Making Medical Products Better, Faster, and Cheaper

What FDA's "Critical Path" Initiative Means to Consumers
Q and A with Janet Woodcock, M.D.

Janet Woodcock, M.D., is FDA's Deputy Commissioner for Scientific and Medical Programs and Chief Medical Officer. Dr. Woodcock received her medical degree from Northwestern Medical School, and completed further training and held teaching appointments at the Pennsylvania State University and the University of California in San Francisco. She joined FDA in 1986.

PSC/Catherine Brown

Q. How do scientific discoveries affect the development of medical products?

A. In recent years, there has been an explosion of scientific discoveries made possible through technologies such as genomics, advanced imaging, nanotechnology, and robotics. These scientific advances can help produce more and better medical products—not just drugs, but biologics such as vaccines, and devices such as pacemakers.

But the efficiency for scientific discoveries being translated into medical products is very low—in fact, it's worse than it was 10 years ago. For example, new drugs go through three phases of progressively rigorous testing, or clinical trials, to show their safety and effectiveness before FDA will consider allowing them on the market. Today, new compounds that make it through Phases 1 and 2 of clinical trials fail 50% of the time in Phase 3 compared to a 20% failure rate 10 years ago.

Q. Why is there a slow-down in the availability of medical products?

A. In FDA's view, new science is not being used to guide the medical product development process in the same way that it is accelerating the discovery process. The path that a medical product takes from development to mass-production and availability to the public—what we call the Critical Path—has become increasingly challenging, inefficient, and costly.

Q. What is the Critical Path Initiative?

A. The Critical Path Initiative, launched in 2004, is FDA's effort to stimulate and assist a national effort to modernize the scientific process—the Critical Path—through which FDA-regulated products are developed, evaluated, and manufactured. We need to improve this Critical Path so we can move medical discoveries from the laboratory to consumers more efficiently.

Q. How can medical products be developed more efficiently?

A. Many of the scientific tools used today to predict and evaluate safety and effectiveness, as well as to manufacture products, are decades old. They are time-consuming, cumbersome, and imprecise. They may fail to predict specific safety problems that ultimately halt development.

We need better tools to predict and detect safety problems early in the Critical Path so that products likely to fail are weeded out and developers can focus on products with a high probability of safety and effectiveness. We also need tools to guide the

Critical Path efforts will modernize the development process so that new medical products can get to Americans more efficiently and at lower cost.

sponsor of a drug in choosing the appropriate dose and regimen or, in the case of a medical device, the right size and placement. And manufacturers need tools to better mass-produce an approved medical product, such as a vaccine, and evaluate the quality of the finished product.

So we need to build a better tool kit. And the tools must be made publicly available for use by all researchers and product developers.

Q. What will the tool kit contain? Can you give an example?

A. One important tool is better biomarkers—indicators that help measure the progress of a disease or the effect of a treatment.

Some genetic mutations can serve as biomarkers. An example concerns warfarin, the generic version of Coumadin, a blood-thinner used by roughly 2 million Americans each year. Warfarin is the second most common drug, after insulin, implicated in visits to emergency rooms because of bad side effects. Too much warfarin can lead to life-threatening bleeding, and too little can result in dangerous blood clots.

Doctors use a trial-and-error process to find the right dose of warfarin for a particular patient. One-third of the patients who take warfarin metabolize it differently than expected. Research has shown that some of this unexpected response depends on a person's variants of two specific genes. FDA recently approved a label update for warfarin to give health care providers information on using genetic tests to improve warfarin dosing for an individual patient.

Although these genetic tests can help, they're still not as precise as we'd like. So FDA, in collaboration with the Critical Path Institute based in Arizona and the University of Utah, is looking further at how genetic differences in people affect the way they respond to warfarin. The goal is to develop a mathematical tool, an algorithm, for doctors to use to help personalize warfarin dosing so that an optimum dose, and a safe dose, can be given to each person based on his or her genetic makeup and other individual factors.

Q. How else will Critical Path efforts help consumers?

A. The payoff for consumers is enormous. Critical Path efforts will modernize the development process so that new medical products can get to Americans more efficiently and at lower cost.

Modern evaluation tools will allow us to learn more about products before they are approved. This will give doctors and patients the best available information about how to use a product to maximize its benefit and minimize side effects. In fact, many of the tools being considered would help personalize medicine by identifying who is likely to respond well to a treatment and who should avoid it.

Critical Path efforts will also help lower the costs of medical products to consumers. Just a 10% improvement in predicting a drug's failure before clinical trials were started could save $100 million in development costs for that single drug—costs that otherwise may be passed on to consumers through higher insurance premiums or more expensive drugs.

Q. Who is involved in the Critical Path efforts?

A. FDA has called for a joint effort of industry, academic researchers, other government agencies, professional societies, trade associations, patient advocacy groups, and others to help in the Critical Path Initiative. We have collaborations established with many of these organizations and are inviting others to help in our efforts.

Q. Since FDA doesn't develop drugs, why is it involved in the Critical Path?

A. FDA is responsible for ensuring that safe and effective medical innovations are available to the public. As part of its regulatory role, the agency sets the standards that products must pass to show safety and effectiveness.

During clinical trials, FDA scientists conduct ongoing reviews of data on safety, effectiveness, and product quality. These scientists see the complete spectrum of successes and best practices, as well as the failures, barriers, and missed opportunities that occur during product development. This experience puts FDA in a unique position to set performance standards and guidance that can aid product development.

We must also make sure that developers have the best tools to show that their products meet the standards so that new treatments can be made available to the public as quickly as possible. FDA

For More Information

www.fda.gov/oc/initiatives/criticalpath