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LEGAL RESOLUTION OF DENIAL OF ACCESS TO MEDICAL TECHNOLOGY

I. INTRODUCTION

The rapid expansion of technology has drawn the courts with increasing frequency into areas where their experience is limited. Issues surrounding the decisions of administrative agencies involving the application of technologies such as nuclear energy and deoxyribonucleic acid recombinant technologies continue to be widely debated. Recognition of the court's role and limitations in proscribing applications of developing technologies is not new to science,¹ the judiciary,² or the legislature.³

Solutions to the democratic control of technology have ranged from Kantrowitz's suggestion that a "Science Court"⁴ be created to resolve technologic disputes, to the creation of positions for scientific advisors to sit with judges and advise them regarding the scientific aspects bearing on a particular decision.⁵

Although the question of financial constraints and the relevant application of medical technology have been widely debated by economists, and health planners in medical,⁶ lay,⁷ and legal publications,⁸ it is only recently that these issues have been raised in the context of an individual patient. Recent appeals on national network television for funding of liver and bone marrow transplantation for individual children have raised the issue to its quintessential form: Why are individual patients being de-

1. Kantrowitz, *Controlling Technology Democratically*, 63 AM. SCIENTIST 505 (1975).

2. Bazelon, *Coping With Technology Through the Legal Process*, 62 CORNELL L. REV. 817 (1979).

3. 113 CONG. REC. 15, 256 (1967).

4. Kantrowitz, *supra*, note 1.

5. Leventhal, *Environmental Decision Making and the Role of the Courts*, 122 U. PA. LAW REV. 550 (1974).

6. Enthoven, *Consumer-Choice Health Plan*, 298 N. ENGL. J. MED. 650 (1978).

7. Dept. of Health, Educ. and Welfare, President Carter's National Health Plan Legislation; Detail Fact Sheet 5 (1979).

8. Hamilton, *Perspectives on Health Planning: A State View*, 3 J. LEGAL MED. 535 (1982); Note, *Catastrophic Health Insurance and Cost Containment: Restructuring the Current Health Insurance System*, 6 AM. J. L. & MED. 83 (1980).

nied access to life-sustaining, possibly lifesaving, medical technology?

The media has presented the issue as simply one of economics, but it is more than that. In the majority of cases, patients being denied access to medical technology have private or publicly funded health insurance. While the basis of a medical center's refusal to treat the patient is entirely economic, such a refusal derives from a third-party payer decision to deny coverage. The purported basis for the denial of insurance coverage is not cost, but rather that the technology or the particular application in question is *experimental*. The essence of the controversy is: When is a particular technology or application no longer experimental?

Although these controversies require legal resolution, Justice Bazelon, discussing the need for administrative support, warned that the "substantive review of mathematical and scientific evidence by technically illiterate judges is dangerously unreliable."⁹ The satisfactory resolution of specific controversies, as well as the generic issue, will require a judiciary and bar conversant in the language of scientific medicine and familiar with the rudimentary principles of the scientific method. The latter need only comprise a basic understanding of the collection of scientific data, bias exclusion through controls and "blinding," and the statistical analysis of results.¹⁰

The legal issues involved in technology application are inextricably interwoven with the medical issues. This Comment will first discuss the general principles of the scientific method and then contrast their application to pharmacologic therapy with their application to technologic treatments. Bone marrow transplantation will then be analyzed in depth, illustrating the medical issues that third-party payers consider in coverage decisions. Continuing with the model of bone marrow transplantation, this

9. *Ethyl Corp. v. E.P.A.*, 541 F.2d 1, 67 (D.C. Cir.) (en banc), *cert. denied*, 426 U.S. 941 (1976).

10. A detailed review of statistics is beyond the scope of this Comment. Two of the current controversies in medicine turn on the adequacy of study design and statistical analysis. These controversies have not been resolved five and thirteen years after publication of the original work. See Relman, *The Anturane Reinfarction Trial: Reevaluation of Outcome*, 306 N. ENGL. J. MED. 1005 (1982); Finestein, *Clinical Failures and Fallacies of the UGDP Study*, 19 CLIN. PHARMACOL. THER. 78 (1976).

Comment will conclude with an analysis of legal solutions to the denial of access to medical technology.

II. MEDICAL BACKGROUND

Legal relief for a patient denied access to medical technology will require a demonstration that the particular technology or application is not experimental. This issue is unique to medical technology and has not come before the courts in litigation over medical pharmacologic treatments because of several differences in the manner in which a new drug is developed. The Federal Drug Administration (FDA) does not permit a new drug to be marketed until it is proven efficacious. The process is rigorous and FDA approval of a new drug or application usually assures physician acceptance. There is no similar regulation of medical technologies and, as a consequence, they may be widely used before any agreement is reached in the medical community as to the efficacy of the technology. Furthermore, the evaluation of a new drug is funded by the manufacturer. Thus, if a patient and his physician decide the benefit of treatment with an experimental drug justifies the risk,¹¹ that patient may receive such treatment at a center which is evaluating the drug without charge to third-party payers for the experimental treatment. Consequently, litigation concerning experimental medications has been limited to attempts to force FDA approval of drugs which are in general use in other countries, but which have not been adequately studied in the United States.

Finally, the political pressures of the 1970's greatly influenced the development of nascent biomedical technologies. Since the 1960's, Congress had provided seemingly unlimited funding for biomedical research. In the early 1970's, however, concerns arose over rising health care costs. Critics were quick to point out that, statistically, these increased costs were not reflected in improvements in health or health care. The biomedical research community responded to the critics with the explanation that their discoveries were not being utilized sufficiently by the medical community. Therefrom sprang the soon-to-be-cliched concept of "technology transfer." That term of art represents the idea that if the advances of biomedical research could

11. Both the benefit and the risk, of course, may be unknown early in the investigation of a drug.

be disseminated to the practicing medical community, the benefits soon would be reflected in improved national health statistics. Even at the onset, testimony before congressional committees warned against the introduction of inadequately proven technologies and their expansion from areas of known efficacy into unproven areas.

Nevertheless, on November 8, 1978, President Carter signed Public Law 95-623 which created the National Center for Health Care Technology (NCHCT) with the express purpose of intensifying assessment of technological applications and the avowed goal of disseminating their benefits. This created great pressures in the academic biomedical research community to validate their claims (if not their very existence) by pushing forward with the clinical application of new technologies.

These differences in the development of a drug and a medical technology are at least partially responsible for the different criteria utilized in denominating a treatment as experimental. If one is trying to decide whether a medication is experimental, it is only necessary to establish that the drug has been approved for the particular usage in question by the FDA. A drug so-approved will have undergone a rigorous application of the scientific method and therefore will have been conclusively shown to be effective. On the other hand, a new technology is not subjected to any regulatory process and thus there is no convenient line drawn between experimental and non-experimental technologies. Physicians and third-party payers, therefore, must assess the technology by other means. The approach may vary, but must, by lack of any alternative, consist of a scientific assessment of the medical literature or solicitation of expert opinion. A working understanding of the scientific method is required to evaluate and utilize medical opinion and testimony as well as the validity of the third-party payer's denial of coverage.

The scientific method in its classic form¹² is often not strictly adhered to in therapeutic trials because many physicians consider it morally unjustified to repeat a successful trial. The principles of the scientific method are adhered to by FDA man-

12. (1) Observation; (2) hypothesis; (3) experiment; (4) result; (5) conclusion; (6) repeat.

date in development of a new drug in the laboratory. A review of the laboratory development of a new drug will facilitate understanding of later applications of these principles to complex human situations.

A prospective new medication is investigated because of a possible advantage it offers over an existing treatment. The pharmaceutical company may begin development of a drug for various reasons. Typically, elimination of a troublesome side effect of a known effective drug is sought through chemical modification of a parent compound. In other instances, a new discovery elucidating a basic disease mechanism will trigger extensive basic research aimed at altering the newly discovered pathogenic mechanism. Equally as frequently, economic considerations will cause a pharmaceutical company to seek to modify a parent compound known to be effective so that it might market the drug under a different name and share in the profits, absent the expense of development. Uncommonly, a chance discovery will result in the recognition of a new drug with significant efficacy.¹³

By whatever pathway development begins, the new drug will undergo extensive laboratory evaluations.¹⁴ The concept is to test the drug in a group of animals which are exactly like a control group of animals. The compound to be tested must be the only variable — the animals must otherwise be treated exactly alike. The care with which this is done might surprise the non-scientist. The animals, for example, must be handled the same number of times in the same way, be in the same part of the laboratory so as to guarantee equal environments and be fed the same formula. In the laboratory the comparability of the two

13. Rosenberg, *Inhibition of Cell Division in E. Coli by Electrolysis Products from a Platinum Electrode*, 205 NATURE 698 (1965).

14. Initial testing of the compound by the chemist for stability, purity and reliability of production culls many drugs from production. Once a satisfactory drug can be produced in satisfactory form, it is subjected to testing in laboratory animals. A drug never progresses beyond this stage in development if it does not produce the desired effect in the laboratory animal. Here is the first of many opportunities for the well-intentioned and competent investigator to go astray. Whether the animal model is meant to be normal, malnourished, stressed or diseased (so rendered by in-breeding, environmental manipulation or surgical removal of a vital organ) it remains a model. Many normal biologic systems are known to function differently in animals and humans. Many others are not known to function differently — but certainly do. Penultimately, many disease models, whether produced by line breeding of strains of diseased animals or by surgery, are manifestly imperfect models.

cohorts, or groups, is assured by a series of disarmingly simple techniques. The animals are randomly assigned by number to the control or treated group. This is done on an even-odd basis, a simple draw (treatment or no treatment) for each animal, or occasionally by computer. Thus, in the animal, the goal of uniformity of treated and untreated population is relatively easily achieved. This principle of *randomization* is a theme which we will have occasion to reexamine below.

The uniformity of samples is further assured by the techniques of "blinding." This process is meant to assure that no bias — either conscious or unconscious — enters the experiment. The treatment is usually packaged in an identical form with an inactive compound so that laboratory personnel will not be aware of which animals are being treated. If both the subject and experimenter are unaware of which animal is a control and which is treated, the experiment is denominated "double blind."

A moment's reflection will reveal the difficulty inherent in assuring the study is "blinded." What if the treatment causes a physiologic change in addition to the effect under study? For example, if the heart rate is slowed, it will be impossible to maintain the blinded nature of the study. What if the compound or its breakdown product has a characteristic odor? This latter problem has rendered suspect all dimethyl sulfoxide research¹⁵ and evaluation of the antiemetic effect of marijuana.

After strictly comparable control and treated groups are assured by random selection and blinded cohorts,¹⁶ the data generated by the experiment must be evaluated. Many studies which are well-designed from the outset are made valueless or misinforming because of errors made at this point.

In strict statistical theory, only the data necessary to prove or disprove the hypothesis should be evaluated.¹⁷ Good scientific procedure, however, dictates that large numbers of variables be recorded. Good scientific practice allows one to take any unex-

15. David, *Pharmacology of Dimethyl Sulfoxide 6544*, 12 ANN. REV. PHAR. 353, 371 (1972).

16. Such an investigation is termed a 'prospective, randomized double-blind study' and is generally agreed to be the format least subject to error.

17. SWINSCOW, STATISTICS AT SQUARE ONE (1980).

pected results and design new experiments to see if the results are reproducible. Although this is relatively simple and usually done in the laboratory, the expense of human studies often leads to premature publication in which such "results" — unexpected observations made during the course of an experiment — are reported as fact. For example, a study may fail to show an expected decrease in blood pressure, but the treated group's cholesterol may fall. The investigator will then report the treatment effective in lowering cholesterol. A variation of this is the technique of dividing the treated and control groups into numerous subclasses after the results are tabulated in an attempt at isolating a subgroup for which the treatment is effective (or a subgroup to delete so that the treatment will appear effective for the remainder).¹⁸ This technique is not statistically valid. The fallacy is explained by simple probability. In a rudimentary way, if one examines enough subgroups, the odds are that by pure chance a subgroup will eventually be found which shows (or appears to show) an advantage to the treated group.

Should the investigator escape this temptation to select data, there are many other statistical pitfalls for the unwary. To begin with, as will be further explored below, the investigator must design the study with some statistical forethought. If the new medication the investigator is evaluating would be worth manufacturing if it were ten percent better than an existing drug, the number of animals in each group would have to be large enough to demonstrate such a relatively small difference with certainty. Many studies suffer from study groups which are too small to demonstrate the difference the investigators sought.¹⁹ This error (known as a Beta/Type II error) produces a result which shows no benefit when there may actually be one. A different and more pernicious error is the confusion of biologic and statistical significance.²⁰

18. Salzman, *Aspirin to Prevent Arterial Thrombosis*, 307 N. ENGL. J. MED. 113 (1982).

19. Freiman, *The Importance of Beta, The Type II Error and Sample Size in the Design and Interpretation of the Randomized Control Trial*, 299 N. ENGL. J. MED. 690 (1978).

20. For example, a diet might be shown to reduce the cholesterol from 285 to 280 milligrams percent in the treated group. A fairly large sample size would show this to be a statistically significant difference. In fact, if a large enough sample size were selected it could be shown to be "very highly statistically significant" (probability = less than .0001; that is, less than 1/10,000 likelihood that the result was chance). On the other

A fourth statistical pitfall (in addition to data selection, inadequate sample size, and confusion of biologic and statistical significance) is the assumption of inadequate sample size. This is common in the laboratory evaluation of medications, but epidemic in the evaluation of new technologies. Here the investigator uses a statistical analysis which shows that the treatment is not statistically better than the control group. The statistical analysis is then juxtaposed to the raw data which, especially in small study sizes, may result in the impression that the treatment is remarkably effective.²¹ The investigator then presumes that the result of treatment which "approached statistical significance" would have been statistically significant if the sample size had been larger.

