DEPARTMENT OF HEALTH AND HUMAN SERVICES

NATIONAL INSTITUTES OF HEALTH

# The Transformative Power of Biomedical Research

Witness appearing before the

House Appropriations Subcommittee on Labor, HHS, Education, and Related Agencies

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Good morning, Chairman Cole, Ranking Member DeLauro, and distinguished Members

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.

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

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.