DEPARTMENT OF HEALTH AND HUMAN SERVICES

NATIONAL INSTITUTES OF HEALTH

Hearing on "Prioritizing Cures: Science and Stewardship at the National Institutes of Health"

Witness appearing before the

Senate Health, Education, Labor, and Pensions Committee

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August 23, 2018

Good morning, Chairman Alexander, Ranking Member Murray, and distinguished Members of the Committee. I am Francis S. Collins, M.D., Ph.D., and I have served as the Director of the National Institutes of Health (NIH) since 2009. It is an honor to appear before you today.

Before I discuss NIH's diverse investments in biomedical research and some of the exciting scientific opportunities on the horizon, I want to thank this Committee for your sustained commitment to NIH to ensure that our nation remains the global leader in biomedical research and advances in human health.

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. As some of you have witnessed first-hand on your visits to NIH, our leadership and employees carry out our mission with passion and commitment. This extends equally to the hundreds of thousands of individuals whose research and training we support, located in every State of this great country, and where 81 percent of our budget is distributed.

One of my personal priorities is developing the next generation of talented biomedical researchers. Last year, I shared with the Committee NIH's plans to build on our support for early-stage investigators through a new initiative known as the Next Generation Researchers Initiative. NIH is developing evidence-based, data-driven strategies to assure that NIH investments are directed in ways that maximize scientific output. We are being aided in these efforts by an expert Working Group of the Advisory Committee to the Director, who will present recommendations in December 2018. But several important steps are already being taken: Institutes and Centers are placing greater emphasis on current NIH funding programs to identify,

grow, and retain new- and early-career investigators across these critical career stages. The Office of the Director is tracking progress across NIH in order to assess if these strategies are working. NIH remains committed to the development, support, and retention of our next generation of investigators.

NIH is also committed to funding the highest priority scientific discoveries while also maintaining fiscal stewardship of Federal resources. Truly exciting, world class science is taking place. I would like to provide just a few examples of the depth and breadth of the amazing research NIH supports across the Institutes and Centers.

The Brain Research through Advancing Innovative Neurotechnologies® (BRAIN) Initiative is revolutionizing our understanding of the human brain, the most complex structure in the known universe. Launched in 2013, this large-scale effort is pushing the boundaries of neuroscience research. Ultimately, these insights will have profound consequences for the prevention or treatment of a wide variety of brain disorders. By accelerating the development and application of innovative technologies, researchers are producing a revolutionary new dynamic picture of the brain that, for the first time, shows how individual cells and complex neural circuits interact in both time and space. This picture is filling major gaps in our current knowledge and providing unprecedented opportunities for exploring exactly how the brain enables the human body to record, process, utilize, store, and retrieve vast quantities of information, all at the speed of thought.

This year, the BRAIN Initiative will support critical areas including data infrastructure and sharing, the BRAIN Initiative Cell Census Network (which is developing an atlas of brain cell types), the Team Research Brain Circuits Program, and human brain studies. In human studies, the BRAIN Initiative is advancing brain imaging and non-invasive brain stimulation, and

public private partnerships are investigating self-adjusting implanted brain stimulation therapies that are already showing promise. Ultimately, this will lead to an increased understanding of brain health, and a means of preventing brain disorders such as Alzheimer's disease, Parkinson's, schizophrenia, autism, and drug addiction.

In April 2018, NIH launched the HEAL (Helping to End Addiction Long-term) Initiative, an aggressive, trans-agency effort to speed scientific solutions to stem the national opioid public health crisis. NIH has and will continue to support cutting-edge research on new treatments for the millions of Americans with opioid addiction, and for the millions more with daily chronic pain. Both pain and addiction are complex neurological conditions, driven by many different biological, environmental, social, and developmental contributors. To build on this understanding, NIH will: explore new formulations for overdose reversal medications capable of combatting powerful synthetic opioids; search for new options for treating addiction and maintaining sobriety; continue to research how best to treat babies born in withdrawal through our ACT NOW study; develop new non-addictive treatments for pain through the study of novel targets and biomarkers; and build a new clinical trials network focused on pain. NIH, in partnership with the Substance Abuse and Mental Health Services Administration (SAMHSA), will also study how effective strategies for opioid addiction and overdose reversal can be put into practice in places severely affected by the opioids crisis through the HEALing Communities study. Thanks to your support, all hands are on deck at NIH for this public health crisis.