The requirements for good research are easily met when the drug is being studied in the laboratory. Human study, however, is more demanding. We will continue to trace the development of a hypothetical drug through human testing to demonstrate the process that a technology would need to undergo to be deemed efficacious. The next phase of the evaluation is usually a human study for toxicity. The major pitfall here is the chance that a rare, but very serious, side effect will be missed because of the relatively small sample size. The thalidomide story²² is a particularly disturbing example of this phenomenon. The FDA

hand, the biologic significance of reducing the cholesterol five milligrams percent is certainly zero. In addition, it is likely that the treatment was associated with at least some undesirable side effects. Even more subtle, and therefore more pernicious, is the technique of reporting results as a percentage reduction in mortality. The distorting power of this technique is multiplied if the duration of the investigation is short. Sackett, *Bias in Analytic Research*, 32 J. CHRONIC DIS. 51 (1979), criticized in Pickering, *Treatment of Mild Hypertension and the Reduction of Cardiovascular Mortality: the 'Of of By' Dilemma*, 249 J.A.M.A. 399 (1983).

The example given above is a fairly apparent one. The error is often compounded by a further assumption illustrated by the slight lowering of cholesterol values in the example. It has never been conclusively shown that lowering cholesterol by any amount is beneficial. Thus an insignificant reduction based on an unproven assumption (that lowering of cholesterol will decrease the chance of an individual having a myocardial infarction) is given legitimacy by an impressive degree of statistical significance.

21. This is often described by authors as "almost statistically significant"!

22. Smithells, *Thalidomide and Malformations in Liverpool*, Vol. i LANCET 1270 (1962).

The British drug thalidomide is a tragic example of a drug with a rare side effect not uncovered until after marketing. This drug was widely prescribed in the United Kingdom during pregnancy and not until after its release did it become apparent that it could cause severe birth defects. Some 250 children were affected before the connection was established.

currently recommends post-marketing surveillance²³ of newly marketed drugs to mitigate this problem.

The next phase of evaluation has the greatest opportunity for erroneous results. After toxicity studies established the safe dose of the drug in humans, it must be evaluated for efficacy. The evaluation for efficacy in humans requires the same care to ensure valid (a scientist might say reproducible — recognizing that the truth changes) results.

When dealing with humans who suffer from a disease, investigators often fail to take the requisite first step to guarantee that the control group and treated groups are uniform — that is, strictly comparable. They may fail to include a control group. If a control group is used, it may not be selected in a randomized prospective double-blinded fashion, but rather an attempt is made to compare the treated group to another set of patients alleged to be controls.

This comparison often involves the use of “historical controls.” The investigators select a group of patients previously described and compare the results in the currently treated group with the results reported in the previous paper. The opportunities here for erroneous conclusions are legion. The previous group of patients may have been different in a variety of ways. The historical group may have been more or less sick or younger or older, for example. On the other hand, the supportive care may have improved with time, leading to improved outcome unrelated to the treatment being evaluated. Surprisingly, even the natural history of the disease may have changed.²⁴ Although

23. See Melmon, *Drug Interactions and the Prepared Observer*, 304 N. ENGL. J. MED. 723 (1981) for one example of such surveillance.

24. Panem, *Interferon and The Cure of Cancer*, 249 ATL. MONTHLY (December 1982), at 84.

Much of the initial enthusiasm for Interferon can be traced to studies in the early 1970's done at the Karolinska Institute in Stockholm on patients with osteogenic sarcoma. Eighty percent of the young adults with this bone cancer were known to develop metastatic lung lesions within one year and subsequently to progress to death, in spite of aggressive amputation undertaken as primary treatment. Interferon was studied as an adjuvant treatment with the hope that it would stimulate the patient's immune system to destroy the cancer cells which remained after the original amputation and thus prevent clinically apparent lung metastasis. Only forty percent of the patients treated developed lung metastasis. Thus, Interferon was launched on its relatively disappointing promotion against recommendations by a panel of National Cancer Institute scientists who

rarely recorded, the halls of academia ring with discussions deriding the ability of a particular group of physicians as an explanation of differences in results. This is particularly true when surgical techniques are evaluated or when medical and surgical techniques are contrasted.²⁵

These rigorous standards are often met in the trial of a drug — especially if it is being tested in a setting where there is no other effective treatment (the patient is allergic to the drug or other known treatments have failed, for example). Such standards are rarely met in surgical or technologic applications, however. Some of the great physicians and medical tragedies of our time²⁶ have been joined by the failure of adequate control.

cautioned that only *historical* controls had been used. It was subsequently shown at the Mayo Clinic that the natural history of the disease had changed and “only” forty percent of the patients with osteogenic sarcoma treated by amputation alone develop lung metastasis at one year.

25. The validity of historical controls is a highly controversial subject of ongoing debate and a lengthy discussion is beyond the scope of this review. To be certain, the only clear choice is the prospective randomized double-blind study; to be fair, the pressures are great to lower this standard. Not the least is the argument that it is unethical to have a control group of ill patients not receiving some treatment. If the situation permits, a previously known treatment felt to benefit only a small proportion of patients or to be only slightly beneficial to the majority of patients can be selected for the control group. This is probably better than no concurrent control, but is subject to serious problems. One nightmare is that each treatment, as it is compared successively to the previous ones, will show some benefit or at least be “as effective” without the benefit of the first treatment having been adequately proven. This can result in many comparisons and, years later, in an accepted treatment never having been demonstrated to be more effective than a placebo. Prout, *Carcinoma of the Bladder, 5-Fluorouracil and the Critical Role of a Placebo*, 22 *CANCER* 926 (1978). Proponents of strict controls are quick to point out that this problem could not arise if all treatments were evaluated in rigidly controlled studies from the outset.

Another method of avoiding the ethical dilemma of the untreated (or placebo treated) control, with many pitfalls for the unwary, is the technique of selecting matched controls. This technique is frequently used in large epidemiologic studies seeking to associate an undesirable side effect with a particular treatment. Chamblers, *Statistical Methods in the Study of Toxic Shock Syndrome*, 96 *ANN. INTERN. MED.* 912 (1982), included in Todd, *Toxic Shock Syndrome: A Perspective Through the Looking Glass*, 96 *ANN. INTERN. MED.* 830 (1982). The group to be evaluated is often not selected by the treatment given, but rather by the outcome *retrospectively*. These patients are then asked about a particular exposure (environmental or therapeutic) and the frequency of the particular exposure compared to exposure in another matched group. The validity of this technique depends on how closely the comparison group is matched, how carefully the questions asked are administered, and the problem of a kind of selection bias (the natural tendency to “remember” an exposure felt to be causative either by the investigator or the subject).

26. Henderson, *Cancer of the Breast The Past Decade*, 302 *N. ENGL. J. MED.* 17, 22 (1980).

Presuming a carefully designed study with appropriate controls, bias exclusions and adequate numbers to detect relatively small benefits, the elements of analysis of data and conclusion remain in our examination of the application of the scientific method to medical treatments. The scientist in the laboratory relies on reproducibility and the "fit" with other scientific knowledge to insure the validity of the conclusion he draws from a particular investigation. The physician investigator, understandably driven to find a treatment to prevent more human suffering, is usually unwilling to subject his hypothesis that a given drug or technologic application is efficacious to repeated study to verify his results. He consequently often resorts to less trustworthy research methods.

The most notorious is the publication of his results as a letter to the editor or preliminary communication intended to spur other physicians to validate the result through formal investigation. More often than not, these preliminary findings are pressed into clinical practice without the sought-after confirmation. A more acceptable way to validate results without repeated studies is through statistical evaluation.

A simple discussion of the student's T-test as applicable to medical studies is all that need concern us here.²⁷ The student's T-test involves examination of the null hypothesis.²⁸ In order for

Radical mastectomy is a particularly tragic example of a surgical treatment which was widely applied without controlled study. Halstead, the great surgeon, began employing the radical mastectomy fifty years ago because of the widely held concept that the high recurrence rate of breast cancer was the result of local spread. Largely because of Halstead's skill and reputation, this mutilating technique has been applied to generations of women without adequate study. Today most investigators are convinced that such extensive surgery is unjustified in most situations.

27. As a background, however, it is worth briefly exploring the complexity of statistical analysis. Medical schools are currently teaching basic statistics to their students. It is common for major medical journals to utilize statistical reviewers before accepting scientific articles for publication. It is equally common for major scientific studies to be specifically funded for statistical help in the design and analysis of the planned research. Nevertheless, many of the current controversies in medicine are being fought out in the statistical arena while frustrated clinicians and authors stand in the wings. (*See supra*, note 10.)

28. In order to determine whether the results of a treatment and the control group are different, one tests the *null hypothesis* that there is no difference between the results of each group. The issue is what is meant by "no difference." Chance alone will result in some difference. When results are normally distributed, about five percent in a single population will by chance alone be outside the range of two standard deviations from the mean. Thus, if we by definition set the limits within which we regard the results as not

a result to be accepted, it should be "statistically significant," that is, have less than five chances in a hundred of being the result of chance (probability = less than 0.05). This means that a result accepted by the majority of physicians as effective still could be purely chance in 5/100 instances.

The complexity of statistical arguments and knowledge of past medical treatments which have come and gone in spite of statistical "proof" of efficacy often leads physicians to two diametrically opposed conclusions. The first conclusion is that the treatment had more responders than did the control. Therefore, even though there is no "statistical difference" between the two, the treatment is "better," "efficacious," or "approaches statistical significance." Paradoxically, the other extreme is equally common. The critical physician "knows" that "statistics can be used to show anything" and doesn't believe for a moment that results which have shown a treatment to be efficacious have any meaning in "clinical practice." A little reflection will demonstrate how the same attitude can lead to the opposite conclusions. In the first instance, the same physician who accepts an almost "statistically significant" result can reject a result that is statistically significant because it is "barely" so. He will be quick to point out that if one or two fewer patients had shown a benefit from the treatment the results would not have been significant. Conversely, the physician who rejects a statistically valid

having any significant difference at twice the standard deviation, a value outside that range will be different ninety-five percent of the time and the same five percent of the time. This is written "p 0.05" and denominated as statistically significant.

The standard deviation is easily calculated by formula (the theoretical basis of which need not concern us here). One can then use the standard deviation of each sample (the treated group and the control group) to calculate the Standard Error of the Difference (S.E.D.). If one compares the observed difference of the means and determines how many multiples of the S.E.D. it is, all that remains is to look up this number in a probability table. For example, if the observed difference is 1.96 times the S.E.D., reference to a probability table will show that there is a 1:20 chance that the observed difference is due to chance. This is written "p 0.05." The principles as laid out are simple enough but the variations are trying. Suffice it to say, there are innumerable variables which mandate statistical consultation in the design and evaluation of a study.

The p values of tables are accurate in large sample sizes greater than 100, but if the study concerns small numbers of less than sixty there is much more chance of random variation and the T-test must be used. W. S. Gosset first published this test under the pseudonym "Student" (*see infra*, note 74) so that it is often known as the Student's T-test. For practical matters one must only look up the value in a t table instead of a p table. A review of such a table would show that as the sample size becomes smaller a much larger difference is required for a difference to be significant. *See supra*, note 17 at 1-42.

result because statistics can be misused may choose to accept a statistically invalid result for the same reason.

This brief view through the looking glass is not intended to lead to an analogy with Alice in Wonderland, but rather to shed light on the medical community's decision-making process. As a result of the vagaries of human experiments, experimenters, and statistical analysis, most physicians have returned, perhaps unconsciously, to the precepts of the scientific method. Before accepting a new treatment they would like to see results validated by further studies — ideally by competitive groups so that critical analysis can be assured.²⁹

Adherence to the scientific method is more difficult when complex technologies are applied to serious diseases. Patients and their families facing a potentially fatal disease are particularly susceptible to societal beliefs in the power of technology, making a single case report adequate proof to a desperate patient and, sadly, often to their physician. Furthermore, the problem in blinding studies of technologic treatments can be insurmountable, although sham surgery and sham technologic applications have been utilized as controls. Bone marrow transplantation is the archetype of a technologic application to potentially fatal diseases and is, therefore, an ideal focus for detailed evaluation.

III. BONE MARROW TRANSPLANTATION AS A MODEL

Bone marrow transplantation has recently come to public attention through the media because of its apparent curative potential in certain diseases and the denial of coverage for the procedure by third-party payers. The introduction above to the scientific method and its application to biomedical research serves as an appropriate framework through which to examine the applicability of bone marrow transplantation and the denial of patient access. The general categories of patients with illnesses theoretically amenable to treatment by bone marrow transplantation include several major categories of disease. They comprise diseases of bone marrow failure (aplastic anemia), malignant diseases of the bone marrow (principally leukemia), other sensitive malignancies treated with lethal doses of chemotherapy with the

29. Relman, *Lessons from the Darsee Affair*, 308 N. ENGL. J. MED. 1415 (1983).

intention of "rescue" by bone marrow transplantation, radiation accidents, congenital disorders of the red cell (such as thalassemia or sickle cell disease), certain rare immunodeficiency disorders, and even rarer metabolic disorders.

