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NATIONAL INSTITUTES OF HEALTH

21st Century Cures

Witness before the

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Good morning, Chairman Pitts, Ranking Member Green, and distinguished Members of the Subcommittee. My name is Kathy Hudson and I am the Deputy Director for Science, Outreach, and Policy at the National Institutes of Health (NIH).

I want to thank the Members of this Subcommittee for your hard work over the past year on the 21st Century Cures Initiative and for holding this hearing today. It is an honor to appear before you, alongside my colleagues from the Food and Drug Administration (FDA), to discuss how we, as a nation, can accelerate the pace of medical breakthroughs in the United States and get cures to patients faster.

As the nation's premier 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. All of us at NIH believe passionately in this mission, and are dedicated to the pursuit of knowledge and, ultimately, cures.

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. NIH research is also making progress against specific public health threats. For example, cancer death rates have been dropping about 1 percent annually for the past 15 years; each 1 percent decline has been estimated to be worth $500 billion as a result of gains in life expectancy.[2] Meanwhile, HIV/AIDS treatment and prevention may now enable us to envision the first AIDS-free generation since the virus emerged more than 30 years ago. These are extraordinary strides—but we aim to go much further.

The President's Precision Medicine Initiative, a bold new research effort announced early this year to revolutionize how we improve health and treat disease. The proposed initiative included in the

[1] http://www.cdc.gov/nchs/data/nvsr/nvsr64/nvsr64_02.pdf.
[2] Murphy, K.M., & Topel, R.H. (2006). The value of health and longevity. Journal of Political Economy, 114(5), 871-904.

President's Fiscal Year 2016 Budget will pioneer a new model of participant-engaged research that promises to accelerate biomedical discoveries and provide clinicians with new tools, knowledge, and therapies to select which treatments will work best for which patients. Precision medicine takes into account individual differences in people's genes, environments, and lifestyles and gives clinicians tools to better understand the complex mechanisms underlying a patient's health, disease, or condition, to better predict which treatments will be most effective.

The President's Fiscal Year 2016 Budget includes $31.3 billion for NIH, an increase of $1 billion or 3.3 percent above the enacted FY 2015 level, to maintain the nation's leadership in the biomedical sciences. Other countries are expanding their support for medical research, and stable funding for NIH is an important element in America's leadership in medical research and innovation. Other crucial areas of focus and opportunity to work with this Committee include (1) facilitating scientific collaboration and innovation; (2) modernizing clinical research and data access; and (3) reducing administrative burden and increasing efficiency.

Facilitate Scientific Collaboration and Innovation

The NIH supports basic research that is fundamental to the discoveries that have long made our nation the world's leader in biomedical science. In addition, NIH funds translational research, which seeks to find ways to move basic findings toward the clinic, and clinical research, which involves the testing and evaluation of new strategies for disease management and prevention. It is crucial that scientists work together during all stages of research, and I would like to share a few examples of how NIH is encouraging collaboration and spurring innovation.

One way we are working to unravel life's mysteries is with the President's Brain Research through Advancing Innovative Neurotechnologies (BRAIN) Initiative announced in 2013. NIH is partnering with colleagues at the National Science Foundation, the Defense Advanced Research Projects Agency (DARPA), the Intelligence Advanced Research Projects Activity (IARPA), and the Food and

Drug Administration (FDA), in this effort to revolutionize our understanding of the most complicated biological structure in the known universe, the human brain. This multiyear initiative will produce a clearer, more dynamic picture of how individual cells and neural circuits interact in both time and space. By measuring activity at the scale of neural networks in living organisms, we can begin to decode sensory experience and, potentially, even memory, emotion, thought, and consciousness. Ultimately, the technologies developed within the BRAIN Initiative may help reveal the underlying pathology in a vast array of brain disorders and provide new therapeutic avenues to prevent, treat, and cure neurological and psychiatric conditions such as Alzheimer's disease, autism, schizophrenia, traumatic brain injury, and addiction.

