point at which medicine, economics, common law, and common sense intersect, then the funding of clinical trials is that point. There *are* periods of scientific uncertainty. These issues can only be resolved scientifically, if at all. Orders or mandates of payment from courts, states, and accounts are not responsive to this dilemma.

One final issue remains: How should we select clinical trials that are worth financing and incorporating into the health care benefit? Not all clinical trials are of the same value. Two principles should guide the selection. First, the trials selected should be those that undergo the most rigorous protocol review process and receive thorough study oversight. For HDC-ABMT treatment of breast cancer, for example, the most strenuous level of protocol review and oversight is received by NCI-sponsored therapeutic clinical trials conducted by the Clinical Trials Cooperative Groups. Trials approved by other NCI programs or other research entities recognized by the NCI for Cancer Center support grants could be ranked second in importance for health plan consideration. Trials approved only by a hospital institu-

20. Randomized trials are ethical only where the researchers do not know whether the experimental therapy, the standard therapy, or either is superior.

tional review board and funded by the institution or pharmaceutical companies or other local sponsors should be ranked lowest in importance for health plan consideration.

The second guiding principle is that patients should always be treated in the highest phase of a trial for which they are eligible, since this is the phase most likely to yield a positive health outcome. Within the hierarchy of trial phases, Phase III multicenter controlled trials that compare investigational agents with standard therapy should receive the highest priority for health plans. Phase II trials, which determine antitumor activity of agents generally in single-site trials, should be ranked second in importance to Phase III trials for health plan support. Phase I trials, which attempt to establish therapeutic effect and safe dosage, should generally be supported by the research sponsor and not by payers.

CONCLUSION

Some may argue that to embrace this approach in a benefit contract would cause health care insurance premiums to skyrocket. While premiums may well increase in the short term, health plans will realize savings in the long run. Research results will reveal ineffective procedures and open avenues to effective patient treatments. As a result, health plans will no longer pay for ineffective treatments and patient care will be more efficacious. It is thus more productive for health plans to steer their members into well-conceived clinical trials than to remain at the mercy of judicial or legislative mandates. For the present, this seems the best hope for converting coverage controversy into coverage consensus. In the future, it may yet come to pass that a health plan's "clinical trials benefit" provision will be the touchstone of quality for the entire health benefit contract.

Additional Resources

The following resources are quite helpful in understanding and exploring the issue of paying for investigational treatment.

John H. Ferguson et al., *Court-Ordered Reimbursement for Unproven Medical Technology: Circumventing Technology Assessment*, 269 JAMA 2116 (1993).

Mark A. Hall & Gerard F. Anderson, *Health Insurers' Assessment of Medical Necessity*, 140 U. PA. L. REV. 1637 (1992).

Angela R. Holder, *Funding Innovative Medical Treatment*, 57 ALB. L. REV. 795 (1994).

David C. Hsia, *Benefits Determination Under Health Care Reform*, 15 J. LEGAL MED. 533 (1994).

Barbara A. Fisfis, Comment, *Who Should Rightfully Decide Whether a Medical Treatment Necessarily Incurred Should Be Excluded From Coverage Under a Health Insurance Policy Provision Which Excludes From Coverage "Experimental" Medical Treatments?*, 31 DUQ. L. REV. 777 (1993).

Richard S. Saver, Note, *Reimbursing New Technologies: Why Are the Courts Judging Experimental Medicine?*, 44 STAN. L. REV. 1095 (1992).

Denise S. Wolf, Comment, *Who Should Pay for "Experimental" Treatments? Breast Cancer Patients v. Their Insurers*, 44 AM. U. L. REV. 2029 (1995).