A brief description of the procedure of bone marrow transplantation is necessary to place the medical-legal discussion in context. The threshold requirement is the availability of a compatible donor. Although bone marrow transplantation was first reported in 1957,³⁰ marrow engraftment was only temporary. In that same year, French investigators failed in a dramatic attempt to rescue six victims of a radiation accident.³¹ The occasional patient in whom marrow engraftment did occur in the early human experiments died when the transplanted marrow mounted a lethal immunologic reaction against the host.³² This phenomenon, termed 'graft-versus-host disease' (or GVHD), spurred intense animal research which eventually led to the elucidation of a histocompatibility locus antigen (HLA) on Chromosome 6 in humans. This HLA locus contains at least thirty-one separate antigen types resulting in 480 possible genetic combinations and thus is the most complex known genetic region in man.³³ In addition, extensive study revealed that for a graft to be successful, an HLA-D match was required. This matching technique requires the mixing of donor and recipient lymphocytes to be mutually non-reactive. Both marrow graft rejection and GVHD can occur in patients who are fully matched, indicating that there are as yet unidentified genetic determinants of histo-compatibility. While these complexities were being investigated, clinical grafting was successfully carried out between identical twins. The great majority of HLA-matched siblings are compatible by mixed lymphocyte testing while the great majority of non-HLA-matched siblings are not.³⁴ This is presumably

30. Thomas, *Intravenous Infusion of Bone Marrow in Patients Receiving Radiation and Chemotherapy*, 257 N. ENGL. J. MED. 491 (1957).

31. Mathe, *Transfusions et Greffes de Moelle Osseuse Homologue Chez des Humains Irradies a Hautes Dose Accidentellemant*, 4 REV FR. ETUDES CLIN. BIOL. 226 (1959).

32. Mathe, *Immunogenetic and Immunological Problems of Allogeneic Haemopoietic Radio-Chimaeras in Man*, 4 SCAND. J. HAEMATOL. 193 (1967).

33. DAUSSET, *Genetics of the HL-A System: Deduction of 480 Haplotypes*, HISTOCOMPATIBILITY TESTING 53 (1970).

34. Within a family, however, the situation is simplified since only four haplotypes can be involved (two from each parent). See Amos, *Graft Donor Selection Based Upon*

the result of closely linked determinations of histo-compatibility segregating with recognized HLA antigens.

Consequently, although research is ongoing and transplants between matched and partially matched non-siblings have been successfully done, most research centers require that a candidate for transplantation have an HLA-matched donor. The donor is taken to the operating room and up to 150 bone marrow samples are collected, yielding about 750 milliliters of marrow, which are simply infused intravenously in the recipient. The donor is hospitalized overnight and, except for local pain, has no ill effects. It is possible, of course, to have a life-threatening reaction to the general anesthetic.

The recipient, on the other hand, will be critically ill for thirty days and may be in the hospital for three to four months. The expected hospital stay is thirty to forty days for identical twin grafts and forty to sixty days for HLA-identical sibling grafts. Unless the recipient is a twin or has an illness characterized by the absence of an immune response, he will receive large doses of chemotherapy (which will be fatal if engraftment fails) for four days prior to engraftment. The chemotherapy produces severe, albeit transient, nausea. If the patient is being engrafted for a malignant disorder, usually leukemia, he will also receive 1000 rads of total body irradiation in an attempt at preventing recurrent leukemia.³⁵ This alone would be fatal absent a successful engraftment. Following this preparative therapy, the patient can expect to wait twenty to thirty days before the grafted marrow begins to function. During that period, the normal function of the marrow is absent and the patient requires transfusions of red cells, platelets to prevent hemorrhage, and antibiotics to treat infections which will develop. Unfortunately, some of the infections are due to viruses which respond poorly to current available therapy. Some forty to fifty percent of the patients will die either in the immediate post-engraftment period or later of infections or graft-versus-host disease. The procedure is rigorous enough that fifty is the absolute age limit for a patient and most centers only accept patients over forty if they have an identical

Single Locus (Haplotype) Analysis Within Families, 6 *TRANSPLANTATION* 525 (1968).

35. Thomas, *Bone Marrow Transplantation*, Part I, 292 *N. ENGL. J. MED.* 832, 838 (1975).

twin donor. If it is a malignant disease that is being treated, there will be many recurrences. Graft-versus-host disease occurs in approximately fifty percent of patients. Chemotherapy (except in twins) is routinely administered after engraftment for approximately 100 days in an attempt to prevent or ameliorate graft-versus-host disease. In most studies, ten to twenty percent of recipients die of graft-versus-host disease³⁶ and an equal number are left with chronic GVHD characterized by skin and liver disease.

Research is continuing in many centers evaluating new methods of preventing GVHD. The fact that only twenty-five percent of otherwise acceptable recipients have an HLA-D matched sibling has also led many centers to actively investigate methods to select compatible unrelated donors. Nevertheless, these two facts — the limited number of prospective recipients with a match and GVHD — remain the major factors limiting the application of bone marrow transplantation.

The indications for bone marrow transplantation can be conveniently divided into three groups. The first is that group of diseases for which transplantation is an established treatment. Little litigation would be expected to arise in those cases, however neither physicians nor third-party payers are in complete agreement as to which diseases properly belong in this group. The use of bone marrow transplantation has been advocated in many other diseases which are not established indications. These segregate into two main categories, those in which the efficacy (as compared to a more standard treatment) is under investigation and those for which there is lack of any alternative therapy. Litigation most often concerns these patients. A variably detailed analysis of the diseases in these categories follows which is intended to put the coverage decisions of third-party payers in context and supply a template for analysis of the legal rights of patients denied coverage.

A. *Established Indications*

Bone marrow transplantation was first successfully applied to aplastic anemia, a condition of bone marrow failure, in a logical attempt to replace a defective organ with a normal one. Al-

36. *Id.* Part II, at 897.

though this condition may arise from exposure to drugs and chemicals, the majority of cases have no identifiable cause.³⁷

Investigators followed the model scientific method quite closely. Initially, marrow transplants were carried out in patients who were in the end stages of disease and who had a twin donor so that there would be no problem with graft-versus-host disease. These transplants established the feasibility of bone marrow transplantation in aplastic anemia.

A trial was initiated in 1974 to compare the standard treatment³⁸ with bone marrow transplantation.³⁹ The technology itself prevented strict adherence to the principles of the randomized double-blind prospective controlled study. Ethically, control group patients could not be subjected to a sham transplant and they were aware that they were taking androgens instead. The investigators also would be able to tell which patient was on which treatment by the virilizing effect of the androgens and the severe side effects of the chemotherapy necessary for bone marrow transplantation. The control was, therefore, made up of patients who had no donor or who refused transplantation.

The transplanted patients had a significantly better (probability = 0.0002)⁴⁰ outcome as defined by survival. Fifty-seven percent of the transplanted group were alive more than one year after the transplantation — although ten percent had severe graft-versus-host disease — leaving forty-seven percent alive and well. The control group had twenty-five percent alive with twenty-one percent free of disease. Not incidentally, randomization among control patients between androgens therapy and no treatment (other than transfusion) failed to show any benefit for the treated group. Even this study, although ap-

37. Patients suffer from bleeding and infection due to failure of the marrow to produce platelets and white blood cells. Treatment is only supportive. Although patients often seem to respond to androgens, eighty percent of the patients receiving such therapy will be dead within two years. Camitta, *Severe Aplastic Anemia: A Prospective Study of the Effect of Early Marrow Transplantation on Acute Mortality*, 48 BLOOD 63 (1976).

38. Androgens are the standard treatment. In this case the efficacy of the standard treatment had never been carefully investigated and was controversial.

39. Camitta, *supra*, note 37.

40. Thus by Student's T test there would be less than 2: 10,000 probability that this result would occur by chance.

proaching the ideal, has been criticized for not having a true control group.⁴¹ The case for marrow transplant is firmly buttressed, however, by meeting the last requirement of the scientific method — reproducibility.

The International Bone Marrow Transplant Registry, comprising twenty-four worldwide teams, confirmed these results showing a forty-four percent one-year survival.⁴² This study simultaneously answers two questions which arise with the application of any new technology. First, are the results a fluke or in some inapparent way biased? And second, can the results be duplicated by a group or groups without the experience of the original proponent? Bone marrow transplant for aplastic anemia has survived a careful, albeit time-consuming, application of the scientific method and is now the accepted treatment for patients under the age of forty with an HLA-compatible sibling.

The second established indication for bone marrow transplantation is acute leukemia. A complete explanation of the indications for transplantation in acute leukemia depends on the success rate with other treatment in the various age groups and subtypes of leukemia and is therefore beyond the scope of this review. A tracing of the progress through acute myelogenous leukemia in adults,⁴³ however, is worthwhile to demonstrate again the pitfalls of deviations from the scientific method. For simplicity, the series of investigations at one major pioneering bone marrow transplantation center in Seattle will be traced.

The first ten patients were treated when their disease was refractory to all standard therapy — thus justifying the initial experiment. It was hoped that intensive pretreatment with 1000 rads total body irradiation (TBI) would ablate the leukemia and the graft, in addition, would destroy any recurring leukemia cells. Six patients responded to the treatment, but leukemia re-

41. The patients who did not have a donor could in some unknown way have been different. In addition, the patients who refused transplantation could have been different. Did they refuse because their physicians felt that they were not ideal candidates and either consciously or unconsciously transmitted their feelings to the patient? If this were the case, the control group might have represented the sicker patients.

42. Bortin, *Allogeneic Bone Marrow Transplantation for 144 Patients with Severe Aplastic Anemia*, 245 J.A.M.A. 1132 (1981).

43. Again, this must be limited to patients under age forty because of the markedly poor survival in older patients.

curred in five patients within seven months. One patient apparently is cured after nine years.⁴⁴ The Seattle group deduced that the preparation (1000 rads TBI) was not intensive enough to eradicate the leukemia and treated one hundred more patients with refractory leukemia.⁴⁵ No randomized study comparing one treatment to the other was undertaken. This is ethically justifiable on the basis of the poor results of the original pilot study and the presumption that more intensive treatment might eradicate the leukemia. Early mortality was high, but thirteen percent of the patients apparently were cured, being in remission four to eight years after treatment.⁴⁶

Based on these results, which were encouraging in this formerly one hundred percent fatal illness, a presumption was made. The hypothesis that patients who were in remission, and had so few leukemia cells that the cells could not be seen in the bone marrow, would do better was tested in nineteen patients with acute myelogenous leukemia who were in first remission after treatment with chemotherapy.⁴⁷ Twelve of those patients were alive without evidence of leukemia for two to four years after bone marrow transplantation. The majority of those patients presumably are cured. Based on this study, patients less than age forty with acute nonlymphocytic leukemia in first remission are generally accepted as candidates for bone marrow transplantation. It is noteworthy that no randomized study was done. Various groups have been critical of this fact, suggesting that chemotherapy also was capable of producing apparent cures in this setting⁴⁸ and recommending a randomized trial between chemotherapy and bone marrow transplantation.⁴⁹ The Seattle group has initiated such a study.⁵⁰

44. Thomas, *Allogeneic Marrow Grafting for Hematological Malignancy Using HL-A Matched Donor-Recipient Sibling Pairs*, 38 BLOOD 267 (1971).

45. Thomas, *One Hundred Patients with Acute Leukemia Treated by Chemotherapy, Total Body Irradiation, and Allogeneic Marrow Transplantation*, 49 BLOOD 511 (1977).

46. Dinsmore, *Allogenic Bone Marrow Transplantation for Patients with Acute Lymphoblastic Leukemia*, 62 BLOOD 381 (1983).

47. Thomas, *Marrow Transplantation for Acute Nonlymphoblastic Leukemia in First Remission*, 301 N. ENGL. J. MED. 597 (1979).

48. Peterson, *Long-Term Disease-Free Survival in Acute Nonlymphocytic Leukemia*, 57 BLOOD 1144 (1981).

49. Bortin, *Bone Marrow Transplantation for Acute Leukemia: Factors Associated with Early Mortality*, 249 J.A.M.A. 1166, 1174 (1983).

50. Thomas, *Marrow Transplantation for Acute Nonlymphoblastic Leukemia*, 302

The bone marrow transplant team at University of California Los Angeles (UCLA) has reported a prospective study comparing bone marrow transplantation to chemotherapy in first remission.⁵¹ This study is not strictly randomized. Those patients who did not have an HLA-identical sibling donor served as controls and received intensification chemotherapy. The results, when subjected to actuarial statistical analysis, were somewhat surprising.⁵² Bone marrow transplantation was superior to chemotherapy in preventing leukemic relapse, but the actuarial three-year survival was not statistically different. Criticism has not appeared in print, but will surely point out that the study size is small, the control not truly randomly selected and the three-year survival for bone marrow transplantation of forty-three percent is less than the sixty-five percent Seattle continues to report.⁵³

The point is not that the results are confusing, only that strict adherence to the scientific method is required to be certain of results — regardless of alluring preliminary reports. Most hematologists still consider bone marrow transplantation the treatment of choice, largely because they have not experienced the relatively good long-term response seen in the chemotherapy arm of the UCLA study.⁵⁴

The last⁵⁵ and most recent widely accepted indication for bone marrow transplantation is chronic myelogenous leukemia. This is an unusually stereotyped disease. Patients are usually in the third or fourth decade of life and have acquired an abnormal

N. ENGL. J. MED. 409 (1980).

51. Champlin, *Treatment of Acute Myelogenous Leukemia — Bone Marrow Transplantation Versus Consolidation Chemotherapy* 19 AM. SOC. CLIN. ONC. PROC. 180 (1983).