Another exciting area of continued investment is in cancer immunotherapy, in which a person's own immune system is taught to recognize and attack cancer cells. After years of

research supported by NIH, immunotherapy is leading to cures of some cancers like leukemia, lymphoma, and melanoma.

But other cancers, particularly solid tumors like colon, pancreas, breast, and prostate, have proven much less responsive. I am excited to tell you that some of those barriers may be ready to come down. Just last month, a team led by NIH's Dr. Steve Rosenberg announced a novel modification of an immunotherapy approach that led to a complete regression, most likely a cure, of widely metastatic breast cancer in a woman with this previously fatal form of the disease. As always, I must counsel patience – this immunotherapy success story for solid tumors involves very few cases right now, and must be replicated in further studies. But, without doubt, this woman's life-saving experience represents hope for millions more. As exciting as potential cures like this can be, NIH is focused on advancing not just cancer therapies, but also cancer care. I would like to tell you about an NIH-funded trial that beautifully illustrates the progress we are making in this area.

Each year, as many as 135,000 American women who have undergone surgery for the most common form of early-stage breast cancer face a difficult decision: whether or not to undergo chemotherapy to improve their odds. Now, thanks to a large, NIH-funded clinical trial, called TAILORx, we finally have some answers. It turns out about 70 percent of such women actually do not benefit from chemotherapy, and a genomic test of tumor tissue can identify them quite reliably. Clearly, it is best to spare women from the potentially toxic side effects of these drugs, if at all possible. Furthermore, the ability to limit the use of chemotherapy to the 30 percent of women who will really benefit can yield significant cost savings for our health-care system, as much as $1.5 billion a year.

Indeed, figuring out what health approaches work best for each individual—and why—is the goal of another important NIH Initiative: the Precision Medicine Initiative (PMI). Precision medicine is a revolutionary approach for disease prevention and treatment that takes into account individual differences in lifestyle, environment, and biology. While some applications of precision medicine have found their way into practice over the years, this individualized approach is simply not available for most diseases. The *All of Us* Research Program, a key component of PMI, is building a national resource—one of the world's largest, most diverse biomedical data sets in history—to accelerate health research and medical breakthroughs, enabling individualized prevention, treatment, and care. *All of Us* will enroll one million or more U.S. volunteers from all life stages, health statuses, races/ethnicities, and geographic regions to reflect the country's diverse places and people to contribute their health data over many years to improve health outcomes, fuel the development of new treatments for disease, and catalyze a new era of evidence-based and more precise preventive care and medical treatment.

Across the nation, NIH has engaged 10 large health provider organizations, six community health centers, and the Department of Veterans Affairs to be our partners in this ambitious study. The program has funded over 30 community partner organizations to motivate diverse communities to join and remain in the program, with a focus on those traditionally underrepresented in biomedical research.

We began a robust, year-long beta testing phase in May 2017, during which each of our partners were able to test their systems and processes to ensure a good experience for participants and ensure that the security of the data systems was of the highest possible order. I am happy to tell you that *All of Us* launched nationally on May 6, 2018 with events across the country to mark the program's open enrollment. As of August 15, 2018, almost 100,000

individuals have started the enrollment process, and over 50,000 have completed all the steps in the protocol. Of those almost 50 percent are from racial and ethnic groups who have been historically underrepresented in biomedical research.

Following the national launch, we continue to improve and adjust the program based on participant feedback and emerging scientific opportunities and technological advances. We also are currently building the *All of Us* data resource, which is designed to be used by a broad range of researchers to study complex risk factors, support ancillary studies and clinical trials, and link to other large data sets. *All of Us* will be critical to realizing the promise of personalized medicine.

We have never witnessed a time of greater promise for advances in medicine than right now. Your support has been critical, and will continue to be. Thank you again for inviting NIH to testify today. I look forward to answering your questions.