

IN EVERY ISSUE

Encouraging Innovations, Preserving Safety

BY ELLEN V. SIGAL, PHD

Maintaining a balance between access to new drugs and protecting consumers

As Capitol Hill prepared for a leadership change following the 2006 elections, health policy advisers were keenly focused on how the elections would affect the anticipated debate surrounding drug safety. Within the Washington Beltway, the Prescription Drug User Fee Act (PDUFA) reauthorization was widely presumed one of the most important pieces of legislation to receive consideration by the 110th United States Congress.

PDUFA was created in 1992 amid pressure from the HIV/AIDS community about the Food and Drug Administration's prolonged drug review time. While PDUFA should not replace congressionally appropriated dollars, the added resources have greatly improved overall efficiency of product review. But the recent identification of potential risks with products like Vioxx (rofecoxib) and Avandia (rosiglitazone) prompted widespread media scrutiny, reduced public confidence, and the expectation that sweeping drug safety provisions would be included in the reauthorization of PDUFA.

Earlier this year, I was among a group of expert scientists, clinicians, and advocates that issued a report, "Drug Safety & Drug Efficacy: Two Sides of the Same Coin," on how to best achieve a responsible balance between safety and access. In a safety-focused environment, it's important to understand what's at stake.

Scientists and medical professionals acknowledge and accept that drugs are never 100 percent safe and it's impossible to know everything about a drug at the time of approval. Some drug side effects may be difficult to distinguish from the symptoms of the disease itself and may be sufficiently rare as to go undetected during the clinical trial process. When health care providers and their patients look for the best course of treatment, the benefit-to-risk assessment may vary. Cancer patients undoubtedly desire and deserve the safest products possible, but within the continuum of cancer progression— from prevention to late-stage

illness—patients are often willing to accept higher levels of risk. Cancer is not just one disease, but rather hundreds, all with different characteristics and affecting different types of people, most of whom hold hope in an innovative pipeline to develop new products to help their condition.

Because of the amount of time it currently takes to develop new medicine, the community became concerned that an overemphasis on safety could result in more pre-approval requirements for new drugs that would be unlikely to improve a product's overall safety profile and also delay patient access, and eventually reduce the incentive for researchers to develop new therapies for patients most in need of innovation.

Unfortunately, there's no quick fix or guarantee for the safety of prescription drugs, but the appropriate balance of safety and innovation must be guided by science. Advanced science will yield improved methods for evaluating a new product's safety and efficacy while better identifying populations at risk for adverse effects.

Through significant efforts within Congress, the reauthorization of PDUFA takes important steps to further incorporate science and technology into the regulatory arena. Among many updates, this legislation increases the FDA's ability to interact with private health care databases to identify risks that are virtually undetectable in a relatively small pre-approval environment, without slowing patient access to beneficial new products.

The FDA plays a vital role in the continuum of research as the nexus between scientific discovery and clinical application. Safety is paramount, but increased resources for programs like the Critical Path Initiative at the FDA will help bring innovative discoveries to patients. The public, patients, and policymakers must recognize the need for ongoing FDA support.

—Ellen V. Sigal, PhD, is the chairperson and founder of Friends of Cancer Research, a nonprofit organization dedicated to raising awareness and providing public education on cancer research. The full drug safety report, “Drug Safety & Drug Efficacy: Two Sides of the Same Coin,” can be found at www.focr.org