52. Kaplan, *Non-parametric Estimation from Incomplete Observation*, 53 J. AM. STAT. ASSN. (1958).

53. Letter from E.D. Thomas, M.D. (Head of the Oncology Division of the University of Washington School of Medicine and the Fred Hutchinson Cancer Research Center in Seattle, Washington) to Kieran Fitzpatrick, M.D. (Member of the Northern California Permanente Medical Group Bone Marrow Transplant Advisory Board) discussing three-year survival. (March 4, 1980.)

54. Children with acute leukemia generally have a better response to chemotherapy and it is only when they are at "high risk" for relapse that bone marrow transplantation is indicated. A lengthy discussion of those leukemic states considered to be high risk would not serve the purpose of this review.

55. There are very rare non-malignant congenital conditions which because of their rarity are generally accepted indications for bone marrow transplantation although it is not possible to subject them to rigorous scientific scrutiny.

chromosome. The disease is easily controlled with oral chemotherapy which produces few side effects for thirty-six to forty-two months. The disease then, more or less abruptly, changes to an accelerated phase which is fatal within two to nine months. There is no therapy other than bone marrow transplantation which delays the onset of this accelerated phase. The ability to demonstrate the disappearance of the abnormal chromosome provides convincing evidence that early apparent cures may be real. As a result of this lack of alternative therapy and the ability to demonstrate the elimination of the abnormal chromosome,⁵⁶ many hematologists have accepted bone marrow transplantation as the treatment of choice for chronic myelogenous leukemia, in spite of the preliminary nature of the data. No controlled studies have been done. At Seattle sixteen cases have been done and ten patients are in remission.⁵⁷

B. Investigational Indications

It is the patients with the diseases described below who are most frequently denied coverage and are likely to be litigants. As mentioned, bone marrow transplantation theoretically should be applicable to any malignancy which is highly sensitive to chemotherapy or radiation. In theory, doses of treatment could be utilized which would otherwise lead to fatal marrow changes and then the patient could be "salvaged" by bone marrow transplantation. Malignancies which could be described as sensitive to treatment include oat cell carcinoma of the lung, Hodgkin's disease, non-Hodgkin's lymphoma and neuroblastoma. All of these diseases have been treated with bone marrow transplantation in an uncontrolled fashion.⁵⁸ For patients and their physicians who have exhausted conventional therapy for these malignancies, the use of bone marrow transplantation represents an attractive rational therapy. As will be discussed below, because

56. E.g., Feder, *Cure of Hematologic Neoplasia with Transplantation of Marrow from Identical Twins*, 300 N. ENGL. J. MED. 333 (1979).

57. Telephone interview with R. Storb, Ass't. Director of Bone Marrow Transplantation in Seattle (April 20, 1982).

58. Applebaum, *Review of Use of Marrow Transplantation in the Treatment of Non-Hodgkins Lymphoma*, 1 J. CLIN. ONCOL. 440 (1983); see also Seeger, *Neuroblastoma: Clinical Perspectives, Monoclonal Antibodies, and Retinoic Acid*, 97 ANN. INTERN. MED. 873 (1983); Graze, *Induction of Complete Remission from Disseminated Oat Cell Carcinoma by Intense Chemoradiotherapy and Bone Marrow Transplantation*, 19 PROC. AM. ASSOC. CAN. RES. 51 (1978).

of the few cases reported and the high rate of relapse (although successes have been reported), most physicians and all third-party financiers would deem bone marrow transplantation in this setting as experimental. What is needed is the study of a large enough series of patients followed for long enough periods of time so that the risk/benefit ratio can be defined.

A second group of patients in the investigational category likely to be litigants are those patients who are otherwise acceptable candidates for bone marrow transplantation but lack an HLA-identical sibling donor. The literature contains quite a few reports of one or more patients who have had bone marrow transplantation from specially treated HLA-incompatible marrow donors,⁵⁹ or with their own (autologous) marrow frozen and reinfused after otherwise lethal chemotherapy for cancer.⁶⁰ Approximately one-third of the patients who are otherwise candidates for bone marrow transplantation will have an HLA-identical sibling donor. For the other two-thirds, the use of other donors is an attractive alternative which has been accomplished. Again, these techniques have not been studied in sufficient numbers of patients for sufficiently long periods to be considered anything other than investigational. It is understandable that these patients, whose physicians may feel that further study certainly will confirm the validity of the new indication or technique, may have difficulty in understanding why the procedure is considered experimental in their particular case.

Even more compelling is the patient who has no other therapeutic option and in whom bone marrow transplantation might potentially be curative. This would include, for example, patients with the malignancies described above who have relapsed after the initial therapy, patients with sickle cell disease or thalassemia, or patients with A.I.D.S.. Patients with the relapsed malignancies have been transplanted with occasional success.⁶¹ Patients with thalassemia were treated by a prominent hematologist without the approval of the University Human Experimentation committee, resulting in severe sanctions.⁶² At least one

59. Dinsmore, *supra* note 46.

60. Gale, *Autologous Bone Marrow Transplantation in Patients with Cancer*, 243 J.A.M.A. 540 (1980).

61. *See supra*, note 58.

62. Jacobs, *Pioneer Genetic Implants Revealed*, Los Angeles Times, Oct. 8, 1980, at

A.I.D.S. patient has been transplanted unsuccessfully.⁶³

In each of these instances, the patients and their respective physicians made a conscious choice and selected an unknown treatment over supportive care. Although the choice is understandable, if we return to our analogy with the development of a new drug,⁶⁴ these preliminary investigations would be analogous to Phase 1 human studies. The FDA does not allow marketing of a drug at this stage but requires that efficacy be established.

IV. THIRD PARTY FINANCING

The difference in the source of funding (third-party payers versus pharmaceutical companies with vested interests) coupled with the high visibility of the patients with life-threatening diseases being treated has focused attention on individual patients being denied access to bone marrow transplantation because of economic constraints. At the same time, health insurers have found themselves in a new role for which they are variously suited. Health insurance contracts, if they are not to be unenforceable for vagueness, must in some way delimit what the insurer has contracted to pay for. The terms usually delineate specific exclusions to a generally inclusive term. This inclusive phase is designed to exclude payment for treatments which, while perhaps beneficial, are not recognized generally by the medical community.⁶⁵ In addition, one of the exclusion clauses denies payment for treatment which is "investigational," "experimental" or "unproven."⁶⁶

For treatments which involve medications, the classification is relatively easy. Since 1964 the FDA has required that a drug be proven efficacious as well as safe before it can be released for general distribution. Consequently, any released medication is no longer considered either unproven, unsafe nor inefficacious. With the technology explosion spurred on by the "technology

1, col. 1.

63. Hassett, *Bone Marrow Transplantation in A.I.D.S.*, 309 N. ENGL. J. MED. 665 (1983).

64. *See supra*, text accompanying notes 23-25. A phase 1 study is a toxicity study in humans.

65. Chiropractic treatment is one example.

66. *See infra* pages 228-31.

transfer” movement of the 1970’s,⁶⁷ patients were being exposed to new unproven technologies *on the recommendation of physicians*. The majority of these exposures were in the diagnostic area, relatively harmless (from the standpoint of being noninvasive) and a relatively inexpensive part of the individual patient’s treatment. In the aggregate, of course, the application of diagnostic technology is recognized as a major factor in the spiralling cost of medical care.

As technology began to be applied to treatment, its cost and risks rose. At the same time, there was no agency comparable to the FDA charged with the control of the marketplace. Thus the convenient fine line represented by the release of a drug by the FDA did not exist referent to technology.

The National Center for Health Care Technology (NCHCT) was created to evaluate the application of new technologies but does not have the authority to limit that the FDA does. In addition, although insurance carriers might be expected to welcome any guidelines set out by NCHCT even absent the force of law, the majority of the consensus development conferences (CDC) have concluded only that more evidence was needed.

There has been no CDC on the indications for bone marrow transplantation. In addition, the NCHCT was denied funding in 1982 and is not functional. Nevertheless, major carriers have come to remarkably similar positions concerning the funding of bone marrow transplantation. Kaiser, for example, considers bone marrow transplantation to be the standard practice (if the recipient is less than age forty and has an HLA-matched sibling donor) for patients with: (1) severe aplastic anemia; (2) acute leukemia *in remission* — a) adults in first remission, b) childhood acute myelogenous leukemia (high risk) in first remission, c) childhood leukemia (low risk) in second remission; (3) chronic myelogenous leukemia in a controlled phase; (4) non-malignant conditions — a) severe combined immune deficiency, b) Wiskott-Aldrich syndrome.

Blue Shield covers bone marrow transplantation for: (1) aplastic anemia; (2) acute leukemia in remission; (3) severe com-

67. See *supra* pages 205-06.

bined immunodeficiency; and (4) infantile osteopetrosis. The donor must be an HLA-matched sibling.

Medi-Cal, the Medicaid agency of the state of California, covers bone marrow transplantation for severe aplastic anemia and acute leukemia in remission. The donor must be an HLA-match but need not be a sibling. They require that the transplant be done within the state of California unless the centers within the state are at capacity. The recipient must be less than fifty years of age.

California Children's Service, a public agency, covers bone marrow transplantation for: (1) severe combined immunodeficiency; (2) acute leukemia in remission; (3) chronic myelogenous leukemia; (4) agnogenic myelofibrosis; (5) Wiskott-Aldrich; (6) infantile osteopetrosis; (7) granulocyte dysfunction disorders; and (8) Gaucher's Disease.

More important than the covered situations themselves is an analysis of the basis for coverage decisions by third-party payers. For a technology like bone marrow transplant, without a mechanism for government approval or disapproval, these coverage decisions of third-party payers actually determine the rate of utilization.

The approaches vary from carrier to carrier, but most attempt to assess the technology (here bone marrow transplant) through assessment of available medical literature or the solicitation of expert opinion. There are two polar approaches, breached by a continuum, followed by the majority of carriers. Some carriers focus primarily on well-designed clinical trials looking for evidence of safety and efficacy. A decision to cover only those situations where there was *conclusive*⁶⁸ scientific evidence based on controlled trials would limit reimbursement of bone marrow transplant to aplastic anemia and adult acute leukemia in first remission.⁶⁹

68. Student, 6 *BIOMETRIKA* I (1908).

69. If the reviewers were rigid in their demands for scientific validation, they could point to the controlled U.C.L.A. study (*see supra* note 56) which showed no difference between marrow transplant and chemotherapy in first remission, and conclude that bone marrow transplantation was still experimental for acute leukemia in remission.

The other polar approach is to seek to determine what is acceptable medical practice. This usually means that the carrier seeks the opinion of specialists in the treatment of the disease to which the technology is being applied. The carriers often employ medical consultants who decide which experts to consult. They also often seek the opinion of medical specialty organizations.⁷⁰ This approach theoretically integrates a determination of safety and efficacy presuming that the medical community has made such determinations prior to incorporating the technology into their collective practices. This often is not the case in the instance of a technology such as bone marrow transplantation.

A. Position of Major Carriers

Blue Shield's "Medical Necessity" pamphlet which is given, along with a health service agreement, to a consumer or group representative at the time of subscription, includes a general statement of covered services and a specific statement of conditions for which bone marrow transplantation is a covered service.

The "Medical Necessity" pamphlet contains a warning set out in the introductory paragraph in bold-face type that a physician order does not in itself make the service "medically necessary." The definition contains two separate requirements: (1) the treatment must be established as safe and effective; (2) the treatment must be necessary and consistent with generally accepted professional medical standards and not experimental or "investigational." The receipt of requests for coverage from patients who have undergone bone marrow transplantation for a particular indication from a particular type of donor provides a list of emerging applications requiring scrutiny.

New technologies (here new applications of bone marrow transplantation) as they mature are presented to the Medical Policy Committee where the decision is made designating the service as covered or non-covered. The decision as to which ap-

70. In the instance of bone marrow transplantation, the carrier might consult The American Society of Hematology or the American Society of Clinical Oncology. Given the controversy regarding the use of bone marrow transplantation in acute leukemia, the carrier seeking the recommendation of the American Society for Organ Transplantation might expect to receive a different opinion than that given by the aforementioned societies.

plications are ready to be taken before the committee is made by a physician charged with the responsibility of monitoring emerging technologies.

The committee consults local experts and seeks recommendations of committees of specialists. Before coming to a conclusion regarding coverage, the Medical Policy Committee hears invited expert testimony, both pro and con, in an open meeting. By this process Blue Shield recently extended coverage to infantile osteopetrosis, an exceedingly rare disease that has been treated only two or three times by bone marrow transplantation.

Kaiser Foundation Health Plan, Inc. (Northern California Region) distributes to its prospective new members a booklet entitled "Disclosure Form and Evidence of Coverage" which describes covered benefits. The first paragraph in bold face type cautions the reader that it is only a summary and that "[t]he Health Plan Service Agreement must be consulted to determine the exact terms and conditions of coverage. A specimen copy of the service agreement will be furnished to you upon request." The general statement of coverage in bold face type reads: "The services described in this brochure are covered benefits only if and to the extent they are provided, prescribed or directed by a Plan physician. The Health Plan will not pay charges for services from non-plan doctors and hospitals, except as otherwise indicated in this brochure." This appears in section three which contains thirteen subsections describing limitations. Subsection 3-K provides for payment, following written assumption of financial responsibility, for treatment requiring "skills not available within" the Kaiser system. In the Southern California region the Kaiser Health Plan has a bone marrow transplant facility, but in the Northern California region this subsection effectively requires written prior authorization for coverage for bone marrow transplantation. The provisions of section three are often summarized as prescribed medical and hospital services.