Recent advances in genomics, proteomics, imaging, and other technologies have led to the 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 endeavor. To the dismay of researchers, drug companies, and patients, the vast majority of drugs entering the development pipeline never emerge as patient-ready therapies. 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 often don't know how to choose the right clinical pathway to target. If a drug is aimed at the wrong target, it won't work against the disease it was intended to treat.

With this in mind, we were thrilled to launch the Accelerating Medicines Partnership (AMP) last year. This unprecedented public-private partnership is using cutting-edge scientific approaches to identify and validate promising biological targets for therapeutics. Besides NIH, AMP partners include the FDA, ten biopharmaceutical firms, and a number of non-profits, including patient advocacy groups. This pre-competitive partnership is focusing initially on three areas of disease that are ripe for discovery: Alzheimer's disease, type 2 diabetes, and the autoimmune disorders, lupus and rheumatoid

arthritis. Costs are shared equally between NIH and the participating companies, and all data is openly shared. Through this truly innovative and collaborative approach, we believe we can learn how to treat and cure disease faster.

NIH is also working to streamline the therapeutic development pipeline through efforts at the National Center for Advancing Translational Sciences (NCATS). One example is the Tissue Chip for Drug Screening Initiative, a collaboration with the Defense Advanced Research Projects Agency (DARPA) and FDA, with a goal of improving the process for predicting whether drugs will be safe in humans.

More than 30 percent of promising medications fail in human clinical trials because they are found to have unacceptable toxicity, despite promising pre-clinical studies in animal models. The Tissue Chip for Drug Screening Initiative is developing 3-D human tissue biochips that model the structure and function of human organs, such as lung, liver, and heart. These chips will then be combined into an integrated system that can mimic complex functions of the human body. This technology will give researchers in both the public and private sectors the ability to predict more accurately how effective a therapeutic candidate will be in clinical studies, eliminating toxic and/or ineffective drugs much earlier in the development process.

Another way we are working to advance therapeutics development is through the Discovering New Therapeutic Uses for Existing Molecules program. This collaborative approach partners NIH researchers with industry to provide opportunities to reposition and repurpose partially developed therapeutic candidates for new disease indications. By using agents that already have cleared several key steps in the development process, scientists nationwide have a strong starting point to contribute their unique expertise and accelerate the pace of therapeutics development.

For example, through the New Therapeutic Uses program, a team at Yale recently partnered with AstraZeneca to obtain the drug, saracatinib, an experimental drug originally developed to fight cancer.

The drug is now showing promise against Alzheimer's disease – restoring memory loss and reversing brain problems in mouse models of Alzheimer's. Based upon these promising results, the researchers are testing saracatinib's effectiveness in humans.

Despite all the exciting work NIH is doing in the space, there are still challenges to innovation. One of the most important ways that biomedical researchers can learn about exciting breakthroughs and form collaborations is by attending scientific conferences. However, recent travel restrictions have made it difficult for NIH scientists to attend and contribute to these meetings. We appreciate this committee's interest in this issue, as well as this Congress's work to relieve NIH from some of these restrictions and help facilitate scientific collaborations that could lead to breakthroughs and cures.

Modernize Clinical Research and Data Access

The policies governing biomedical research, along with its translation and use, must be as innovative as the science we support. Today, I want to share two areas of ongoing policy evolution: enhancing data sharing and expanding the protections for participants in research.

Dissemination of research findings is fundamental to science and an inherent aspect of NIH's mission. NIH has always endeavored to ensure that, to the fullest extent possible, the results of federally-funded scientific research are made available to and are useful for the general public, industry, and the scientific community. Since 2003, NIH has articulated its commitment to data sharing through various policies and guidances, including the NIH Data Sharing Policy and, more recently, the Genomic Data Sharing Policy.

