DEPARTMENT OF HEALTH AND HUMAN SERVICES

NATIONAL INSTITUTES OF HEALTH

Continuing America's Leadership in Medical Innovation for Patients

Witness before the

Senate Health, Education, Labor, and Pensions Committee

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Good morning, Chairman Alexander, Ranking Member Murray, and distinguished Members of the Committee. 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, alongside my dedicated colleague, Dr. Hamburg, to discuss how we, as a Nation, can drive innovation through Federal investments in scientific research. On behalf of NIH, our employees, grantees, and patient community, I want to thank Members of this Committee for your continued support and for holding this hearing today.

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 innovative strategies to achieve it.

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 cardiovascular disease have been reduced by more than 70 percent in the past 60 years. 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. 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.

Today, I want to share with you a few of the many promising opportunities for biomedical research innovation. I can assure you that the future of scientific research has never been brighter.

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

Scientific advances are accelerating progress toward a new era of personalized medicine. Historically, physicians have had to make 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. Technology developments, along with plummeting costs of DNA sequencing, now make it possible to develop an innovative approach to treatment that accounts for individual differences in patients' genes, environments, and lifestyles. To this end, through the President's Precision Medicine Initiative announced in January, NIH and our colleagues at FDA and the Office of the National Coordinator for Health Information and Technology will work together on this bold new research effort to revolutionize how we improve health and treat disease. A near term goal of the President's Precision Medicine Initiative focuses on cancer; cancer research has been leading the way in precision medicine by defining the driver mutations in individual tumors and using this information to design the ideal therapy for each patient. To accelerate the pace of discovery, this initiative seeks to expand current cancer genomics research to understand the development of resistance to targeted therapy, to apply non-invasive methods to track patients' responses to treatment, and to explore the efficacy of new drug combinations targeted to specific tumor mutations.

As a longer term goal of this initiative, NIH also plans to launch a national research cohort of one million or more volunteers, who will volunteer to play an active role in how their genetic and environmental information is used for the prevention of illness and management of a wide array of chronic diseases. This venture will pioneer a new model for doing science that emphasizes engaged participants, technologically advanced collection of many different data types, responsible data sharing, and privacy protection. A project of this magnitude will lay the foundation for a myriad of new prevention strategies and novel therapeutics. 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 personal approach into everyday clinical practice.

NIH also is supporting the basic science that is fundamental to scientific advances in biomedicine. 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.

NIH is also innovating in translational science -- where basic science findings are developed into clinical benefits. Let me give you a few examples.

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 business. 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 sift through a long list of potential therapeutic targets and biomarkers, and choose those most promising for therapeutic intervention. Besides NIH, AMP partners include FDA, ten biopharmaceutical firms, and a number of non-profits, including patient advocacy groups.

Accelerating Medicines Partnership (AMP)

Government	Industry	Non-Profit Organizations
NIH	AbbVie	Alzheimer's Association
FDA	Biogen Idec	American Diabetes Association
	Bristol-Myers Squibb	Arthritis Foundation
	GlaxoSmithKline	Foundation for the NIH
	Johnson & Johnson	Geoffrey Beene Foundation
	Lilly	Juvenile Diabetes Research Foundations
	Merck	Lupus Foundation of America
	Pfizer	Lupus Research Institute/Alliance for Lupus Research
	Sanofi	PhRMA
	Takeda	Rheumatology Research Foundation
		USAgainstAlzheimer's

This pre-competitive partnership is focusing initially on three disease areas 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 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 the lung, liver and heart. These chips are then combined into an integrated system that can mimic complex functions of the human body. This technology should enable scientists to predict more accurately how effective a therapeutic candidate would be in clinical studies, eliminating toxic and/or ineffective drugs earlier in the development process. Tissue chips will benefit basic and clinical researchers throughout the entire pharmaceutical and biotechnology sector.

Another way NCATS is 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 drugs for new 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. This approach utilizes crowd-sourcing to identify the most promising repurposing opportunities, avoiding research duplication and reducing the time and money required to determine if these well-developed agents can be used to treat other medical conditions.

Today, I have provided you with a 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 in biomedicine, and it is important for the United States to continue to lead in this effort. With your support, we can anticipate a future of accelerating discovery across NIH's broad research landscape, from fundamental scientific inquiry to a new era of personalized approaches to medical treatments.

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