Section five, entitled "Exclusion, Limitations and Reductions of Benefits," contains three pertinent exclusions. The first excludes "experimental" procedures and procedures not gener-

ally available in Northern California.⁷¹ The second excludes blood. Arguably, bone marrow might be considered blood. Finally, all organ transplants except kidney transplants are excluded. The wording of the service agreement exclusion section is exactly the same, although not in bold type.

The Permanente Medical Group (the physicians who contractually supply medical care to members of the Northern California Kaiser Health Plan) established a Bone Marrow Transplant Advisory Board on May 10, 1979. It consists of four hematologists — two of whom are pediatric hematologists. The board uses literature review and solicits expert advisory opinions in order to decide what applications remain investigational and what are, or should be, the standard of practice. The emphasis shifts depending on the rarity of the illness and the availability of alternative forms of treatment. An extremely rare disorder cannot realistically be subjected to controlled blinded study. In considering such applications, the board gives greatest consideration to invited expert opinion. On the other hand, for an application which is relatively common, critical review of controlled studies is emphasized. At its inception the board only approved aplastic anemia with an HLA-matched sibling donor and adult acute leukemia in remission with an identical twin donor. These criteria are reviewed and revised semi-annually as a routine and more frequently if new scientific evidence indicates.

Medicare coverage decisions are a product of federal agencies and enabling acts. Section 1862(a) of the Medicare Law proscribes payment for services “not reasonable and necessary” for diagnosis and treatment.⁷² There is very little legal or administrative precedent interpreting this section of the law.

Public Law 95-623, signed into law by President Carter November 8, 1978,⁷³ established the National Center for Health Care Technology (NCHCT). As part of a duty to assess health care technology, the NCHCT was charged with the responsibil-

71. Bone marrow transplants were not available in Northern California until 1983 but they were reimbursed, when approved, if done outside of Northern California.

72. 42 U.S.C. § 1395y (1976).

73. Perry, *The National Center for Health Care Technology*, 245 J.A.M.A. 2510 (1981). Health Services Research, Health Statistics, and Health Care Technology Act of 1978, Pub. L. No. 95-623, 92 Stat. 3443 (1978).

ity of providing the Health Care Financing Administration (HCFA) with the best available information concerning the technology under review for its use in making a Medicare coverage decision.

Prior to the decision not to fund NCHCT, a Medicare reimbursement decision usually arose when a Medicare carrier received a claim for an unfamiliar service. If the decision could not be made locally, it was referred to HCFA for a central decision. HCFA would in turn refer to the Public Health Service (PHS) any technologic or scientific issue for evaluation. The NCHCT (as an agency within PHS) would then place a notice in the *Federal Register* and begin an evaluation. Their evaluation included literature review and consultation with expert consultants. If necessary, a special panel was convened to arrive at a consensus. The NCHCT then would make a recommendation to HCFA. This procedure continues within PHS in a similar manner now that NCHCT is not funded.

Medi-Cal, the Medicaid agency of California, makes its decision independent of Medicaid directives. They do receive occasional guidelines which are part of the input into the decision-making process. When a patient's bill for treatment involving a new technology is submitted to the Medicaid office, they are approved locally unless the Medicaid advisor is not certain if the service is medically necessary or proven. Directives issue from the Benefit Section in Sacramento, delineating covered indications for bone marrow transplantation. The decision is based on solicited expert opinion and literature review.

Decisions at the California Children's Services (CCS) can be made by medical personnel with or without a formal submission to an advisory panel. The medical director makes decisions based on a review of the pertinent literature and solicited opinions from physician experts not employed by the CCS. The expert opinions and literature review are synthesized and if a new technology, or applications thereof, is a major variation, it may be referred to an advisory committee for final approval.

The major third-party payers, then, uniformly deny coverage for bone marrow transplantation that is still experimental. This determination of investigational status is usually made by a

committee which bases its decision on the scientific analysis of experimental data or solicited expert opinion. These experts presumably base that opinion on a similar analysis of scientific data.

B. Position of Marrow Transplant Centers

The transplant centers themselves, of course, are a theoretical source of financing. As discussed above, the earliest stages of new drug investigation are funded by pharmaceutical houses. When the drug reaches the stage where it is being tested in controlled clinical trials it is often supplied to investigators, and thereby patients, by the pharmaceutical houses. If the drug or treatment is not funded by industry, its cost is ordinarily part of the research grant which the investigator has obtained (most frequently, from the National Institute of Health). Although some of the early transplant work was funded through grants, today bone marrow transplant centers require evidence of "adequate insurance coverage"⁷⁴ or advance funding before they will consider a patient for bone marrow transplantation.

V. COMPENSATING THE INDIVIDUAL PLAINTIFF

The attorney and his client who has been denied coverage for bone marrow transplantation have several options. Occasionally, there may have been new developments since the last review by the third-party payer and therefore a request for reconsideration of the decision is prudent.

As of October 1983, certain states, such as Minnesota and Pennsylvania, have funded extraordinary procedures, including bone marrow transplantation. In California, California Children's Service (CCS) funds indicated bone marrow transplantation for children who have no other coverage and whose parents are financially qualified. It is noteworthy that these state agencies have necessarily set limits on what applications of bone marrow transplantation they will fund.

The most reliable source of funding for patients who either have no insurance or who are not covered for the particular bone

74. Letter from Robert McMillan of Scripps Clinic to L. S. Wilkinson (June 10, 1983).

marrow transplant application has been a media appeal. Although these campaigns sponsored by the local broadcast media generally have been successful, they have major shortcomings. To begin with, rural areas probably do not have the requisite population base to ensure a successful campaign. Time constraints may preclude the media approach altogether. Most importantly, the access to medical technology should not depend on the public relations skills of the patients and their families. Parents, in particular, have found fund raising efforts emotionally draining at a time when their children need them. A group of them have formed the Parents Action for Children's Cancer Treatment,⁷⁵ with the intent of lobbying state and federal governments to fund uninsured investigational procedures, particularly bone marrow transplantation. Spokespersons for the group voice valid concerns that as media campaigns become more commonplace, public donations may decrease significantly.⁷⁶

A. *Declaratory Relief*

Should such a public relations campaign be untenable, the bone marrow transplant candidate who is denied access because of inability to pay has a problem in need of legal resolution. An unsuccessful resolution conceivably could deny life-saving treatment. An insured party whose carrier denies coverage based on a determination that bone marrow transplantation would be experimental will, of course, bring suit against that carrier. The indigent has an equally high profile defendant in their particular Medicaid agency. The uninsured plaintiff who cannot qualify for Medicaid can only hope to look to the bone marrow transplant center.

If consultation is timely, equitable jurisdiction may lie. For those with an insurance contract as well as those whose rights are delineated by statute, declaratory judgment can be sought under the Federal Declaratory Judgment Act.⁷⁷ Forty-nine states also authorize declaratory judgments by statute.

The irreparable harm consequent to denial of a necessary bone marrow transplantation is uniquely susceptible to prospec-

75. Hallissy, *Contra Costa Times*, Aug. 24, 1983, at 3, col. 1.

76. *Id.*

77. 28 U.S.C. § 2201 (1976).

tive equitable relief. If the bone marrow transplant candidate was able to privately finance the bone marrow transplantation and seeks only compensation, a legal remedy may well be adequate. However, if denial of insurance or Medicaid coverage results in denial of bone marrow transplantation and death, the plaintiff's remedy at law would be manifestly inadequate. The Court has construed liberally the language of 28 U.S.C. section 2201, which requires an actual controversy involving legal relations capable of immediate and definite determination, refusing only to answer abstract questions or issue advisory opinions.⁷⁸ The bone marrow transplant candidate seeking judicial construction of a statute or an insurance contract would, therefore, not have to commence another action to meet the statutory requirements for a declaratory judgment. The patient refused bone marrow transplantation coverage for an unproven indication would encounter several obstacles in their efforts to obtain a declaratory judgment.

The most onerous to overcome is the caveat in *Public Service Comm'n v. Wycoff Co.*⁷⁹ where the Court stated that declaratory relief should be granted with caution if it would reach far beyond the particular case. Any judicial construction of a health insurance contract or a state or federal statute which granted coverage would be expected to foster litigation by other similarly situated plaintiffs. The defense certainly would contend that such a decision would lead to a flood of litigation and that it was just such a prospect that engendered the Court's caution in *Wycoff*.⁸⁰

In California, both statutory⁸¹ and case law⁸² emphasize the court's discretionary power to refuse to exercise equitable jurisdiction. Nevertheless, the Supreme Court has found declaratory judgment applicable to questions arising from the construction and operation of health insurance policies.⁸³ Declaratory relief is

78. *Aetna Life Ins. Co. v. Haworth*, 300 U.S. 227 (1937).

79. *Public Service Comm'n v. Wycoff Co.*, 344 U.S. 237 (1952).

80. The Court did not discuss this caution except to note that the legal issues decided by the declaratory judgment must be clear to enable a court to see the ramifications of its decision. 344 U.S. at 244.

81. CAL. CIV. PROC. CODE § 1061 (West 1980).

82. *Hannula v. Hacienda Homes Inc.*, 34 Cal. 2d 442, 211 P.2d 302 (1949).

83. *Aetna Life Ins. Co. v. Haworth*, 300 U.S. 227 (1937); *Stoner v. New York Life Ins. Co.*, 311 U.S. 464 (1940).

particularly appealing when applied to denial of coverage for bone marrow transplantation because it serves to prevent the harm rather than compensate the victim. Unfortunately, the particular plaintiff's illness may progress at a rate which precludes even the relatively rapid relief of a declaratory judgment. This would seem to suggest that a judicial construction should be sought by a covered party who is well in order to establish their contractual rights (and, in the process, other similarly situated parties' rights) at a time when more deliberation would be possible.

There are more serious limitations to this approach. The first is the universal statutory requirement for an actual controversy, which has been construed generally to mean that declaratory relief is not available for future or contingent rights. Some courts have construed the actual controversy requirement more liberally, holding that where the future event is reasonably certain to occur and where all interested parties are represented, declarative relief may be granted even in the absence of a present controversy.⁸⁴ The Supreme Court, in construing a Tennessee statute,⁸⁵ has held that all interested persons must be made parties even in the setting of a present controversy. This second requirement is easily met when there is a present controversy (that is, the plaintiff has a transplantable illness). The number of persons seeking coverage for a bone marrow transplantation for a particular disease should be relatively small and discoverable. In the instance of a future or contingent right, all covered persons would have to be represented by a class action. The requirement for reasonable certainty could be met for the relatively common disorders. That is, given insurance or Medicaid coverage of a certain number, the evidentiary burden of showing that a disease of a given prevalence is reasonably likely to occur would be a matter of relatively simple statistics. With a rare disorder, however, it would take an enormous covered population to make it statistically "reasonably likely" that the future event would occur.

The requirements that: (1) the plaintiff seek counsel prior to the harm, (2) there be a present or a reasonably likely future

84. *Caldwell v. Rosenberg*, 47 Cal. App. 2d 143, 117 P.2d 366 (1941).

85. *Nashville C. and St. L. R. Co. v. Wallace*, 288 U.S. 249 (1932).

controversy, (3) that all interested persons be represented, and (4) that the court exercise its discretionary power with caution if its decision would have far-reaching effects, limit the remedy of declaratory relief to a small fraction of patients denied (or potentially denied) bone marrow transplantation access. Declaratory relief would not be applicable to patients who were neither insured nor indigent.

B. Due Process

It is clear that should the plaintiff fail to obtain declaratory relief, irrespective of the theory under which relief is sought, the case will turn on an examination of the process by which the carrier or agency denies coverage. The extensive state regulation of hospitals receiving Medicaid funds, brought about by efforts at cost-containment, raises the possibility that hospital decisions can be challenged successfully as violative of the procedural due process requirements of the fourteenth amendment.

The Supreme Court was asked to uphold such a successful due process challenge in *Blum v. Yaretsky*.⁸⁶ The action was brought by nursing home residents representing a class of Medicaid patients alleging that they were transferred without notice or opportunity for a hearing. The facts were not disputed and only the "state action" requirement of the fourteenth amendment was litigated. In spite of finding extensive state involvement,⁸⁷ the Court found insufficient nexus between the state and the transfer decision, quoting with favor limiting language from *Jackson v. Metropolitan Edison Co.* and its predecessors.⁸⁸ The suspicion that unwritten policy considerations influenced the Court's failure to find "state action" is supported by a strong

86. *Blum v. Yaretsky*, 457 U.S. 991 (1982).

87. Ninety percent of the medical care of the residents was paid for by the state. The level of care stratification which the nursing home applied was promulgated by the state.

88. "That Amendment erects no shield against merely private conduct, however discriminating or wrongful." *Shelley v. Kraemer*, 334 U.S. 1, 13 (1947). "The mere fact that a business is subject to state regulation does not by itself convert its action into that of the State for purposes of the Fourteenth Amendment." *Jackson v. Metropolitan Edison Co.*, 419 U.S. 345, 350 (1974). "The complaining party must also show that 'there is a sufficiently close nexus between the State and the challenged action of the regulated entity so that the action of the latter may be fairly treated as that of the State itself.'" *Blum v. Yaretsky*, 457 U.S. 991, (1982), citing *Jackson v. Metropolitan Edison Co.*, 419 U.S. 345, 351 (1974).

dissent from Justice Brennan, joined by Justice Marshall. Nevertheless, it is doubtful that any amount of creative lawyering could make a stronger case for state involvement in a private health care facilities decision than that made by the facts of *Blum v. Yaretsky*. A decision by a Veteran's Administration Hospital or a military facility to forego bone marrow transplantation might be better attacked if it could be shown that the decision was an administrative one rather than an individual medical judgment. The evidentiary burden would constitute a significant hurdle in any such litigation.

