

From the NIH Director



Photo: NIH

The Importance of Clinical Trials

Francis S. Collins, M.D., Ph.D., Director of the National Institutes of Health, led the successful effort to complete the Human Genome Project, a complex multidisciplinary scientific enterprise to map and sequence human DNA. He spoke in early June to attendees of a 2011 conference, “Clinical Trials: New Challenges and Opportunities,” cosponsored by the National Library of Medicine, the Friends of the National Library of Medicine, and the American Association for the Advancement of Science.

Since its launch in 2000, Clinicaltrials.gov has grown in a breathtaking fashion. This free online database, created in response to a legislative mandate to help the public learn more about clinical trials, today contains descriptions, locations, and other vital information about more than 109,000 clinical trials.

Despite this great progress, many difficulties remain — difficulties that can delay or even thwart efforts to move scientific discoveries from the lab to the medical clinic. One of the biggest challenges is that very few Americans with common diseases are currently enrolled in clinical trials. For example, clinical trial participation stands at just 3 percent among U.S. adults with cancer.

If clinical trials are to be successful, it is critical that

more people get involved. We need to spread the word about the value of participating in clinical trials. Signing up for a clinical trial may indeed benefit medical research and help future generations. But it is not strictly an altruistic endeavor. In many instances, trial participants do gain personal advantages, such as improved disease outcomes or better health. And we should not be shy about telling that story.

We also need to make it easier and more convenient for people to take part in clinical trials. One way in which we might do this is by making the process of research oversight less bureaucratic. Perhaps we need to rethink all of those 22-page consent forms that nobody reads anyway!

Furthermore, the National Institutes of Health (NIH)

needs to take a hard look at the ways in which we support clinical trials. Are we making wise choices? Are we covering the bases that most need attention in the most effective way? And, when we fund a clinical trial, are we making sure that it has sufficient power—that it will enroll enough participants—to produce a meaningful result? Small trials with uncertain

plan to launch this fall, will be to catalyze the generation of innovative methods and technologies that will enhance the development, testing, and implementation of diagnostics and therapeutics across a wide range of diseases and conditions. Such activities will complement, and not compete with, translational research being carried out at NIH and elsewhere

in the public and private sectors.

In the realm of clinical trials, NCATS will offer researchers a chance to develop and test more flexible, or adaptive, trial designs. Also, because we are learning that the best treatments for many diseases will likely consist of multiple drugs or other therapeutics, NCATS may support efforts to develop innovative trials focused on combination therapies.

Given the economic challenges facing our nation today, I want to emphasize that NCATS represents an efficient use of taxpayer dollars. It will pull together existing resources that are currently scattered across NIH and integrate them into one cohesive unit. Furthermore, NCATS will work together in partnership with academia, industry, regulators, nonprofits, and patient advocates to achieve its aim of delivering solutions to the millions of people awaiting new and better ways to detect, treat, and prevent disease.

In fact, I think the United States is very wise to invest in clinical trials, NCATS, and the many, many other types of biomedical research. Not only do such investments save lives and improve health, they can have a powerful effect on our economy. Take the case of the Human Genome Project, the publicly funded effort to read all 3 billion letters in the human DNA instruction book. A recent analysis concluded that the roughly \$4 billion spent on this project generated \$796 billion in economic growth within the first decade. Not a bad return on investment!

To Find Out More

- To search a free database of clinical trials being conducted across the United States and around the world, go to clinicaltrials.gov/.
- MedlinePlus: Clinical Trials general information and Web links.
www.nlm.nih.gov/medlineplus/clinicaltrials.html

Photo: National Cancer Institute



“We need to spread the word about the value of participating in clinical trials,” says Dr. Collins. “Signing up for a clinical trial may benefit medical research and help future generations. But it is not strictly an altruistic endeavor. In many instances, trial participants do gain personal advantages, such as improved disease outcomes or better health.”

endpoints may cost less than larger, well-designed trials, but may not teach us what we need to know.

Now is an opportune time to be asking these and other questions that lie at the heart of translational science—the field of research that seeks to use advances in biomedical knowledge to develop new and better strategies for detecting, treating, and preventing disease. In fact, we at NIH have taken bold steps aimed at revamping our thinking about this important field and underscoring its relevance.

Why now? Over the past few years, there has been a deluge of discoveries generated by basic scientists about the genetic and environmental causes of disease, findings that likely contain a wealth of new targets for combating disease. At the same time, the rate at which new drugs and other therapeutics are reaching patients has not improved. If anything, the pace of therapeutic development appears to have slowed, despite the many new opportunities uncovered by basic science.

In response to this dilemma, the Scientific Management Review Board recently recommended that NIH form a new entity, the National Center for Advancing Translational Sciences (NCATS). The mission of this new Center, which we