

FROM THE EDITOR-IN-CHIEF: “REGULATORY REFORM AT A CROSSROADS”

KENNETH I. KAITIN, PhD

Editor-in-Chief, *Drug Information Journal*

THERE HAS BEEN enormous change in the regulatory environment for pharmaceutical innovation within the past 10 years that is creating new opportunities—and new challenges—for pharmaceutical and biotechnology firms. Harmonization efforts in Europe, Japan, and the United States herald a new spirit of cooperation and industry-agency partnership globally. Through the International Conference on Harmonization (ICH), the three major pharmaceutical markets have standardized many technological requirements for new drug approval in the safety, quality, and efficacy areas. Moreover, finalization of a Common Technical Document (CTD) will allow sponsors to produce a technical information package, with standardized content and format, that can be submitted to all three ICH regions.

Perhaps nowhere has the change in the regulatory environment been more dramatic than in the United States. In the 10 years following passage of the Prescription Drug User Fee Act of 1992 (PDUFA) and its reauthorizing legislation, the Food and Drug Administration Modernization Act of 1997 (FDAMA), FDA has nearly doubled its staff, substantially improved the efficiency of the drug review process, and significantly cut approval times for new drugs. FDA has ac-

complished this while maintaining the highest standards for quality of review. Rarely has a single piece of regulatory legislation had so profound—and positive—an impact on the drug development environment in any country. In fact, these reforms in the United States are responsible in part for stimulating many of the regulatory reform efforts elsewhere around the world.

Whereas PDUFA focused primarily on FDA's review process, FDAMA contained provisions that specifically focused on how FDA can assist industry in shortening lengthy drug development times. These provisions included restrictions on the amount of information the FDA can require from a sponsor to initiate clinical research, the establishment of procedures for resolving scientific disputes between the agency and a drug firm, and FDA authorization to approve a New Drug Application on the basis of one adequate and well-controlled clinical study with confirmatory evidence in certain circumstances. FDAMA also led to new procedures for holding formal meetings between the agency and sponsors, and established the “Fast Track Process” for speeding the development and approval of drugs that address unmet medical needs. Analyses of these changes are explored in a series of articles, edited by Tom Copmann, in this issue of the *Drug Information Journal*, starting on page 247.

The industrial and societal gains achieved through faster approval times and earlier access to important new medicines are signifi-

Reprint address: Kenneth I. Kaitin, PhD, Director, Tufts Center for the Study of Drug Development, Tufts University, 192 South St., Suite 550, Boston, MA 02111.
E-mail: kenneth.kaitin@tufts.edu.

cant. But there is no guarantee that these trends will continue. With the current user fee legislation set to expire on September 30, 2002, the United States Congress will begin hearings in the coming months to reauthorize the user fee act. Congress, however, is keenly sensitive to public sentiment. And in recent years public sentiment has reflected a growing concern over several critical policy issues: the safety of the drug supply, the cost and affordability of new medicines, and the conduct of clinical trials. These concerns will play an important part in the upcoming hearings.

People who work in and with the research-based pharmaceutical and biotechnology industries know that new drug development is a complex, time consuming, and expensive process, with many false starts. They know that the search for ever more complex drugs to treat a growing array of diseases means

lengthier and more expensive research and development cycles, just as pressure is building to contain costs and make expensive drug therapies available to more and more patients. Drug developers facing these realities—in the United States and abroad—can either push onward or exit the business.

The challenge before the research-based drug industry is just as much a challenge for the public at large. As the demand for speedy access to promising new therapies grows, it is in the public's interest to support regulatory efforts to encourage and speed the development of effective and safe new pharmaceutical and biopharmaceutical products. Indeed, this has been the case in the United States. The promise of new medicines will expand the cornucopia of health benefits that drug therapies have spawned in the last century and which continue to improve the quality of life.