The Medicare/Medicaid decision-making process is the most susceptible to a successful due process challenge. The decision for coverage is made variously at local or national levels. Medicare decisions are not applicable to bone marrow transplantation because of age limitations. In California, Medi-Cal has made a decision to fund only acute lymphoblastic leukemia and aplastic anemia, although individual exceptions have been made. The decision-making process may or may not be adequate, but the patient has neither hearing nor notice. The decisions made by state officials, administering Medicaid funds under Title XIX of the Social Security Act, 42 U.S.C. section 1396, are clearly exercises of state power which must conform to the restraints of the fourteenth amendment.

*Blum v. Yaretsky*⁸⁹ appears to limit due process challenge to that class of patients denied bone marrow transplantation by direct state actions. For that plaintiff, however, injunctive relief based on violation of the Due Process Clause of the fourteenth amendment should be available. This remedy might be made impracticable by the time required for an evidentiary hearing and administrative resolution of the claim. Presuming, however, that bone marrow transplant centers would be willing to proceed under those uncertainties, injunctive relief could provide prompt legal resolution for the individual plaintiff. Significant societal gains could be expected to follow, both initially, from the administrative decision made establishing coverage for the illness suffered by the particular plaintiff, and subsequently, from procedural reform.

89. *Blum v. Yaretsky*, 457 U.S. 991 (1982).

C. *Anti-Trust Action*

Recent cases brought before the Supreme Court seeking application of the Sherman Antitrust Act⁹⁰ to health-insurance carriers⁹¹ suggest an alternative cause of action. Prior to 1975 the health care industry was not exposed to antitrust liability under the Sherman Act because it was thought not to meet the requirement for interstate commerce.

The Supreme Court, in *Hospital Building Co. v. Trustees of the Rex Hospital*, however, found that typical hospital activities brought most hospitals within interstate commerce.⁹² The learned professions formerly were held exempt from the Sherman Act, but in recent years have been brought under the Act's requirements.⁹³

The McCarran-Ferguson Act⁹⁴ exempts the "business of insurance" from the Sherman Act to the extent it is regulated by state law. In *Group Life and Health Insurance Co. v. Royal Drug Co.*, however, the Supreme Court narrowed the definition of "business of insurance"⁹⁵ thereby removing *McCarran-Ferguson* protection from many of the practices of insurance carriers. The Court held that only risk-spreading activities were the business of insurance, and specifically found that "risk-reducing" activities were *not* the business of insurance.⁹⁶

Therefore, both hospitals and health-insurance carriers have

90. 15 U.S.C. §§ 1-7 (1976).

91. *Group Life and Health Insurance Co. v. Royal Drug Co.*, 440 U.S. 205 (1979), *reh'g. denied* 441 U.S. 917; *Virginia Academy of Clinical Psychologists v. Blue Shield of Virginia*, 469 F. Supp. 552 (E.D. Va. 1979), *rev'd*, 624 F.2d 476 (4th Cir. 1981), *cert. denied*, 450 U.S. 916 (1981).

92. 425 U.S. 738, 744 (1976).

93. *Group Life and Health Insurance Co. v. Royal Drug Co.*, 440 U.S. 205; *Virginia Academy of Clinical Psych. v. Blue Shield of Virginia*, 624 F.2d 476; *Ballard v. Blue Shield of Southern West Virginia, Inc.*, 543 F.2d 1075 (4th Cir. 1976) *cert. denied* 430 U.S. 922.

94. 15 U.S.C. § 1012(a) (1976).

95. *Royal Drug* involved an antitrust challenge to a Blue Cross/Blue Shield provider arrangement whereby policyholders were given preferred status at participating pharmacies. Nonparticipating pharmacies challenged the provided agreement alleging it was a price fixing conspiracy. The insurance carrier tried to assert the *McCarran-Ferguson* defense. The Court found, however, that provider arrangements were risk-reducing, not risk-spreading, and that only the latter constituted the "business of insurance" exempt from antitrust liability under the *McCarran-Ferguson* exception. 440 U.S. 205 (1979).

96. *Id.* at 214-215, n.12.

antitrust liability for certain activities which fall within the Sherman Act. Exclusion clauses and decision-making processes about covered technologies are arguably such activities. The health-insurance carrier could be expected to assert that the exclusions (for experimental or investigational services) were necessary to enter into an insurance contract (risk-spreading agreement) and thereby should be considered risk-spreading activity. The Court, however, in *Royal Drug* specifically rejected that argument.⁹⁷ Furthermore, the Court rejected the defendant's argument that activities which resulted in a reduced premium should be considered risk-spreading, specifically distinguishing risk-spreading from risk-reducing.⁹⁸

Merely finding that hospitals and non-risk spreading activities of insurance carriers are not exempt from the Sherman Act does not, of course, establish liability. Section One of the Sherman Act prohibits combinations, contracts, or conspiracies in restraint of trade.⁹⁹ The plaintiff must prove two elements to establish a Section One violation: (1) a "concerted action," and (2) a resultant restraint of trade.¹⁰⁰ Courts apply either the per se rule¹⁰¹ or the rule of reason¹⁰² in deciding whether an activity is a concerted action that restrains trade and, therefore, violative of the Sherman Act.

If a plaintiff could make the showing of a concerted action, a court would likely apply the per se rule, which is conclusory, and would allow the plaintiff to prevail. The health-insurance carriers are highly competitive and it seems unlikely that a plaintiff could make such a showing. It could be argued, however, that the similarities in coverage for bone marrow transplantation amongst the various third-party payers is indicative of an agreement. The same competitiveness that makes agreement unlikely would also mandate the presumption of harmful, illegitimate conduct that underlies the per se rule. Absent evi-

97. "[I]t does not follow that because an agreement is necessary to provide insurance, it is also the 'business of insurance.'" *Id.* at 213-214, n.9.

98. *Id.* at 214-215.

99. 15 U.S.C. § 1 (1976).

100. L. SULLIVAN, ANTITRUST SECTION 109 (1977).

101. *Broadcast Music Inc. v. Columbia Broadcasting Sup.*, 441 U.S. 1, 7, 8 (1979) *rehg. denied* 450 U.S. 1050.

102. *National Soc'y of Professional Engineers v. United States*, 435 U.S. 679, 687-692 (1978).

dentiary support of an agreement, it seems unlikely that a court would hold the similarities in coverage to be conclusive of concerted action.

The application of antitrust law to the bone marrow transplantation services which, it will be remembered, also deny access to medical technology if the bone marrow transplant candidate has no proof of adequate funding, is more feasible. The physicians and other health care personnel who comprise such a bone marrow transplantation service or team are frequently on salary and therefore relatively unconcerned about the source or extent of a bone marrow transplant candidate's funding.¹⁰³ The same cannot be said of hospital administrators who are universally caught between increasing efforts at cost containment by third-party payers and the spiralling cost of medical care. Although to an extent competitive, the hospitals have a uniform concern that unfunded care be kept to a minimum. The cost to the patient of a bone marrow transplant may exceed \$100,000. Additionally, a highly-publicized charitable bone marrow transplantation could open the "floodgates of supplication."

If the plaintiff could make a showing of concerted action, the conduct probably would withstand any balancing required by the rule of reason and be held to be in restraint of trade. The extent to which the facts of the particular case pointed toward agreement among the hospitals would most likely be determinative as the policy considerations required by the rule of reason are equivalent in merit. Although it is obviously repugnant to the concept of justice that hospitals should conspire to deny access to a life-saving treatment, it is also quite untenable that the stability of hospitals be threatened by the financial burden of mandated charitable bone marrow transplants.

In summary, in spite of recent developments in antitrust law, it seems unlikely that third-party payers would be found by a court to be in illegal concert to restrain trade by restricting access to bone marrow transplantation or other medical technologies. On the other hand, hospitals have the economic incentive to deny such coverage and their denial of access to bone

103. Institutional economy is becoming a major issue even in affluent university hospitals and the physicians, at least, are undoubtedly concerned to the extent that unfunded bone marrow transplants may jeopardize institutional viability.

marrow transplantation on the basis of inadequate funding is uniform. Given sufficiently probative evidence of agreement, a court might well sustain an antitrust action brought by an unfunded bone marrow transplant candidate denied access by a hospital.¹⁰⁴

D. Interpretation of a Contract of Adhesion

The courts in California have long looked with a jaundiced eye at contracts of adhesion. This hostility has its foundation in concepts of consumer protection and unequal bargaining strength of contracting parties. In *Schmidt v. Pacific Mutual Life Insurance Co.*, the court defined the adhesion contract as: "[A] standardized contract which, imposed and drafted by the party of superior bargaining strength, relegates to the subscribing party only the opportunity to adhere to the contract or reject it."¹⁰⁵ Although such contracts of adhesion are enforced according to their terms in the absence of ambiguity,¹⁰⁶ courts have been quick to find ambiguity. Insurance contracts are the archetype adhesion contract. The subscriber is verbally told the terms of the coverage, often with more than a little puffery, and subsequently signs a form contract characterized by oblique or obscure language.

The court in *Maxon v. Security Ins. Co.* recited three rules of construction for insurance contracts:

[A]ny ambiguity or uncertainty in the contract is to be resolved against the insurer; if semantically permissible, the contract will be given such construction as will fairly achieve its object of securing indemnity to the insured for losses to which the insurance relates; and if the insurer uses language which is uncertain any reasonable doubt will be resolved against it¹⁰⁷

104. Hospitals which have used Hill-Burton funds to build or expand their physical plants have a mandate to provide community service to an extent equal to six percent of their incomes. An imaginative court could design a remedy directing the preferential use of those funds for bone marrow transplantation.

105. 268 Cal. App. 2d 735, 737, 74 Cal. Rptr. 367, 369 (1968).

106. *Neal v. State Farm Ins. Cos.*, 188 Cal. App. 2d 690, 694, 10 Cal. Rptr. 781, 783 (1961).

107. 214 Cal. App. 2d 603, 611, 29 Cal. Rptr. 586, 590 (1963).

The California Supreme Court has further refined the standards of construction for contracts of adhesion. In *Atlantic Nat. Ins. Co. v. Armstrong*,¹⁰⁸ the court found that the form of an insurance contract must be interpreted in light of the reasonable and normal expectations of the parties with regard to the extent of coverage. And, in *Steven v. Fidelity Gas Co.*,¹⁰⁹ the California Supreme Court found that if an insurer "deals with the public upon a mass basis, the notice of non-coverage of the policy, in a situation in which the public may reasonably expect coverage, must be conspicuous, plain and clear."¹¹⁰

Although these rules of construction set a high standard for the insurer, other California courts have required even more. In *Arata v. Cal. Western States Life Ins.*,¹¹¹ the court set a very high standard indeed. The action pivoted on judicial construction of the contract. In *Arata*, the widow of the insured, a hemophiliac attorney, brought suit to recover under an accident insurance policy which had an exclusionary clause denying benefit if death was contributed to by disease. He slipped and fell, striking his head, and subsequently died a few days later of intracranial hemorrhage. The court found that the deceased had congenital factor VIII deficiency (hemophilia) and would not have died but for the bleeding disorder. The court, nevertheless, found that the factor VIII deficiency did not contribute to death *within the intent of the exclusionary clause* since the accident was the "prime or moving" cause of death. This verdict is difficult to reconcile with even the most rigorous application of adhesion contracts' principles. The decedent was a lawyer with a congenital bleeding disorder of which he was aware. The exclusionary clause was unambiguous. The reasonable expectation of both parties must have been that, should the insured die of an injury which would not have been fatal but for the underlying bleeding disorder, there would be no recovery. The result appears to amount to strict liability for insurance carriers. "It has nothing to do with insurance. It has nothing to do with subjective fault. It has to do with compensation for a loss resulting from a deliberately assigned risk — assigned, that is, to the

108. 65 Cal. 2d 100, 112, 416 P.2d 801, 809, 52 Cal. Rptr. 569, 577 (1966).

109. 58 Cal. 2d 862, 377 P.2d 284, 27 Cal. Rptr. 172 (1962).

110. *Id.* at 878, 377 P.2d at 294, 27 Cal. Rptr. at 182.

111. 50 Cal. App. 3d 821, 123 Cal. Rptr. 631 (1975).

other fellow."¹¹²

A subsequent California Supreme Court case provides an interesting contrast. In *Madden v. Kaiser Foundation Hospitals*,¹¹³ the court enforced an arbitration provision in a medical services contract between the plaintiff's employer and Kaiser. The court explained that the policy behind adhesion contract construction was prevention of the imposition of contract provisions by a stronger party on a weaker. The court found the Kaiser contract in question was negotiated for the plaintiff by representatives of his union who had parity of bargaining strength with Kaiser. There were no oppressive features of the contract and the provisions benefited Kaiser and the members equally. It is interesting to speculate whether this holding results from strong policy considerations in favor of arbitration or is an attempt to limit the virtual strict liability imposed by *Arata*. Although third-party payers have contracts with groups, they also have many individual subscribers. It seems nonsensical to apply different rules of construction to *precisely the same contract provisions* depending on whether the plaintiff is part of a group or an individual member.¹¹⁴

The health-insurance carriers' disclosure forms, as discussed above, contain conspicuous statements of the exclusions. The language is clear and not misleading. A case brought for breach of contract by a plaintiff denied coverage for bone marrow transplantation would turn on the meaning of the words "investigational" and "experimental." These words appear in virtually all health insurance contracts.¹¹⁵ Ironically, as we have seen above,

112. Cowen, *Some Policy Bases of Products Liability*, 17 STAN. L. REV. 1077, 1092 (1965).

113. 17 Cal. 3d 699, 552 P.2d 1178, 131 Cal. Rptr. 882 (1976).