Last November, the Department of Health and Human Services and NIH released for public comment two proposals to increase the transparency of information about clinical trials through ClinicalTrials.gov, a publicly accessible database operated by the NIH National Library of Medicine (NLM). The first proposal was a Notice of Proposed Rulemaking (NPRM) that describes proposed

regulations to complete the implementation of Title VIII of the Food and Drug Administration Amendments Act of 2007, which apply to both publicly and privately funded trials of certain drug, biological, and device products regulated by the FDA. One key provision of the proposed rule is the expanding of the scope of clinical trials required to submit summary results to ClinicalTrials.gov to include trials of unapproved, unlicensed, and uncleared products. At the same time, NIH issued a draft policy that would apply the same registration and reporting requirements to *all* clinical trials funded by NIH, including both phase I trials that are not otherwise subject to the Title VIII requirements and trials of behavioral and other interventions not regulated by FDA. Both proposals aim to improve public access to information about specified clinical trials and to ensure that information about clinical trials and their results are made publicly available via ClinicalTrials.gov.

Increasingly, the scientific community and the public expect data generated with Federal funds will be shared to enable further insights to be gained, to help enhance the quality of research, to increase transparency in Federal research spending, and to improve the return on investment in research. Although data sharing is becoming a more integral part of the research process, NIH is stepping up its efforts on a policy front to advance data sharing. Explicit statutory authority allowing the NIH Director to require sharing of scientific data generated from NIH-funded grants would strengthen these efforts.

Medical advances would not be possible without the individuals who volunteer to participate in research. Patients, and their loved ones, need new and better diagnostics, treatments and prevention strategies. They want the research enterprise to move as quickly as possible. To speed the pace of research and increase efficiency, NIH has taken steps to modernize institutional review board (IRB) policies. IRBs play a critical role in assuring the ethical conduct of clinical research, and studies must be reviewed and approved by an IRB before they can begin. When the regulations for protection of human subjects were first published, most clinical research was conducted at a single institution. Since then, the research landscape has evolved, and many studies are carried out at multiple sites and within large

networks. Studies that go beyond a single site are often able to recruit more individuals from diverse populations. These multi-site studies can often generate important results in less time. However, working through IRB review at each site can delay initiation of the research without increasing the protections for the research participants. To help address that issue, NIH issued a draft policy in December 2014 to promote the use of single IRBs in multi-site clinical research studies. The draft NIH policy proposes that all NIH-funded, multi-site studies carried out in the United States should use a single IRB of record. Exceptions to the policy would be allowed if local IRB review is required by federal, state, or tribal laws or regulations or if necessary to meet the needs of specific populations. Increasing the use of single IRBs for multi-site studies will help reduce duplication of effort, speed the initiation of important research, and save time and taxpayer funds while maintaining the highest ethical standards.

Although NIH is taking many steps to protect research participants, there are some ways Congress can be of assistance. NIH supports strengthening protections for patient information, particularly individual level genomic data. Because individual-level genomic data are unique, new genomic technologies, when coupled with identifiable reference data, make it possible to identify an individual. Genomic data can reveal significant and sensitive personal information, including risks of developing conditions such as cancer or Alzheimer's disease. A statutory change establishing that individual-level genomic data are confidential would provide research participants with more robust privacy protections and enhance public trust and confidence in medical research. This will be particularly important as major new research efforts, such as the President's Precision Medicine Initiative, move forward.

Reduce Administrative Burden and Increase Efficiency

The significant administrative burdens placed on researchers may jeopardize or delay scientific progress. In 2009, the Federal Demonstration Partnership found that, based on a survey of faculty on active federally funded research grants, these individuals spend 42 percent of their time on administrative tasks related to grant rather than on research. Given this significant finding, NIH is committed to reducing administrative burdens on researchers and appreciates the committee's interest in reducing the administrative burdens on our grantees. As a Federal research agency, we are acutely aware that to achieve our mission we must serve as effective and efficient stewards of the resources provided by the American people. We would like to work with you on ways to enhance transparency and accountability without adding new, burdensome requirements. Reducing administrative burdens and eliminating duplicative requirements will allow Federal agencies and the research community to focus resources on the most value-added activities in finding cures.

Today, I have provided you with a brief overview of some of the exciting science supported by NIH, as well as some of the challenges facing the biomedical research enterprise. With your support, we can anticipate a bright future of accelerating discovery across NIH's broad research landscape, from fundamental scientific inquiry to translational and clinical research.

This concludes my testimony, Mr. Chairman. The NIH looks forward to continue working with you as the 21st Century Cures Initiative moves forward and I welcome any questions.