Food and Drug Administration
Silver Spring MD 20993

Speeding Progress to Patients

Recent U.S. investments in biomedical research have dramatically expanded our understanding of biology and disease. Yet the development of new therapies has been in decline, and the costs of bringing them to market have soared. Results of this unfortunate trend include lost opportunities to improve the effectiveness of U.S. medicine and growing threats to the economic health of the innovative U.S. biotechnology industry. New approaches and partnerships in the emerging field of Regulatory Science are urgently needed to bridge the gap between drug discovery and patient care.

A key problem is that the ways in which new therapies are developed and tested have failed to keep pace with advances in biomedical research, leaving patients without the benefits of the advances. As the FDA's independent Science Board recently found, "American lives are at risk" because:

> *While the world of drug discovery and development has undergone revolutionary change — shifting from cellular to molecular and gene-based approaches — FDA's evaluation methods have remained largely unchanged over the last half century.*

Think of a rower on the Potomac with one powerful arm and one scrawny arm. Rowing with all his might, he can only go in circles. So it goes with U.S. biomedical advances over the past several decades. Basic science, spearheaded by NIH, is pulling hard. But it also takes muscle to create innovative tools, standards, and approaches for the efficient assessment of medical product safety, efficacy, and quality.

Without these complementary advances in Regulatory Science, promising therapies may be discarded during the development process simply for the lack of tools to recognize their potential, and outmoded review methods can unnecessarily delay approval of critical treatments. Conversely, many dollars and years may be wasted assessing a novel therapy that with better tools might be shown to be unsafe or ineffective at an earlier stage.

Strengthening Regulatory Science is a vital and urgent challenge for the brightest minds in academia, industry, and government. Advances in this field will help modernize product development to provide better tools, standards, assays, disease models, and science-based pathways to improve the speed, efficiency, predictability, capacity, and quality of product development, evaluation, and manufacturing. For example:

- **Stem Cells for Parkinson's Disease.** Promising research is underway using stem cells to restore brain function lost in patients with Parkinson's disease. But before these treatments can reach patients, we must develop scientifically valid standards for stem cell therapies so that they can be produced reliably and safely.

- **An Artificial Pancreas for Juvenile Diabetes**. NIH, industry, and foundations are working to support the development of novel devices that continuously monitor a patient's blood sugar and automatically inject the right amount of insulin. Such devices have the potential to reduce the

devastating complications of the disease and improve patients' quality of life. But for patients to benefit, we must develop a scientifically solid path for testing to make sure the devices control blood sugar levels without placing patients at risk for life-threatening hypoglycemia.

- **Personalized Treatment for Cancer.** Basic research studies are identifying potential markers that can indicate whether a patient's cancer will respond to a specific therapy or combinations of therapies. But for these markers to be applied in clinical practice, we must use cutting edge science to guide the evaluation and use of new diagnostic tests so that mistakes are not made and every patient is able to get the best possible treatment for their disease, ushering in an era of "personalized medicine."

- **Safer pain medications.** There is a global epidemic of prescription pain medicine abuse and misuse. At the same time, patients in agonizing pain are still often undertreated. New pain pathways have been discovered and new medicines are being developed that can help. But to bring progress to patients, we need to find better pain models, measurement tools (including patient reported), and clinical trial designs to enable development of effective pain medications with less potential for abuse.

- **Vaccines and New Drugs for Tuberculosis**. NIH and others are supporting exciting research into treatments to stop drug resistant TB and to develop TB vaccines. Both are needed to protect millions of people everywhere, including US citizens, travelers, and troops. We must develop approaches that can more quickly evaluate TB treatments and vaccines, including identifying markers that can predict cure and protection.

With so much at stake for public health, FDA Commissioner Dr. Margaret A. Hamburg has made advances in Regulatory Science a top priority. The agency is both supporting mission critical science at FDA and exploring a range of new partnerships with NIH and academic institutions to develop the science needed to maximize the returns from our investments in biomedical research and bring the development and assessment of promising new therapies into the 21st century. With this effort, FDA is poised to support a wave of innovation to transform medicine and save lives.