114. A recent California appellate court case, while setting out no new principles for adhesion contract interpretation, illustrates the extent to which courts will enforce the reasonable expectations of the insured. In *Ponder v. Blue Cross of So. Calif.*, the plaintiff was denied coverage by Blue Cross for treatment of temporomandibular joint syndrome. The contract specifically excluded coverage for dental care and within that exclusion denied coverage for temporomandibular joint syndrome. The court found that the term was not comprehensible to lay persons and therefore did not meet the "plain and clear" requirement of *Steven v. Fidelity and Casualty Company*. (Steven, 58 Cal. 2d 862, 377 P.2d 284, 27 Cal. Rptr. 172; *Ponder v. Blue Cross of Southern California*, 145 Cal. App. 3d 709 (1983).)

115. Bunker, *Evaluation of Medical-Technology Strategies*, 306 N. ENGL. J. MED. 624 (1982).

the decision-making processes of the insurance carriers also turn on the same words.

If the Technology-Assessment Committee of Blue Shield or the Bone Marrow Transplant Advisory Committee of Kaiser were to apply the same strict scientific standards to bone marrow transplantation that the FDA applies to the evaluation of a new drug for safety and efficacy, there would be very few covered indications for bone marrow transplantation. The FDA considers (and enforces its decision by withholding the drug from the market) a drug investigational until it has been proven safe and efficacious through rigorous application of the scientific method. This showing of efficacy requires that the drug be investigated in controlled clinical trials by multiple groups, and that these trials, when subjected to a statistical analysis, prove the drug to be efficacious. The determination of safety is a weighted analysis of risk and benefit, taking into consideration the availability of alternative treatments. The drug does not have to be more efficacious than existing drugs on the market.

In contrast, only twenty percent of procedures of medical technologies have been adequately tested by randomized clinical trials.¹¹⁶ Bone marrow transplantation has been proven efficacious in only one controlled clinical trial which evaluated the procedure in patients with aplastic anemia. That study was controlled but not randomized.¹¹⁷ Patients without matched donors served as the controls. Additionally, of course, it is not feasible to "double blind" a procedure such as bone marrow transplantation. Observer bias was excluded by comparing only patient survival, death being an endpoint subject to little observer variation. If health-insurance carriers were to require scientific validation of efficacy, then bone marrow transplantation would be considered not investigational or experimental only as treatment for aplastic anemia.

Most third-party payers also cover bone marrow transplant for acute leukemia in remission. This decision is based on large studies which used as a control survival rates from the literature or their own institution. Bone marrow transplantation for leuke-

116. Lashof, *Government Approaches to the Management of Medical Technology*, 57 BULL. N.Y. ACAD. MED. 36, 39 (1981).

117. Camitta, *supra*, note 37.

mia in remission thus became an "acceptable medical practice" or even "standard medical practice" without adequately controlled studies. Major third-party payers have, at least tacitly, accepted these terms as antonyms for "investigational" and cover bone marrow transplantation for acute leukemia in remission. It is for less common or rare diseases where evidence from well-designed clinical trials is unavailable (or because the relative scarcity of bone marrow transplant candidates with an acceptable donor will take many years to accumulate) that coverage is more likely to be denied. In many instances, bone marrow transplant centers are transplanting patients with these diseases regularly, yet there are no published reports of the results (or at best, a few case reports). In most cases, experts would agree that attempted treatment with bone marrow transplantation is reasonable. On the other hand, most, if not all, disinterested experts would agree that the efficacy of the application is unproven. This dichotomy is the nexus of the dilemma. Third-party payers who insist on at least published evidence of efficacy¹¹⁸ will lag behind enthusiasts in their coverage of the application of bone marrow transplantation.

The patient who becomes a bone marrow transplant candidate, when a new application for bone marrow transplantation is under investigation, is the most likely to have coverage denied and, at the same time, to be an insured who has a 'reasonable and normal expectation' of coverage.¹¹⁹ If the court took the position of the *Arata* court,¹²⁰ almost any construction of the term 'experimental' would probably lead to plaintiff recovery. The only defense the carrier would have to recovery would seem to be the introduction of a "patient consent to investigational therapy" form if the bone marrow transplant center required the patient to sign such a release. This would, of course, serve to evidence notice and concurrence on the part of the plaintiff, as well as impeach the expert witness. The court in *Ponder*, however, found that the time for interpretation of an adhesion contract is at acceptance, "not when the incident triggering coverage first

118. Many experts, cognizant of the proliferation of medical technology without collected study, would require that an expensive and high risk (40% mortality) procedure like bone marrow transplantation be shown to be more efficacious than existing treatment before deeming it the standard of practice for a given indication.

119. *Armstrong*, 65 Cal. 2d at 112, 416 P.2d at 809, 52 Cal. Rptr. at 577.

120. 50 Cal. App. 3d 821, 123 Cal. Rptr. 631.

arises."¹²¹ In spite of the arcane neologisms in the court's conclusion,¹²² the reasoning in the body of the opinion is sound¹²³ and other courts are likely to take a similar view of defenses arising concurrently with the need for coverage. A defense based on a plaintiff's knowledge or understanding at the time of treatment, therefore, is likely to be futile.

On the other hand, if the court were to find that the insurance contract was not a contract of adhesion, then coverage might well depend on which measure of "non-investigational" the court adopted. If the court accepted a likely defense assertion that a technology must be scientifically proven to be safe and effective to be deemed non-investigational, a plaintiff seeking coverage or recovery for bone marrow transplantation done for a rare indication would probably be denied coverage. If, on the other hand, the probable assertion of the plaintiff that a "medically acceptable" measure of "non-investigational" be applied, both the plaintiff and defense could assemble an impressive panel of expert witnesses.

The plaintiff's case might turn on whether he or she was a group or individual subscriber to the health-insurance contract. Although the *Madden*¹²⁴ court emphasized this difference, the announced policy basis underlying the decision was the parity of bargaining strength between the parties. It could be successfully argued that although the extent of coverage, in regard to special inclusions, may vary between individual and group subscribers, the general extent of coverage and the exclusionary clause for experimental procedures are identical. The defense could certainly assert that this identity of terms makes the individual subscriber the beneficiary of the bargaining strength of the group subscriber. It seems likely that both the *Madden* and *Arata* decisions arose primarily from policy considerations.

The outcome of a breach of contract action based on interpretation of the term "experimental" in an adhesion contract would also be likely to turn on policy considerations. The court,

121. 145 Cal. App. 3d at 727.

122. "We diagnose the exclusion of this contract to suffer from 'complexified occultation clause syndrome' [and] [t]hat malady alone is fatal. . . ." *Id.* at 728.

123. *Id.* at 726, 727.

124. *Madden*, 17 Cal. 3d 699, 552 P.2d 1178, 131 Cal. Rptr. 882 (1976).

concerned with consumer protection, would have to balance benefit to the individual of construction of the contract against the insurer, with the risk to society (both economically and medically) of the proliferation of unproven medical technology.

If the bone marrow transplantation was undertaken on the basis of a logical extension of current applications in a patient who had exhausted known therapy, the plaintiff's case is much more difficult. If the court considered the contract one of adhesion and took the *Arata* approach, the plaintiff might recover. That is, although the bone marrow transplantation was proven neither efficacious nor "standard medical practice," the court could find that the bone marrow transplant was not investigational *within the intent of the exclusionary clause*. The court in *Arata* did precisely that by finding that the death of the plaintiff's deceased would not have occurred but for his hemophilia while holding that his death was not contributed to by a disease within the meaning of the exclusionary clause. The plaintiff's deceased in *Arata* was an attorney and, therefore, bargaining parity could be presumed. The policy considerations that buttress the opinion are not easy to extract. Nevertheless, a similar "construction" of the terms of a bone marrow transplant plaintiff's contract might result in recovery.

Under any other construction, whether or not the court found the contract to be one of adhesion, it is difficult to see how a plaintiff might argue that his transplant was not investigational. If bone marrow transplantation had been successfully undertaken once before in a similar situation, the plaintiff could argue that one success was sufficient to show that bone marrow transplantation could work. Therefore, the procedure was not an experiment, and the only real question was how often it would work.

In summary, the plaintiff with health insurance or Medicaid may well be able to get relief under an adhesion contract theory. If the bone marrow transplant was done for an uncommon condition with small numbers of patients available for study, and had been successfully done at more than one center, the court would likely conclude that the procedure was not experimental within the expectations of the insured. If the indication was one covered by some third-party payers and not others, the plaintiff

should prevail with little difficulty. At the other end of the spectrum, if the bone marrow transplantation were the first or second one performed for the plaintiff's condition or if all others had been unsuccessful, the plaintiff's only remedy would be in the *Arata* approach or under strict liability.

E. Strict Liability in Health Care

The *Arata*¹²⁵ case suggests that a plaintiff seeking recovery for denied bone marrow transplantation coverage plead a cause of action in strict liability. Strict liability concepts, although rooted in the implied warranty doctrine of sales contracts, have been widely applied by the courts. Although most frequently utilized in products liability applications and sales contracts, the principles of strict liability have also been applied to other types of transactions. In *Holmes Packaging Mach. Corp. v. Bingham*,¹²⁶ implied warranty was held applicable to bailment contracts. The courts in California have distinguished contracts for services from contracts for goods and labor. "[T]he well settled rule in California is that where the primary objective of a transaction is to obtain services, the doctrines of implied warranty and strict liability do not apply."¹²⁷ Strict liability has been denied for various contracts for professional services.¹²⁸

*Aced v. Hobbs-Sesack Plumbing Co.*¹²⁹ provides a foundation for extension of strict liability to insurance contracts. *Aced* involved a suit by a general contractor against a subcontractor (by cross-complaint), seeking damages for a faulty heating system which leaked after installation. The California Supreme Court held that even absent the statutory law applicable to sales contracts, "similar warranties may be implied in other contracts not governed by such statutory provisions *when the contracts are of such a nature that the implication is justified.*"¹³⁰ The court applied the doctrine of strict liability in *Pollard v. Saxe*

125. 50 Cal. App. 3d 821, 123 Cal. Rptr. 631 (1975).

126. 252 Cal. App. 2d 862, 60 Cal. Rptr. 769 (1967).

127. *Allied Properties v. John A. Blume & Associates*, 25 Cal. App. 3d 848, 855, 102 Cal. Rptr. 259, 264 (1972).

128. See *Roberts v. Karr*, 178 Cal. App. 2d 535, 3 Cal. Rptr. 98 (1960) (surveyor services); *Gautier v. General Telephone Co.*, 234 Cal. App. 2d 302, 44 Cal. Rptr. 404 (1965) (communications services); *Shepard v. Alexian Bros. Hosp.*, 33 Cal. App. 3d 606, 109 Cal. Rptr. 132 (1973) (blood transfusion).

129. 55 Cal. 2d 573, 360 P.2d 897, 12 Cal. Rptr. 257 (1961).

130. *Id.* at 582, 360 P.2d at 902, 12 Cal. Rptr. at 262 (emphasis added).

and *Yolles Dev. Co.* to real property construction contracts.¹³¹ In so doing, the court restated the policy considerations underlying the doctrine of implied warranty in sales contracts and found similar policy considerations in construction contracts. "The doctrine of implied warranty in a sales contract is based on the actual and presumed knowledge of the seller, reliance on the seller's skill or judgment, and the ordinary expectations of the parties."¹³² Taken together, *Aced* and *Pollard* suggest that the court is willing to extend strict liability when there are sufficient parallels with the policy considerations reiterated in *Pollard* to "justify" extension. In a health insurance contract, the purchaser is faced with a seller of superior knowledge on which the purchaser must rely to achieve his ordinary expectation that should he become sick, the economic costs will be insured. At issue in any attempt to extend the doctrine of strict liability to health insurance contracts would be the nature of the contract. The insurer would try to characterize the transaction as one having the primary objective of obtaining health care and, thereby, bring it within the language of *Allied Properties*.¹³³ *Allied Properties*¹³⁴ involved the professional services of a marine engineer in the structural design of a pier. In declining to find strict liability the court reasoned that "the general rule is . . . that those who sell their services for the *guidance* of others in their economic, financial, and personal affairs"¹³⁵ are not liable absent negligence or intentional misconduct.

In a health insurance contract, the service sought is risk-spreading — there is no element of guidance. When the insured enters the contract he or she is not seeking health care, but rather assurance that should such care become necessary, economic disruption will not result. Holding health-care insurers strictly liable on the theory of an implied warranty of suitability for a particular purpose would be a logical extension of the law. The non-parity of knowledge and bargaining power of the parties suggest that overreaching would be as likely here as in a

131. *Pollard v. Saxe & Yolles Dev. Co.*, 12 Cal. 3d 374, 525 P.2d 88, 115 Cal. Rptr. 648 (1974).

132. *Id.* at 379, 525 P.2d at 91, 115 Cal. Rptr. at 651.

133. *Allied Properties v. John A. Blume & Associates*, 25 Cal. App. 3d 848, 855, 102 Cal. Rptr. 259, 264 (1972).

