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NATIONAL INSTITUTES OF HEALTH

The NIH: Investing in a Healthier Future

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Good morning, Chairman Blunt, Ranking Member Murray, and distinguished Members of the Subcommittee. I am Francis S. Collins, M.D., Ph.D., and I am the Director of the National Institutes of Health (NIH). It is an honor to appear before you today to discuss how NIH is investing in a healthier future for all Americans.

NIH has been advancing our understanding of health and disease for more than a century. Scientific and technological breakthroughs generated by NIH-supported research are behind many of the improvements our country has enjoyed in public health. For example, our Nation has gained about one year of longevity every six years since 1990.¹ A child born today can look forward to an average lifespan of about 78 years – nearly three decades longer than a baby born in 1900. Deaths from heart attack and stroke have been reduced by more than 70 percent in the past 60 years. Thanks to NIH-developed anti-viral therapies, HIV-infected people in their 20s today can expect to live into their 70s. This compares to a life expectancy measured in months when the disease first appeared in the 1980s. Cancer death rates have been dropping about one percent annually for the past 15 years. These are extraordinary strides – but we aim to go much further.

On behalf of NIH, our employees, grantees, and patient community, I want to thank the Members of this Subcommittee for your continued support, and for holding this hearing today.

This investment could not come at a better time. We are in the midst of a remarkable stream of scientific advances spurred by dramatic advances in biotechnology. Today, I want to share with you a few of the many promising opportunities before us that will lead to a healthier future for all. I can assure you that the future of scientific research has never been brighter.

¹http://www.cdc.gov/nchs/data/nvsr/nvsr64/nvsr64_02.pdf.

Many recent breakthroughs stem from our nation's commitment to investing in basic science research. Basic science lays the foundation for advances in disease diagnosis, treatment, and prevention by providing the building blocks for clinical applications. Basic science is generally not supported in the private sector, and NIH's focus on understanding fundamental biological processes fosters innovation and ultimately leads to effective ways to treat complex medical conditions. But the lead time for medical breakthroughs to arise from basic science research is often measured in decades, and it is generally not possible to predict which basic investigations are going to be the most fruitful in the long run. NIH's successful investment in basic science is reflected by the awarding of 145 Nobel prizes to NIH-supported scientists; the vast majority of these individuals were recognized for basic science advances.

A compelling example of how we are trying to unravel life's mysteries through basic science is with the Brain Research through Advancing Innovative Neurotechnologies (BRAIN) Initiative. With nearly 100 billion neurons and 100 trillion connections, the human brain remains one of the most daunting frontiers of science and one of the greatest challenges in medicine. This bold, multi-agency effort to revolutionize our understanding of the human brain will require the development of entirely new technologies. Engineers, computer scientists, nanotechnologists, physicians, and neuroscientists will need to work together and challenge the limits of their respective fields of science. By measuring real-time activity at the scale of complex neural networks in living organisms, we can explore how the brain enables the human body to record, process, utilize, store, and retrieve vast quantities of information -- all at the speed of thought. Ultimately, the foundation of understanding developed by the BRAIN Initiative will help reveal the underlying pathology in a vast array of brain disorders and provide new therapeutic avenues

to treat, cure, and prevent neurological and psychiatric conditions such as Alzheimer's disease, autism, schizophrenia, depression, epilepsy, and addiction.

Five years ago, a project like this would have been considered impossible. But with your support, it is now underway. The first two rounds of grant awards have been made – and they are tremendously exciting. In the year since the inaugural round of awards, totaling \$46 million, was issued, several exciting new tools and techniques have been developed for studying brain structure and function. One such technique, called Drop-Seq, groups neurons based on the genes that they express, getting us closer to having a complete parts list for the brain. Another tool, called DREADD (Designer Receptors Exclusively Activated by Designer Drugs), used designer drugs to turn on and off genetically engineered neural receptors. While its inventors used DREADD to precisely control a mouse's motor movements, the tool may potentially provide a way to restore proper neural function. Among the second round of awards, totaling \$85 million, announced last week are projects aimed at delivering targeted electrical pulses to the brain to treat illnesses such as traumatic brain injury and epilepsy, as well as collaborations with physicists towards building non-invasive tools that can observe neural activity deep within the brain with unprecedented spatial detail.

We need to continue to ramp up this effort, and we need your support for that, as requested in the President's Budget. While the goals of this initiative are ambitious, the time is right to inspire a new generation of neuroscientists to undertake this groundbreaking approach to understanding the human brain.

Another area of exceptional scientific opportunity I want to highlight today involves one of our nation's most feared killers: cancer. Until recently, our weapons for attacking cancer have been largely limited to surgery, radiation, and chemotherapy—all of which can be effective, but

carry risks and toxicities. Now, after years of intense basic and translational research, we have two exciting new possibilities: targeted therapeutics and cancer immunotherapy. I want to particularly focus on the latter.

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 important built-in checkpoints to prevent our immune systems from running amok and killing healthy cells. Certain white blood cells called T-cells—the armed soldiers of the immune system—are designed to go after foreign invaders, but they also need a stop signal to prevent going into overdrive. One way to do this is through a receptor on the T-cell called CTLA-4 that inhibits its function. Tumor cells have figured out how to take advantage of this pathway by upregulating CTLA-4; the result is to put the brakes on the immune system, giving the green light for the cancer to grow.

