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Saving Lives Through Medical Research

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Mr. Chairman and members of the subcommittee, thank you for the opportunity to appear before you to discuss how investments in biomedical research save lives every day through the development of new therapies and treatments.

My name is Timothy J. Eberlein. I am an actively practicing physician and also serve as the Chairman of the Department of Surgery at the School of Medicine at Washington University in St. Louis. I also serve as the Director of the Alvin J. Siteman Cancer Center.

Thank you, Chairman Blunt and Ranking Member Murray, for the opportunity to speak to the Subcommittee today, and for your leadership in working to ensure that the federal government makes a significant and sustained investment in biomedical research. I also want to thank the full Subcommittee for your work in providing substantial new resources for the National Institutes of Health and for continuing to make NIH a priority in a challenging federal budget environment.

Our cancer center is a joint venture of Washington University and Barnes-Jewish Hospital in St. Louis, Missouri. We are the only NCI-designated comprehensive cancer center in the State of Missouri, and our 450 physicians and scientists care for over 50,000 cancer patients every year, patients who come to St. Louis from Missouri, surrounding states and across the nation.

Our patients are seeing the benefits of federal investments in research. Washington University was highly involved in the Human Genome Project, contributing roughly 25% of the final code. We have used that expertise to pioneer the sequencing of cancer genomes, allowing us to identify the genetic differences between healthy and cancerous tissues. As our scientific understanding has advanced, we have sought to apply this research to the clinical setting. One illustrative example involves Dr. Lukas Wartman, an oncologist and leukemia survivor, who experienced a second relapse of his disease while a fellow at Washington University. Researchers performed a detailed analysis of Lukas's cancer genome, and they found a gene which was expressing at a much higher level than normal. The research team then identified an existing drug typically used to treat kidney cancer, which targets tumors with this specific gene. Through this precision therapy, Dr. Wartman's disease went into remission, further enabling him to undergo a stem cell transplant. He now is working to care for cancer patients, and under his leadership, we have established a multidisciplinary Genomics Tumor Board that meets regularly to identify patients who might benefit from genome sequencing. Dr. Wartman embodies the idea behind the Cancer Moonshot and the Precision Medicine Initiative, where we target therapies and treatments to the unique genetic characteristics of the patient and their disease.

Utilizing sophisticated genomic analysis, we are on the cusp of fundamentally changing how we think about treating cancer by using targeted therapies that avoid unnecessary expensive treatments. By combining genomic mutational analysis of an individual's cancer, we are now doing clinical trials that treat many solid tumors such as breast, brain, melanoma, lung and head and neck cancers using a vaccine tailored to eradicate the patient's specific tumor with minimal side-effects and morbidity. Another opportunity comes through the use of nanoparticles to deliver therapies. Multiple myeloma is a cancer of the bone marrow that responds initially to chemotherapy, but the cancer usually recurs and becomes more resistant to treatment. We have had drugs that should eradicate the disease, but they tend to degrade once administered to the patient. Putting these drugs into nanoparticles, however, we are able to target the myeloma cancer cell, eradicating it with minimal side effects. Each of these novel clinical trials occurred at our cancer center funded through investigator-initiated research made possible by the NIH.

In my own practice, I treat patients with breast cancer. Traditionally, we have operated on pre-menopausal patients who have early stage breast cancer, and then treat them with radiation therapy and chemotherapy. However, we know that approximately four out of five of these patients might be cured with surgery and radiation therapy alone. The scientific challenge is that we do not yet know how to distinguish between the 20 percent who need the additional chemotherapy from the 80 percent who don't. Can you imagine for a moment, what that would mean to patients if we were able to make this determination – how their lives would improve if they were not subjected to the side-effects of chemotherapy? Can you imagine how the cost of their care would decline, if we avoided unnecessary therapy in four out of five of these cases? As we continue to develop our genomic understanding of cancer, I am confident we can get to the point where affordable

personalized cancer treatments will be widely available but we need sustained, stable federal support for research to get us there.

Another challenge I face in the operating room is being able to distinguish between cancerous and healthy tissue, and knowing exactly how much tissue to remove. Dr. Samuel Achilefu, a Washington University professor of radiology, has developed a set of goggles that help surgeons see and remove cancerous tumors as small as 1 mm in diameter, the thickness of about 10 sheets of paper. After a dye is injected into a patient's tumor, the cancerous cells "glow" when bathed in infrared light and viewed by the goggles. Dr. Achilefu's lab is also investigating phototherapy, killing cancer with light, through a new approach that utilizes already available radiopharmaceutical drugs that can create a light source within tumor cells. The light stimulates light-sensitive molecules that have been delivered to the cancer cells, converting them into highly toxic drugs. The advantage of this strategy is that it minimizes the impact on neighboring healthy tissue, which could lead to reduced side effects and better outcomes overall.

Advances such as these are why we have been successful in reducing cancer mortality by 25% since 1991. The change in mortality in children has been even more dramatic, with the death rates among those aged birth to 19 having dropped 66 percent between 1970 and 2014. I am particularly heartened by this progress with children. Adults have a greater ability to modify their behaviors that can lead to cancer – such as smoking or unhealthy diet. Children typically do not control the environment or the lifestyle decisions that can lead to their cancer. Thus, the reduction in mortality for children is a direct result of improvements in treatment-treatments largely discovered through investigations made possible through grants from the NIH.

Sustained appropriations with increased funding to advance novel discoveries and insights are responsible for the dramatic examples and improvement of life expectancy of patients with cancer in the United States. These investments have allowed us both to understand the fundamental biology behind disease and then to develop the strategies needed to develop therapies and cures. What may be even more important than the actual research, is the fact that virtually every scientist -- whether in academia or industry -- likely benefitted by training through the National Institutes of Health either by training in Bethesda, like I did, or through utilization of NIH training grants and/or career development grants. The reach of this funding in providing jobs and sustaining careers is monumental. But, even more critical, this training equips scientists with the skills needed to develop 21st Century cures. By equipping our nation's best and brightest minds to tackle these incredibly difficult problems, we are making an investment for which the country will reap benefits for decades to come.

Thank you again for the opportunity to speak today, and I look forward to answering any questions you may have.

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[1] Siegel, R. L., K. Miller, A Jemal "Cancer Statistics, 2017." *CA: A Cancer Journal for Clinicians.* January/February 2017. p. 18.
[1] Ibid. p. 27