134. *Id.*

135. *Allied*, 25 Cal. App. 3d at 856, 102 Cal. Rptr. at 264 (emphasis added).

sales contract. The ordinary expectation of the insured is that his medical bills will be paid. A court in California might well hold that these conditions could make the contract "of such a nature that the implication"¹³⁶ of implied warranty is justified.

A characterization by the courts of an insurance transaction as one primarily for services would not necessarily bar recovery in strict liability. The courts in California have found that contracts for professional services do not have an implied warranty.¹³⁷ Although the contract itself is fiscal and, as analyzed above, has the characteristics which justify strict liability, the entire transaction could conceivably be characterized as aimed at assuring access to medical care. A strong argument could be made, buttressed by the inability of the plaintiff to obtain bone marrow transplantation without funding, that the insured intended to obtain medical care (albeit a need which had not yet and might never arise) when he or she contracted with the health insurer. This apparently plaintiff-oriented argument would have, in California, the paradoxical result of at once stating the equitable principles underlying recovery in strict liability and, at the same time, deny that recovery. A court seeking to indemnify the bone marrow transplant candidate might be willing to extend strict liability while viewing the transaction as one intended to obtain medical services. Although this would be a case of first impression in California, courts in Texas have extended strict liability directly to health care providers.¹³⁸

VI. POLITICAL REFORM

A. Catastrophic Health Insurance

While the courts are uniquely suited to provide remedies for individual plaintiffs wrongly denied access to bone marrow transplantation (or any other medical technology), the case by

136. *Aced v. Hobbs-Sesack Plumbing Co.*, 55 Cal. 2d 573, 582, 360 P.2d 897, 902, 12 Cal. Rptr. 257, 262 (1961).

137. *Allied*, 25 Cal. App. 3d at 848, 102 Cal. Rptr. 259.

138. *Providence Hospital v. Truly*, 611 S.W. 2d 127 (1980). In *Truly*, the appellate court upheld a jury verdict finding a hospital liable to a patient under a breach of warranty action for the intraoperative injection of a contaminated drug.

In *Thomas v. St. Joseph's Hospital*, the plaintiff was severely burned when his hospital gown was ignited by a lighted match he had dropped. The appellate court found that a hospital could be held liable in strict liability for supplying a defective gown and remanded the issue to the trial court. 618 S.W. 2d 791 (1981).

case adjudication of what procedure is or is not experimental, and thereby, is or is not covered by the third-party payer, is probably not the best way to assess medical technology. This is especially true when one contemplates that decisions made on the basis of strict liability or adhesion contract principles do not address the validity of the technology or its application. Furthermore, access to legal resolution is not without denial. Finally, the pyrrhic legal victory that flows to the bereaved is not restitution.

These considerations have led some commentators to call for catastrophic health insurance.¹³⁹ Generally, proponents of catastrophic health insurance have in mind an economically defined catastrophic illness.¹⁴⁰ Thus, an immediately fatal illness which would incur few medical bills would not be included within the definition of a catastrophic illness. Although politicians emphasize that such plans would provide coverage for the poor, they are also capable of being incorporated into existing insurance systems by mandating that the employers cover employees with a plan meeting minimum standards.¹⁴¹ The economic problems of such plans are beyond the scope of this article; suffice it to say that budgetary demands would require limits. Those limits would have to have a rational basis if the ethical concerns that spawned the concept of catastrophic health insurance were to be maintained.

The only rational basis for coverage decision would be to fund all medically necessary treatments which were not experimental or investigational (presuming the definition of catastrophic illness). The decision then would involve the definition of the very words which determine coverage under current third-party decisions.¹⁴² Although one might expect a federally funded insurance program with a broad legislative mandate to construe the requirement of medical necessity widely and the experimental exclusion narrowly, the experience with bone marrow trans-

139. Havighurst, *Strategies in Underwriting the Cost of Catastrophic Disease*, 40 *LAW AND CONTEMP. PROB.* 122 (1976).

140. *Id.*

141. *See generally*, Note, *Catastrophic Health Insurance and Cost Containment: Restructuring the Current Health Insurance System*, 6 *AM. JOUR. LAW & MED.* 83 (1980).

142. *See* discussion of statutes and exclusionary clauses *supra*, pages 228-31.

plantation does not support that supposition. Of the major third-party payers, Medi-Cal covers bone marrow transplantation for the fewest indications. California Children's Services (CCS), with its requirement that the family have an adjusted gross income of less than \$40,000,¹⁴³ comes very close to catastrophic health insurance in design and operation. It is interesting to note that, although using the same criteria for coverage decisions as Medi-Cal, CCS covers bone marrow transplantation for many more indications. One presumes that this coverage arises out of empathy for children and not the advocacy skills of physicians and lawyers working with higher income families.

In spite of the admirable goals of catastrophic health insurance, fiscal realities and ethical imperatives dictate that coverage decisions would have to be made. The risk-spreading aim of insurance is to spread costs over large segments of the population — not to spread the risk of experimental procedures to all segments of the population.

B. Medical Technology Assessment

Current practices by third-party payers may result in variable or denied coverage. Judicial remedies, if sought after denial of access, are inadequate. Furthermore, judicial remedies, if relied on to make social change, could have untoward results. Courts might give individual plaintiffs declaratory relief.¹⁴⁴ This relief would have to be founded on adhesion contract or strict liability principles and would thus, in effect, adjudicate a finding of "safe and efficacious" without the necessary scientific evaluation. Because of cost constraints, catastrophic health insurance could not, *and should not*, cover any application of medical technology without an administrative determination of the safety and efficacy of the treatment.

Individual plaintiffs denied access to bone marrow transplantation will, and often should, seek legal remedy. Irrespective of any societal developments, plaintiffs will continue to find themselves with an illness for which bone marrow transplantation, for example, has been recommended by a physician and

143. CAL. HEALTH AND SAFETY CODE § 255 (West 1980).

144. This, of course, presumes that the disease being proposed has a predictable natural history which progresses slowly enough to allow declaratory relief.

denied third-party coverage. There will always be indications undergoing evaluation, the efficacy of which will lie somewhere along the continuum from unknown to proven. Unfortunately, there will also always be the technologies whose place along the continuum depends on how one defines the endpoint. The great majority of medical technologies, accepted by physicians, patients and third-party payers alike, have not been proven to be safe and efficacious by rigorous application of the scientific method.¹⁴⁵ Therefore, if one uses the "standard of practice" or "generally medically accepted" definitions, these procedures are proven. If one uses the "safe and efficacious" definition, these procedures are not proven.

With the rapid development of medical technology, some sort of formal mechanism for assessing medical technology seems imperative. The National Center for Health Care Technology (NCHCT) was established in 1978¹⁴⁶ for that purpose. The National Council on Health Care Technology was established by the same law to act as an advisory body.¹⁴⁷ It was made up of eighteen members, including six scientists, two physicians, two hospital administrators, two lawyers, one ethicist and three persons from the general public.¹⁴⁸ The NCHCT was not funded for 1982 and thereby ceased to exist.¹⁴⁹ Although the agency did not fund the basic research, it collected available data on a particular technology, made recommendations for the generation of new data if it was needed, and sought a consensus concerning the particular technology. The results of the assessment were then published.¹⁵⁰

The establishment of a private institute to evaluate new technologies has been proposed.¹⁵¹ The intent is for this Institute for Health-Care Evaluation (IHCE) to be composed of groups representing a broad cross-section of health-care inter-

145. Lashof, *supra*, note 116, at 39.

146. Health Services Research Health Statistics, and Health Care Technology Act of 1978, Pub. L. No. 95-623, 92 Stat. 3443 (1979) [hereinafter cited as N.C.H.C.T.].

147. Perry, *The Brief Life of the National Center for Health Care Technology*, 307 N. ENGL. J. MED. 1095 (1982).

148. *Id.*

149. *Id.*

150. N.C.H.C.T., *Technology Assessment Forum, Coronary Artery Bypass Surgery*, 246 J.A.M.A. 1645 (1981).

151. Bunker, *supra*, note 115.

ests. Technology assessment would be one of the goals of IHCE. The proponents of an IHCE envision it as a non-profit corporation which would assume some of the functions of the former National Center for Health Care Technology. Because the insurers would benefit from having a formal non-interested group provide guidelines for coverage decisions on new technologies, they might be expected to at least partially fund the organization.

The organization would not necessarily do any research on its own. It would, however, be hoped that by collecting and analyzing data as it is generated by major centers, the institute could make recommendations concerning where along the continuum of evaluation that a medical technology lies. This would serve not only to assist third-party payers in deciding when or whether to cover new technologies, but would identify areas where more research is needed before a technology can be considered not investigational. The argument that there is a need for scientific evaluation of new medical technology was developed extensively above through the vehicle of tracing the indications for bone marrow transplantation. There are, unfortunately, several recognized problems with the development of a private institute for health care evaluation.¹⁵²

At the outset, although the institute would have no official regulatory functions, its recommendations would be strongly probative in a courtroom. Consequently, third-party payers, whether private or government, may be less than enthusiastic about supporting such an institute — fearing that coverage decisions would in effect be taken from them. The *Royal Drug* case¹⁵³ raises serious concerns about whether such an institute would be open to an antitrust action. It is uncertain whether a court would consider such activities risk-spreading and thus uphold a *McCarran-Ferguson* defense,¹⁵⁴ or find them aimed at risk-reducing. The concerted action element would be manifestly present.

152. See generally, *id.*

153. *Group Life Insurance Co. v. Royal Drug*, 440 U.S. 205 (1979), discussed *supra* at pages 238-39.

154. 15 U.S.C. § 1011 (1976).

The current publicity concerning children denied bone marrow transplantation on the basis of a coverage decision has resulted in demands for a political solution.¹⁵⁵ The solution generally sought is a funding guarantee for procedures which are not covered by third-party payers because they are considered experimental. As the discussion of catastrophic health insurance demonstrates, this "political solution" would still require some decision as to which procedures are experimental and which are not. This coverage decision is mandated from both ethical and fiscal considerations so that no degree of cost-shifting can obviate the need to make such a decision. On the other hand, the concept of a private institute to evaluate new technologies is an alluring one. It squarely faces the necessity of evaluating new technologies for efficacy and the moral imperative that coverage availability (and thereby, actual availability for an expensive procedure such as bone marrow transplantation) not depend on which third-party payer the insured selects or must accept.

VII. CONCLUSION

Access to medical technology is currently being denied many patients. In the instance of bone marrow transplantation, where all the indications have a fatal outcome if not transplanted, the case for a thorough evaluation is manifest.

The presumption is that if a physician recommends a bone marrow transplantation, and a transplant center is willing to perform it, that any denial of access based on a coverage determination is an affront to justice. This presumption is based on a further presumption that the treatment is efficacious. This determination of efficacy, however, is rarely made on the basis of the scientific method when new medical technologies are under development, resulting in technologies being applied enthusiastically by medical innovators without any demonstration of efficacy. At this stage in the development of a new technology, experts may disagree about the indication for application of a technology such as bone marrow transplantation. "Experts usually disagree not so much about the objectively verifiable facts, but about the inferences that can be drawn from the facts."¹⁵⁶ A

155. See *supra*, p. 233. (Parents' Action for Children's Cancer Treatment, for example).

156. Bazelon, *supra*, note 2, at 827.

patient who has an illness which can be treated with bone marrow transplantation, who has exhausted other medical treatment, will very likely have a bone marrow transplantation recommended by his or her physician. Coverage is equally likely to be denied by the third-party payer on the basis that the application is investigational. Such a patient's denial of access is amendable to legal resolution. A court would probably find for the plaintiff on the basis of strict liability or adhesion contract principles. If declaratory relief were given, the remedy would be legally adequate. The real dilemma is whether a bone marrow transplant is in the patient's best interest.

[We] are all becoming increasingly conscious of the extent to which many supposedly scientific or technical decisions involve painful value choices, and pose difficult policy problems. We have come to realize that virtually every technological innovation may carry unwanted consequences, and that technological progress may therefore cause, as well as solve, critical societal problems.¹⁵⁷

This warning applies to the patient who may be harmed by the unproven therapy in attempts to offer him or her every treatment that might be effective. It is equally applicable to society in general. Because of fiscal limitations which are finite regardless of politicized budgetary philosophies, "purchase of care which is ineffective or of undocumented efficacy for some patients will almost certainly result in a failure to provide effective care to other patients."¹⁵⁸

The need is for critical scientific assessment of medical technologies to allow third-party payers, referring physicians and patients to make rational decisions. Many applications of new technologies, such as bone marrow transplantation for infantile osteopetrosis, will never be undertaken in sufficient numbers to allow rigid statistical validation. Whatever body evaluates such applications will have to retain a "generally accepted" standard in the evaluation process if ethical decisions are to be made.

The concept of a private institute for medical technology

157. *Id.* at 819.

158. Bunker, *supra* note 115, at 691.

evaluation has much to recommend it. It would require more cooperation amongst health-insurers, medical institutions and governmental agencies than has been seen in the past. It remains to be seen whether the crises of rapidly expanding, inadequately evaluated medical technology can foster such cooperation.

By whatever method medical technology is distributed, the legal system will retain a role. The individual plaintiff who needs a bone marrow transplant for an indication not proven efficacious will always need to seek legal redress regardless of how the technology assessment was made. Courts, in their scrutiny, can do much to assure that the decision-making process is rational. The striking similarity of coverage and exclusion determinations for bone marrow transplantation by third-party payers suggests that these determinations have been made on a rational basis to date. The pressing need is for critical scientific evaluation of new applications of bone marrow transplantation and other technologies as they emerge.

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