

DEPARTMENT OF HEALTH AND HUMAN SERVICES

NATIONAL INSTITUTES OF HEALTH

The Future of Biomedical Research

Witness appearing before the

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Good morning, Mr. Chairman and distinguished Members of the Subcommittee. I am Francis S. Collins, M.D., Ph.D., Director of the National Institutes of Health (NIH). It is an honor to appear before you today to provide an overview of NIH's critical role in enhancing our nation's health through scientific discovery.

As the nation's biomedical research agency, NIH's mission is to seek fundamental knowledge about the nature and behavior of living systems and to apply that knowledge to enhance human health, lengthen life, and reduce illness and disability. I can report to you that NIH leadership, employees, and grantees continue to believe passionately in this mission.

Before I discuss the tremendous strides we have made and the exciting scientific opportunities on the horizon, I want to thank you, Mr. Chairman, and Ranking Member DeLauro, as well as your colleagues, for the recent Fiscal Year (FY) 2014 Omnibus Appropriation bill. The Subcommittee came together in a bipartisan way to increase funding for NIH and we are truly grateful for your action. The past year has been challenging for us: the sequester reduced funding for

more than 30 years ago. NIH research also has given us vaccines to protect against an array of life-threatening diseases, including cervical cancer, influenza, and meningitis. We can look forward to a future in which advanced prevention and treatment strategies such as these allow everyone to have a

disseminate new analytical methods and software, enhance training of data scientists, and facilitate broad use and sharing of complex biomedical datasets. With sustained investment and effort, we will overcome the challenges associated with Big Data to accelerate real-world applications of basic science discoveries.

We are also excited about another area of intense interest: the development of therapeutics. Recent advances in genomics, proteomics, imaging, and other technologies have led to the recent discovery of more than a thousand risk factors for disease—biological insights that ought to hold promise as targets for drugs. But drug development is a terribly difficult and failure-prone business. To the dismay of researchers, drug companies, and patients, the vast majority of drugs entering the development pipeline fall by the wayside. The most distressing failures occur when a drug is found to be ineffective in the later stages of development—in Phase II or Phase III clinical studies—after years of work and millions of dollars have already been spent. A major reason for such failures is that scientists en

disorders, lupus and rheumatoid arthritis. Through this team effort, we believe we can reach our shared goals of treating and curing disease faster.

Preventing disease is another top priority, and influenza is one area of prevention in which we are poised for rapid progress. Currently, to provide protection against the rapidly evolving influenza virus, a new vaccine must be produced each year and we all need to get an annual flu shot. Also, despite best efforts, the vaccine isn't always ideal. In an average year, the flu claims up to 49,000 American lives and costs the U.S. economy about \$87 billion. But it does not have to be that way. NIH-funded researchers are now working on a universal flu vaccine—designed to protect people against virtually all strains of the flu for extended periods of time and, thus, potentially reduce the need for annual flu shots. Of critical importance, such a vaccine could also protect against a future global flu pandemic.

While we are several years away from having a universal flu vaccine available to the public, our researchers have already demonstrated proof of concept and are testing a number of approaches, including two-stage “prime boost” vaccines and ferritin nanoparticles. Clearly, the prospect of a universal flu vaccine is not science fiction. Early clinical studies are already underway. With sustained investment, the United States may be a few years away from realizing its potential to benefit our health and our economy.

As impressive as a universal flu vaccine would be, it is not the only trick we are teaching our immune systems. We are also aiming to harness the body's own immune system to fight cancer. Until recently, our weapons for attacking cancer have been largely limited to surgery, radiation, and chemotherapy—treatments that carry risks and cause adverse side effects. Now, after years of intense basic and translational research, we have an exciting new possibility: cancer immunotherapy.

Researchers have long been puzzled by the uncanny ability of cancer cells to evade the immune response. What stops the body from waging its own “war on cancer?” As it turns out, our bodies have built-in checkpoints to prevent our immune systems from going into overdrive and killing healthy cells. Now, NIH-funded researchers have discovered a way to genetically modify certain white blood cells called T-cells—the soldiers of the immune system—to attack tumor cells. In this new approach, T-cells are collected from cancer patients and engineered in the lab to produce special proteins on their surface, called chimeric antigen receptors (CARs). When the modified cells are infused back into patients, they multiply and, with guidance from their newly engineered receptors, seek and destroy tumor cells. Promising results in patients with leukemia prompted *Science* magazine to name this its 2013 Breakthrough of the Year.

Today, I have provided a very brief overview of NIH’s past successes and continuing commitment to basic, translational, and clinical research. Our nation has never witnessed a time of greater promise for advances in medicine. With your support, we can anticipate a future of accelerating discovery across NIH’s broad research landscape, from fundamental scientific inquiry to human clinical trials. The “National Institutes of Hope” is ready to move forward.

This concludes my testimony, Mr. Chairman. I look forward to your questions.