THE LAST WORD

BIOCARE: PUBLIC POLICY ISSUES FOR AN EMERGING INDUSTRY

by Michael Osband and Gary Cashon

Contrary to common wisdom, biotechnology's most important contribution to healthcare has not been in new diagnostics and therapeutics. Instead, it is the impact of being able to provide customized, patient-specific medical treatment—what we have coined "biocare." Biocare represents a departure from the traditional view of biotechnology as a source of standardized products and reagents. Most biocare involves the therapeutic use of living cells, in which every treatment dosage is custom prepared for each patient: *in vitro* fertilization; bone marrow transplantation; autolymphocyte therapy; somatic gene therapy; use of laboratory-grown skin; implanted "neuronal bridges" to aid healing in spinal cord injury; and transplanting pancreatic islet cells for treating diabetes. This new form of medical care presents considerable challenges to the existing health care system.

In several areas, biocare differs significantly from the traditional practice of medicine: It imposes special conditions on a number of familiar processes, among them validation, diagnosis, reimbursement, administration, and regulation. For now, we will consider only the last:

How Should Biocare Be Regulated?

The Food and Drug Administration (FDA) has the statutory authority in the United States to regulate the manufacture and sale of drugs, biologicals, and medical devices. It approves the sale of such products only when they are proven to be both safe and effective. The FDA does not regulate the clinical practice of medicine. In the surgical suite, for example, the FDA regulates anesthetic drugs, the masks, and the quality of packaged scalpel blades. It does not regulate the surgeon performing the surgery. Instead, the specific surgical techniques used and the clinical indications for these procedures are controlled by other forces, including hospital practice committees, peer pressure, medical-legal practice concerns, and state medical licensing boards.

Why doesn't the FDA regulate biocare? There are four possible answers.

First, although biocare involves the use of sophisticated biotechnology, it has more in common with the clinical practice of medicine than it does with the manufacture of drugs and devices; FDA's position on biocare is fully consistent with its policy not to regulate the practice of medicine.

Second, biocare is for the most part not yet a commercialized activity. Note, however, that this dearth of commercial activity is rapidly changing; there are already several companies providing biocare to patients.

Third, biocare involves very complex issues that arouse intense emotional and political reactions in both the general public and the medical community. The FDA already faces significant public and political pressure to approve new drugs and devices. Why voluntarily take on a new burden?

Finally, FDA, like many federal agencies, generally reacts to developments, rather than involving itself prospectively. Therefore, the lack of biocare regulation by the FDA may not represent a final policy decision, but instead, a "wait-and-see" approach.

What position should the FDA take with regard to biocare? Clearly, there are aspects that deserve regulatory scrutiny and improved quality control. The specific biotechnological methods used in various biocare treatments, however, will make it impossible to regulate this form of medical treatment in a manner comparable to the regulation of manufactured drugs and devices. It would be impossible to regulate as drugs the activated lymphocytes generated for the adoptive immunotherapy of cancer, or the sperm and ova used for *in vitro* fertilization, or the stem cells infused in bone marrow transplant: each of those cell types is prepared separately for each individual patient. They cannot be tested prior to actual use.

We believe, then, that biocare is the practice of medicine. We suggest that the FDA should regulate the devices and reagents used in the bioprocessing of living cells for therapeutic use, but not the living cells themselves. For example, in the case of adoptive immunotherapy for cancer, the FDA should regulate the culture media, culture bags, pheresis machines, and infusion tubing, but not attempt to regulate the infused cells themselves. Moreover, we believe that it would be in the interest of patients and the general public, if the bioprocessing laboratories involved in preparing cells were regulated—though not necessarily by the FDA. State and local authorities might do this more efficiently.

Moreover, all the processes used to prepare the cells or cell products used in biocare should be performed in accordance with the Good Manufacturing Practices (GMP) established by the FDA for manufacturing conventional pharmaceutical products. The goal of GMP in the bioprocessing lab is to create a Bioprocessing Master Record (BMR). The BMR should contain information on the processed cells analogous to that currently required with conventional drugs and products including the cell specifications, complete bioprocessing procedures, quality control testing, quality assurance requirements, labelling, and the proper handling and usage of the processed cells. While we do not believe that the FDA should specifically regulate the cells used in biocare as a drug, we believe that creating a Bioprocessing Master Record will be of immeasurable help in insuring the safe and effective use of biocare.

Michael Osband is director of the Joint Clinical Immunotherapy Program of Boston University School of Medicine (125 Parker Hill Avenue, Boston, MA 02120) and the New England Baptist Hospital, and Chief of the Division of Pediatric Hematology-Oncology of the Department of Pediatrics of Boston University School of Medicine and Boston City Hospital. He is also chief technical officer and executive VP of Cellcor Therapies. Gary Cashon is chairman of the board of directors of Cellcor Therapies, and managing partner of Hillman Medical Ventures. These opinions are the authors' own, and are not necessarily those of *Bio/Technology*.