NIH-funded researchers have discovered a way to release the brakes by introducing a monoclonal antibody against CTLA-4, allowing the normal immune response to be re-activated. Dr. James Allison, who led the basic science efforts that led to these insights, was just honored with the receipt of the Lasker Award, the “American Nobel Prize.” Promising results in patients with metastatic melanoma and lung cancer are making this and other immunotherapies the breakthrough treatment of the future. After President Carter was diagnosed with stage 4 metastatic melanoma, he received immunotherapy as part of his treatment.

A final area I wish to highlight is precision medicine. As you know, in his State of the Union address in January of this year, President Obama announced his intention to launch the Precision Medicine Initiative (PMI). This is a bold new research effort to revolutionize the prevention and treatment of disease, and I thank the Committee for including the requested \$200

million for PMI in its FY16 appropriations bill. We believe that the time is right for this audacious undertaking, and, with your support, the NIH and our HHS partners, the U.S. Food and Drug Administration (FDA), the Office of Civil Rights, and the Office of the National Coordinator for Health Information Technology (ONC), will work with great intensity to achieve this vision.

Historically, physicians have had to make most recommendations about disease prevention and treatment based on the expected response of the average patient. This one-size-fits-all approach works for some patients and some conditions, but not others. Precision medicine is an innovative approach that takes into account individual differences in patients' genes, environments, and lifestyles. This concept is not new; blood typing, for example, has been used to guide blood transfusions for a century. Prescription eyeglasses are tailored specifically to the patient's individual needs. Moreover, the identification of the *BRCA1* and *BRCA2* genes has made it possible to provide options for women at high risk for breast and ovarian cancers. And, the gene implicated in cystic fibrosis has led to widespread availability of screening and targeted therapeutics.

The prospect of applying this concept broadly to virtually all diseases, and to disease prevention, has been dramatically improved by the development of powerful and affordable methods for characterizing personal biological attributes (such as genomics and metabolomics), the widespread adoption of electronic health records, the recent revolution in mobile health technologies, and the emergence of computational tools for analyzing large biomedical data sets. These advances will help make possible the dream of personalizing a wide range of health applications.

With this in mind, we are excited to take a lead in the two key components of the President's Precision Medicine Initiative that will be managed by NIH. First is a near-term goal that will focus on cancer, building on advances in genomics and immunology that make it increasingly possible for specific therapies to be designed for the individual, based on the precise molecular characteristics of their tumor. Second is a longer-term aim to generate knowledge applicable to the whole range of health and disease. Both components are within reach, due in large part to recent scientific breakthroughs. Let me tell you just a little bit more about the longer term project.

In order to achieve the President's ambitious plan, NIH will build a large national research cohort of one million or more Americans that will provide the platform for expanding our knowledge of precision medicine approaches and benefit the nation for years to come. These volunteer participants will agree to share health information, provide biospecimens, and wear sensors that will detect environmental exposures and body performance – all with appropriate privacy protection. They will be true partners in this research. Not subjects, not patients—partners. They will play an active role in how their genetic, environmental, and medical information is used for the prevention of illness and management of a wide array of chronic diseases. The goal will be to expand the benefits of precision medicine into myriad aspects of health and health care. Participants will be at the center of the project design, and they will have access to their own health data, as well as research using their data, to help inform their own health decisions. Through this dynamic community, researchers will be able to advance the information derived from this cohort into new knowledge, approaches, and treatments.

Researchers from many organizations will, with proper protection of patient information, have

access to the cohort's data so that the world's brightest scientific and clinical minds can contribute insights.

In order to help inform the vision for building the national research cohort of one million or more volunteers, I formed a Precision Medicine Initiative Working Group, as part of my Advisory Committee, to develop a specific design plan for creating and managing such a research cohort. To help carry out its charge, the Working Group engaged with stakeholders and members of the public through workshops and requests for information, focusing on issues related to the design and oversight of the cohort. Public engagement, as well as internal discussions, led to the vision for the design and utility of the program, and the Working Group released their report just three weeks ago. The report includes recommendations in six areas critical to the development, implementation, and oversight: cohort assembly, participant engagement, data, biobanking, policy, and governance. We plan to move swiftly to build the infrastructure so that participants can begin enrolling in the cohort in 2016, with a goal of at least one million participants by 2020.

A project of this magnitude will lay the foundation for a myriad of new prevention strategies and novel therapeutics. Although the initiative will likely yield its greatest benefits years down the road, there will be successes in the relatively near future as well. Moving forward, this pioneering research initiative will require the involvement of many different sectors of science and society, including biologists, physicians, technology developers, data scientists, health care organizations, and, most importantly, the American people. Given related efforts in a few other countries, we will aim to forge collaborations on a global scale.

With sufficient resources and a strong, sustained commitment of time, energy, and ingenuity from the scientific, medical, and participant communities, precision medicine's full

potential can be realized to give everyone the best chance at good health. There's no better time than now to embark on this ambitious new enterprise to revolutionize medicine and generate the scientific evidence necessary to move this individualized approach into everyday clinical practice.

Today, I have outlined for you just a few of the very many promising scientific opportunities on the horizon. With your support, the future of medicine can be very bright. This concludes my testimony, and my colleagues and I look forward to answering your questions.