BIOTECH SAN FRANCISCO

A NOVEL PROPOSAL TO SPEED CANCER DRUG TRIALS

SAN FRANCISCO—"The current system of getting anti-cancer drugs and biologics through clinical trials and into the market simply isn't working," according to Eugene Rothman, vice president for research at Alex. Brown & Sons (Boston, MA). Rothman, who spoke at Online's conference in November, says that Phase I, II, and III clinical trials take too long, and "too many patients are denied the chance to receive experimental therapies." He stresses that the patients chosen for Phase Is are terminally ill and have failed other therapies; any data gleaned from these trials may be difficult to interpret. As for safety, Rothman asks, "How are you really going to tell whether IL-2 is safe or not in Phase I trials when the patients are so desperately ill?"

Rothman believes the traditional system is failing because it is dominated by caution. Protecting patient safety and limiting use to strictly defined indications are stressed over the potential of helping more cancer victims. Although this is an appropriate approach for most drugs, for the new anti-cancer drugs, Rothman argues, "the need for effective therapy is so large that it simply isn't appropriate to have a regulatory system that is dominated by this concern."

Rothman notes that the current regulatory process makes it very expensive and time-consuming to bring a new drug to market—a minimum of 3–5 years at a cost of $50 million. For this reason, many of the smaller pharmaceutical houses and biotechnology firms become discouraged about developing anti-cancer drugs. Only the large pharmaceutical companies, he adds, have the money and the resources to see many of these new drugs through.

Rothman proposes that "we scrap the entire Phase I, II, III process and really rethink the problem from the beginning." His objective is "to get promising new therapies to as many patients as quickly as possible." He claims this could be achieved by specifically designating a group of medical centers to experiment with novel procedures. These centers would not necessarily be limited to major nonprofit medical institutions; any company or institution that met guidelines established by the oncologists, the Food and Drug Administration (FDA), and the National Cancer Institute (NCI), could potentially participate. And it should be the oncologists who lead the way—not the drug sponsor and FDA, as is now the case. The oncologists would decide which drugs to use, design the protocols, evaluate the results, and change the course when needed; FDA and the drug sponsor would assume advisory roles. This would put the control in the hands of the people who are actually treating the patients.

Once the drug has completed trials, the FDA can review it and make it generally available to the population if it is safe and efficacious. This approach will also achieve medical acceptance of a new drug in concert with regulatory approval.

Rothman feels that this approach—in conjunction with prompt publication of data—will move therapies to market more quickly. He is the first to admit that the proposal is going to be controversial and subject to improvement—but it's a place to start.

—Jennifer Van Brunt