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Implications for Policy

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In summary, the confluence of technological advances in apheresis equipment and recent scientific research linking many chronic disease conditions to immunological dysfunction has served to expand dramatically the number of apheresis procedures in the past 10 years. Therapeutic apheresis has exhibited many of the classic features that have come to characterize the hopes, concerns, and fears about medical technologies over the last three decades.

Utilization and diffusion of therapeutic apheresis seems to have closely followed Warner's (141) "desperation-reaction" model. Initial rapid diffusion has occurred in the absence of safety and efficacy evidence. The rapid diffusion is due in part to a lack of a suitable alternative technology, in part to claims—some of them dramatic—of the technology's beneficial effects, and in part to desperation on the part of patients and of providers responsible for treatment. In chronic and life-threatening situations, apheresis has found its broadest and most frequent application.

Most recently, however, the lack of well-validated clinical evidence has influenced provider behavior. Ambiguous results have given rise to physician caution, while lack of evidence and high costs have provoked increased regulation by medical insurers, possibly slowing diffusion. Best estimates are that utilization and diffusion have plateaued, at least for the present. The future of therapeutic apheresis seems predictable in that increases or declines in use will be predicated on newly available evidence (35,95).

Several recurring issues in need of further study and resolution have run through the examination of therapeutic apheresis. One issue, given the current state of this technology and many unanswered questions about patient criteria for use, is what constitutes the appropriate timing of intervention in the course of a disease and whether the procedure to be followed in performing therapeutic apheresis is adequately standardized. Such questions are basic in the development of the tech-

nology, and research to address them is necessary, as it forms a foundation for the conduct of well-controlled testing and clinical trials. The Apheresis Panel of the American Medical Association's Council of Medical and Scientific Affairs has recently discussed the idea of a national apheresis registry that would track use and form a knowledge base for development of well-controlled studies (32). On a smaller scale, the American Red Cross has requested its regional blood services to register all apheresis patients at the onset of treatment and report treatment methods and results upon completion (1.21).

A second issue, which arises where conditions of use have been sufficiently standardized, is the lack of well-designed research studies and the need for such undertakings. There have been at least two obstacles preventing the accumulation of valid evidence of safety and efficacy: the ethics of providing sham apheresis or conventional therapy for control group patients, and the high costs of such trials. Long hours of sham apheresis procedures, while possibly inflicting on control group patients some of the same side effects of apheresis as treatment group patients, has led to questions of the ethical implications of such trials. Furthermore, in life-threatening or severely debilitating situations, doctors feel they cannot ethically deny apheresis therapy to control group patients.

The obstacle of costs of well-designed studies has been partially offset by a recent infusion of Government and foundation funding. Should costs continue to be a problem, one alternative might be to have third-party payers, including Medicare, selectively reimburse for therapeutic apheresis in return for clinical data. If implemented properly, this alternative could substantially increase the quality of information available for public and private reimbursement coverage decisions. Evidence of the technology's cost effectiveness could result in yielding substantial budgetary savings. Even if the results of such trials were disappointing, they could lead the way to unexpected advances in research (47).

Because of the promise of apheresis for certain disease complications, this technology would appear to be a particularly choice candidate for such a policy course. In such conditions as Goodpasture's syndrome, for example, effective alternative therapies are very limited and the disease is frequently fatal. Because apheresis has been claimed effective, selective reimbursement could be of great utility from both research and clinical standpoints.

There would be problems in implementing this alternative (see e.g., 104), primarily concerning the legal and ethical implications of selectively reimbursing for health care. It seems clear, however, that third-party payers could use this approach to encourage less costly and more effective forms of treatment. In the case of Medicare, too, elements of the Public Health Service could be involved in developing research protocols and in interpreting research evidence from the resulting experiments.

A recent precedent exists for third-party payer participation in clinical trial funding for apheresis. Five Midwestern State or local Blue Cross/Blue Shield groups and other third-party payers have agreed to reimburse five centers involved in a randomized clinical trial of apheresis for multiple sclerosis. Both the investigational procedure and a sham procedure are covered. Medicare and the State Medicaid groups, on the other hand, are not participating, but administrative and other research costs of the trial are being funded through a National Institutes of Health grant (97).

The Arthritis Foundation and the National Multiple Sclerosis Society are also sponsoring a meeting (to be held in July 1983) at which they hope to develop proposals for third-party payer participation in funding other clinical trials. Representatives of both private and public insurers will be participating (97).

A *third* issue is the possibly transitional nature of this technology. Some major new hardware developments are now undergoing clinical tests. These use adsorption columns and membranes that work like molecular sieves. When a specific fraction whose removal is desired can be identified, an adsorption column containing an antibody to that fraction can remove it from the plasma as

it passes through. Another method, resembling hemodialysis, passes the blood across a membrane with a specific antibody attached to it. A third technique uses a membrane filter to remove fractions of a specific molecular weight (80).

These advances in equipment may, in the course of the next decade, be reinforced or even overshadowed by advances in basic biomedical research or in emerging parallel developments such as biotechnology. The National Cancer Institute, and the National Heart, Lung, and Blood Institute, for example, are currently supporting strategies for the separation of complex blood proteins. Advanced separation technologies could make it possible to index most human proteins. Once proteins are displayed and distinguished from one another, investigators might then tease out individual functions and relate them to the DNA code. Other activities could include the detection of abnormal protein patterns in disease states (e.g., leukemia), and the corresponding production of preventive or neutralizing elements (e.g., monoclonal antibodies) to these noxious or damaging processes (53,60).

In the final analysis, such a state of scientific and technological flux has important policy implications. Therapeutic apheresis, as a medical intervention, falls into a category of medical technologies classically referred to as half-way *technologies* (133). These are generally treatments directed at correcting the effects of a disease or palliating them. It has been pointed out and illustrated repeatedly in the literature and research community that such measures are less satisfactory and more costly than so-called *definitive technologies*, which effectively prevent or control a disease or condition (e.g., poliomyelitis vaccine). As Robbins (116) and numerous others have asserted, "where alternatives exist, resources should be directed so as to encourage the development of definitive technologies as opposed to half-way measures." To the extent that such alternatives can be identified, considerable attention should be given to the possibility of devoting resources to their development. Indeed, one of the critical, ongoing policy issues in medicine is how to establish the most rational and productive balance between development and support of half-way technologies and that of basic research toward definitive technologies.