DEPARTMENT OF HEALTH AND HUMAN SERVICES

NATIONAL INSTITUTES OF HEALTH

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Witness appearing before the

Senate Subcommittee on Labor ±HHS ±Education Appropriations

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Good afternoon, Mr. Chairman and distinguished Members of the Subcommittee. I am

TCGA research network of investigators recently identified promising new therapeutic targets in squamous cell carcinoma of the lung, the second most common form of lung cancer, including three families of enzymes that act as molecular switches.[2] These findings lay the foundation for the development and implementation of advanced diagnostics and treatments for squamous cell cancer. Moreover, they underscore the value and promise RI RXU QDWLRQ¶V LQYHVWPH

Another new and exciting area of basic research is the Human Microbiome Project. Microbes inhabit many parts of the human body and have often had a bad reputation for causing sickness. But more often than not, they actually contribute to the health of their human hosts. In a five-year endeavor supported by the NIH Common Fund, 200 scientists at 80 institutions sequenced the genomes of bacteria from multiple body sites of 250 individuals, with striking results. The research showed that certain communities of bacteria help keep people healthy, whereas others appear to make people more susceptible to disease.[3] When the bacterial population in the intestinal tract gets disrupted, chronic conditions such as obesity can result; this new understanding may provide us with novel ways to address this serious health threat. An unexpected result from another NIH-funded study was that poor diet is not the only contributor to malnutrition. In fact, a bad assortment of microbes in the gut can conspire with a nutrient deficient diet to lead to severe malnutrition.[4]

A final example I want to provide of how NIH-supported research is accelerating scientific discovery is in the area of stem cells. Induced pluripotent stem (iPS) cell technology is revolutionizing the way we study disease, and holds the promise of dramatic advances in treatment. iPS cells are patient-derived cells, typically from skin, that scientists can reprogram back to an embryonic stem cell-like state. These cells can then be induced to turn on specific sets of genes to differentiate into a variety of cell types, including blood cells, liver cells, or neurons. This means researchers can re-FUHDWH D SDWLHQW¶V GLVHDVH LQ D compounds against the cells ² rather than the patient ² to determine drug toxicity and efficacy. %XW LW¶V DOVR SWKDW WKHVH FHOOV FRXOG EH XVHG WKH genetic misspellings could be corrected in their own iPS cells, and then programmed and delivered to a tissue where they are sorely needed. Recent NIH-funded studies have developed copy-editing enzymes that are making it faster, easier, and cheaper to correct genetic typos. In 2011, researchers used a specially engineered copy-editing enzyme to find and correct the mutation that causes sickle cell anemia using iPS cells derived from a patient with the disease.[5] Two very recent, groundbreaking discoveries along this same avenue are the development of the QH[W JHQHUDWLRQ PHWKRGRORJ\ RI µILQG DQG UHSODFH¶ copy-edit the genome.[6,7]

While these exciting findings have led to a much deeper understanding of health and human disease, much more work needs to be done in order to move these strategies and others like them out of the lab and into the clinic ² and to do so as quickly as possible. To this end, the

[2] http://www.nature.com/nature/journal/v489/n7417/pdf/nature11404.pdf
[3] http://www.nature.com/nature/journal/v486/n7402/pdf/nature11209.pdf
[4] http://www.sciencemag.org/content/339/6119/548.full.pdf
[5] http://onlinelibrary.wiley.com/doi/10.1002/stem.718/pdf
[6] http://www.sciencemag.org/content/326/5959/1501.full.pdf
[7] http://www.sciencemag.org/content/339/6121/819.full.pdf

that we are eager to pursue in order to determine if early intervention can influence this terrible disease.

With advancing scientific and technological capabilities, such as genome sequencing machines and high resolution medical imagers, biomedical researchers are generating huge amounts of data at an unprecedented pace. The need to integrate and analyze massively complex datasets is referred to as the Big Data challenge ±a challenge that we must overcome to gain a deeper understanding of disease and develop the next generation of therapeutic targets.

Managing Big Data is a critical part of translating scientific discoveries into clinical applications. To address this challenge, NIH is developing the Big Data to Knowledge (BD2K) program, which will be launched in FY 2014. BD2K will support four programmatic efforts: (1) facilitate the broad use and sharing of large, complex biomedical data sets through the development of policies, resources and standards; (2) develop and disseminate new analytical methods and software; (3) enhance training of data scientists, computer engineers, and bioinformaticians; and (4) establish Centers of Excellence to develop generalizable approaches that address important problems in biomedical analytics, computational biology, and medical informatics. In FY14, NIH will invest at least $40 million in the BD2K program through the Common Fund, and each Big Data Center of Excellence will be funded at $2 million to $5 million per year for three to five years. As Big Data challenges in biomedical research are shared with other areas of scientific research such as energy and space research, BD2K will also require effective collaboration and coordination with other government agencies tackling similar challenges, including the National Science Foundation and the Department of Energy, as well as privately funded efforts. With the proper investments and efforts, we will overcome

grant applications.