

DEPARTMENT OF HEALTH AND HUMAN SERVICES

NATIONAL INSTITUTES OF HEALTH

The Transformative Power of Biomedical Research

Witness appearing before the
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Good morning, Chairman Cole, Ranking Member DeLauro, and distinguished Members of the Subcommittee. 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, and it was a pleasure to host many of you at NIH in February.

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 Subcommittee for your sustained commitment to NIH to ensure that our nation remains the global leader in the life sciences 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 believe passionately in our mission. This extends equally to the tens 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.

I would like to provide just a few examples of the depth and breadth of the amazing research being supported across the Institutes and Centers of NIH.

The core of our mission remains basic biomedical science. Given the exploratory and, hence, unpredictable nature of fundamental discovery, basic science is generally not supported in the private sector – but it provides the critical foundation for advances in disease diagnosis, treatment, and prevention through future clinical applications. Virtually none of the substantial gains in reducing human suffering and extending longevity over the last century would have happened without basic science. NIH's emphasis on fostering innovation to understand

fundamental biological processes has led to no fewer than 149 Nobel Prizes to our grantees, and is leading year by year to new and more effective ways to treat complex medical conditions.

As a current example, the emergence of “cryo-EM,” a new form of electron microscopy, has dramatically sped up the time needed to visualize the exquisite details of biological structures including protein-protein and protein-drug complexes. This is a major revolution in structural biology that already is transforming drug design.

Basic research is also fueling new advances in our understanding of the brain, which will be critically important for treating diseases such as Alzheimer’s disease, Parkinson’s disease, autism, epilepsy, traumatic brain injury, and others. Through the Accelerating Medicines Partnership (AMP), a public-private partnership between NIH, the Food and Drug Administration (FDA), 10 biotechnology companies, and nonprofit organizations, we have joined ranks across sectors to expand our understanding of Alzheimer’s disease. In one component of AMP, researchers are analyzing large-scale molecular data from thousands of affected and unaffected human brain samples, including genomic, gene expression, and protein measures. With this information, NIH and our partners are building new molecular pathways to understand the cause of Alzheimer’s, and charting a course for entirely new ways to detect and treat this devastating disease that go beyond the previous understanding of the amyloid and tau proteins. By working with industry and sharing data widely in the scientific community, NIH aims to shorten the time between these discoveries and the development of new strategies for Alzheimer’s disease treatment and prevention.

Rare diseases also represent an area of great need and great opportunity, one which NIH continues to be uniquely positioned to address. Though such diseases are individually rare, collectively an estimated 25 to 30 million Americans are affected. Great advances have been

made through genomic science in uncovering the cause of rare diseases, and that has led to dramatic improvements in diagnosis. But of the 6,500 identified rare and neglected diseases for which the molecular cause is now known, only about 500 have approved treatments. The private sector generally finds it difficult to mount expensive initiatives for such small markets – the risks are too high. Finding new treatments thus requires NIH to play a lead role – by investing in the early stage of therapeutic development to “de-risk” such projects. While almost all Institutes and Centers at NIH work on rare diseases, the National Center for Advancing Translational Sciences (NCATS) has a particular focus on this area of opportunity.

As an example, autoimmune pulmonary alveolar proteinosis (aPAP) is a rare, potentially fatal disease marked by a build-up of lipids and proteins in the lungs, and leads to respiratory failure. The current treatment for severe aPAP is whole-lung lavage, whereby both lungs are repeatedly filled and washed with a salt solution. This procedure is complicated, dangerous, and must be repeated throughout a patient’s entire life. NCATS has supported efforts to develop an inhaled treatment for aPAP, providing support and expertise to the basic research, pre-clinical research and testing, and early-phase clinical trials.

Other transformative technologies are offering dramatic new approaches to achieving a truly molecular cure of rare diseases. For example, experts are now testing genetic therapy in bone marrow stem cells as a curative treatment for sickle cell disease, the first human disease understood at the molecular level and the most common inherited blood disorder in the United States, affecting over one hundred thousand Americans at a yearly cost of hundreds of millions of dollars.

As a final example, consider how fundamental research over many years now promises to transform medicine for patients with advanced cancer: immunotherapy. For decades, basic

scientists have worked to understand how the immune system functions at the molecular level. Now, thanks to a series of dramatic advances, we can not only watch the immune system at work, we can instruct it – “send it to school.” In a recent breathtaking example, a young woman with widely metastatic breast cancer, whose cancer had failed to respond to several rounds of chemotherapy, enrolled in an experimental protocol at the NIH Clinical Center as a last hope. Her tumor genome was sequenced, and rare immune cells in her body with the potential to seek and destroy those cancer cells were identified. After those immune cells were massively expanded in the laboratory, and then unleashed to go after the cancer, her tumors started to recede within days. Now more than a year later, there is no evidence of any remaining cancer in her body. She is part of a revolution in cancer treatment, all made possible by years of dedicated basic research in fields like immunology and genomics.

So the future has never been brighter for advances in biomedical research than right now. Imagine what this feels like for a talented and curious new investigator. Early-stage investigators are responsible for many of the advances I’ve told you about today, and our future depends on them and their bright ideas. Those young men and women are thrilled by the prospect of exploration, and driven to help people. NIH is responsible for training these scientists, and for making sure that our investment in their careers, and the potential advances they will bring to patients, are sustained into the next stage. They are our most important resource. If advances in medical research are to continue, if research is to lead to breakthroughs that can reduce health care costs, if the considerable economic return on research is to continue, and if America is to continue its global leadership in biomedicine, we need to be sure this next generation has the confidence that there will be support for them. This is a priority for me.

NIH is preparing to implement a new measure to allow a broader number of meritorious investigators, particularly those in early- and mid-career, to receive NIH funding through new and renewed grants. A number of recent studies have demonstrated that while NIH support is essential to ensure the productivity of an investigator, there is a point of “diminishing returns” if an investigator becomes overextended. Quality science and fiscal stewardship require time and effort, and it stands to reason that a person can be stretched too thin. We are therefore proposing to work with NIH grant applicants and their institutions to limit the total NIH support that any one principal investigator may receive through research currently funded by NIH, allowing NIH funds to be more broadly distributed. Opening up opportunities for highly meritorious investigators at all stages of career development will ensure that NIH will remain a good steward of trusted public dollars, and strengthen the biomedical research workforce for the future. We are working with stakeholders now to determine the best way to move forward on this important goal.

I have provided you with examples of how investments in bright new ideas in biomedical research are advancing human health, spurring innovations in science and technology, stimulating economic growth, and laying the groundwork for the future of the United States biomedical research enterprise. 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.

This concludes my testimony, and I look forward to answering your questions.