

Critical Path Initiative Fact Sheet

The Critical Path Initiative (CPI) is the FDA's premier initiative to identify and prioritize the most pressing medical product development problems and the greatest opportunities for rapid improvement in public health benefits. Its primary purpose is to ensure that basic scientific discoveries translate more rapidly into new and better medical treatments by creating new tools to find answers about how the safety and effectiveness of new medical products can be demonstrated in faster timeframes, with more certainty, at lower costs and with better information.

THE PROBLEM

In recent years, rapid advances in the biomedical sciences have raised expectations of similar progress in the development of products for the prevention and treatment of serious illnesses. Despite huge strides to decipher the intricacies of human biology, medicine today remains, to an unfortunate degree, an attempt to balance the risks of treatments against their uncertain potential to cure. Physicians earnestly attempting to provide the best treatments, along with their patients -- who may be suffering from any of a host of debilitating, even fatal, diseases -- are too frequently left waiting for treatments that are expensive and, ultimately, may not work for them. Worse, the number of new drugs and other treatments approved each year for use in the United States is steadily dropping, in no small part because scientists test new discoveries using outdated and inefficient tools and techniques. The result is a slow, expensive process. It produces fewer and fewer treatments that can be approved as safe and effective, and it leaves consumers to grapple with the question marks of treatment and a short list of prevention options.

- According to a JAMA article published in September 2005, entitled "Financial Anatomy of Biomedical Research," biomedical research funding has increased from \$37.1 billion in 1994 to \$94.3 billion in 2003 and has doubled when adjusted for inflation.
- Currently, nine out of ten experimental drugs fail in clinical studies because we cannot accurately predict how they will behave in people based on laboratory and animal studies.
- According to the Tufts Center for the Study of Drug Development, as of the year 2000 the cost of developing a new prescription drug had hit \$802 million -- up from \$231 million in 1987, and from \$54 million in 1976. In inflation adjusted dollars, total cost increased at an annual rate of 7.4% above general price inflation.

THE SOLUTION

In its 2004 Critical Path Report, the FDA presented its diagnosis of one of the scientific challenges underlying the medical product *pipeline problem*. The report then laid out a path forward, beginning with extensive outreach and consultation with public and private stakeholders. Stakeholders confirmed our diagnosis and provided examples of scientific investments that could revolutionize medical product development.

The Critical Path Initiative will harness science's newfound knowledge of how and why diseases afflict each of us, to create a new pathway for rapidly developing and approving safer treatments tailored to individuals, at lower cost, and with a greater degree of success. Experts estimate, the new tests and tools developed under the Critical Path Initiative will modernize the drug development process by 2010 and help to get new medical discoveries to Americans faster and at a lower cost.

OBJECTIVE

Under the Critical Path Initiative, the goal is to stimulate the development of powerful new scientific and technical tools -- such as proven biomarkers, innovative clinical trial designs, simulation models of physiology and disease processes, and manufacturing quality assessment

methods -- capable of rapidly predicting the safety, effectiveness, and quality of new medical products. The FDA is uniquely qualified to provide leadership for this seminal initiative. In addition to the agency's 100-year experience in dealing with medical products, the FDA's scientific reviewers have the best overall understanding of the most successful practices as well as the failures, roadblocks, bottlenecks, and missed opportunities along the Critical Path.

STATUS

In March 2006, the agency the FDA released The Critical Path Opportunities List based on feedback from stakeholders and the special insights of FDA's product reviewers. The list outlines an initial 76 "science projects" to bridge the gap between the quick pace of new biomedical discoveries and the slow pace at which those discoveries are currently translated into therapies for patients. The release of the list marks a starting point in identifying the essential development priorities to be accomplished under the agency's Critical Path Initiative. The initial list is comprised of highly-targeted research projects divided into six key areas:

- Better Evaluation Tools -- Biomarkers and Disease Models
- Streamlining Clinical Trials
- Harnessing Bioinformatics
- Moving Manufacturing into the 21 st Century
- Products to Address Urgent Public Health Needs
- At-Risk Populations

Accomplishment of research priorities over the next four years will require unprecedented collaboration among public and private sector partners. In order to facilitate completion of these projects in a timely manner, the FDA will act a convenor in bringing together partnerships and consortia to accomplish a majority of the projects. FDA's unique regulatory experience and first-hand knowledge of the hurdles in scientific research will enable the agency to play a consultation role in many of the projects. In addition, the initiative will require a new, cooperative partnership among the primary research, evaluation, approval and medical treatment delivery and reimbursement divisions of HHS, including the FDA, NIH, CMS, and AHRQ.

OUTLOOK

In April 2006, the FDA will look for ways to support selected Critical Path priorities on the Opportunities List. FDA then plans to announce which Critical Path projects it has decided to undertake. For more information, see <http://www.fda.gov/oc/initiatives/criticalpath/>