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Chairman
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United States Senate Committee on Appropriations
Driving Innovation Through Federal Investments
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Chairman Mikulski, Ranking Member Shelby, and members of the Committee: On behalf of tens of millions of American families suffering from Alzheimer's disease and dementia today – and the tens of millions more who will receive this sentence if the current trajectory of the disease remains – I thank you for calling this hearing to provide a comprehensive overview of our government's core research enterprises. Unfortunately given the way our government and appropriations bills are organized, we rarely have such an opportunity to have the leaders of these departments and agencies available for one session, potentially hindering our ability to set national research goals and priorities, to maximize national research coordination, to track progress against goals and to adopt innovative practices in how we most productively invest our research resources. I hope this session results in a candid interchange and leads to some actionable ideas to strengthen and enhance our research activity.

As proven many time before, a robust, well-functioning and properly prioritized federal research agenda is necessary to spur innovation and discovery, sustain our standard of living, improve life for our citizens and those of the world and define the United States as the world's top innovator. There is no guarantee that any single research project will bear its intended fruit, and as any scientists can attest, failure is far more common than success. But failures in a scientific context can yield important information, and we should not fear or deter failing; rather, we must figure out how to learn from them and to front-load such experiences to the greatest extent possible. What we should fear is failing too late in the research process, when such setbacks are far more costly than they would have been had they occurred earlier.

We should also make greater efforts to establish national research goals and priorities, to establish research projects deemed to be of greatest need within those priorities, to drive greater levels of collaboration and coordination where appropriate and to increase our support for platforms focused on goal-oriented, milestone-driven and high-risk/high-reward projects.

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The benefits of a robust government research enterprise are numerous. More than a half-century ago, a commitment to research positioned the United States as the world's innovator for decades. Commitments to medical research have enabled us to make tremendous strides against numerous diseases including polio, cardiovascular disease, HIV/AIDS and many forms of cancer. They have also yielded numerous discoveries, like the mapping of the human genome, that have increased our understanding of human development, knowledge that applies to countless diseases and conditions.

Despite these successes, many more challenges of equal or greater magnitude remain before us: Translating the immense knowledge of our genetic makeup into treatments and therapies for thousands of diseases, developing commercially viable renewable energy solutions, improving the sustainability of our agricultural system to feed a growing world population, developing space travel technologies to

probe the outer corners of the universe and preventing tens of millions of Americans from losing their minds to Alzheimer's and dementia.

With government budgets constrained today and for the foreseeable future, I would urge this committee to use its immense influence in establishing priorities to make biomedical research a top priority. I recognize that doing so means you will need to make and justify tough decisions – in a flat or declining environment, any dollar increase to research will have to come from somewhere else. In addition to striving to increase funding for research, I would urge that this be paired with a complementary effort to better organize and prioritize the research enterprise to ensure it is focused on addressing the most urgent issues facing our nation today rather than locked into a funding pattern from yesteryear.

In setting our research priorities, it is, in our view, imperative that we recognize that our nation is facing a historic challenge of financing health care for our aging populations. Entitlement spending is consuming an ever-increasing portion of our national budget as our Baby Boomers pass age 65, at a rate of 10,000 a day. This is a challenge, but it is also an opportunity. Our national growth in the last half of the 20th century was driven in significant part by the growth and productivity of our work force. This will also be the case in this century because it will be those nations that best understand how to use their aging populations as an element in their national economic strategy and as a force for national competitiveness that will succeed. Those that regard their aging populations simply as beneficiaries of entitlement systems will, in our estimation, fail.

Older populations can add experience and scale to our workforce, and they can continue to contribute to our economy from home or the workplace, so long as they remain intellectually and cognitively alert. Through new communications and other technologies, work can move to wherever the worker may be, again, so long as the worker is cognitively healthy. To achieve this, we should set a goal of increasing the percentage of our population above the current retirement age that is able to engage in such work and contribute economically, and develop a research agenda to make this goal possible.

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The budget for the National Institutes of Health is an example of the impact possible when we set and fund research priorities as well as for the need to more regularly, transparently and thoroughly revisit our priorities. A quick review of NIH spending tables over the past 30 years demonstrates that we have made progress by-and-large in the fields that have been most heavily funded. In 1984, the top funded NIH institutes were those focused on cancer, health lung and blood disorders, diabetes, digestive and kidney disease and general medicine. Ten years later, the top funded institutes were NCI, NHLBI, NIAID – reflecting the greater focus on HIV/AIDS research – and NIGMS; and in 2014 the top-funded institutes are NCI, NHLBI, NIAIDS and NIGMS. While the last thing I would desire would be igniting a wildfire that pits disease/condition advocates or institutes and centers against one another, the data appears to raise the valid question: Is the NIH focusing its scarce resources where they are most needed? If not, what can Congress do to make sure such evaluations occur regularly and in a transparent and open manner?

It is imperative in this climate of stable or declining funding that we explore new or reformed processes for selecting the most meritorious projects put forward and that we not simply reward tenured investigators or those positing relatively "safe" projects. Rather, we must strive to develop and fund balanced portfolios that include a healthy amount of meritorious proposals that may be real game

changers in their fields. We did not get to the moon or develop disease modifying drugs for HIV/AIDS by playing it safe.

Related to this point, I think we can learn a lot of lessons from DARPA, which is one of our nation's crown jewels of the research ecosystem. Many in government and the private sector have attempted to replicate the impact of DARPA with mixed results, and it has been noted by experts that many characteristics – particularly time-bound projects, limited employment tenure and regular movement of employees and no fear of failure – are vital. Rather than seek to and fail at developing DARPA-lite models, I believe we should consider ways to leverage the existing DARPA – and its core components – by perhaps developing more collaborative approaches between the agency and other research agencies, like NIH, to advance a core set of projects each year supported by a defined budget from the collaborating agency.

For example, if two percent or nearly $600 million of the NIH budget were committed each year to advance a set of goal-oriented, milestone- driven, time-bound and high-risk/high-reward initiatives, could we help overcome some of the most challenging barriers in our way today? In the Alzheimer's space alone, I could think of several candidate topics – such as low-cost, non-invasive biomarkers that predict with confidence the likelihood of developing Alzheimer's or affordable tools to detect the disease at its earliest stages possible – that would be worthy candidates for such an effort.

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Finally, I would urge that your committee work with the research leaders to ensure that we are not funding unnecessarily duplicative studies and to maximize the likelihood that the fruits of government research funding are of a high quality. Earlier this year, the Government Accountability Office (GAO) released a report on duplicative programs that included a concern that 84 percent of the 1,200 autism projects funded by the federal government between FY 2008 and 2012 have the potential to be duplicative. This concern came despite an Interagency Autism Coordinating Committee that exists to help drive greater levels of coordination and prevent such duplication. While the GAO was careful to note it found the potential for – rather than conclusive proof of – duplicative funding, its identification of limited agency coordination are troubling and suggest that more must be done, particularly in areas being explored by multiple funders.

In addition to preventing duplication, we need to make sure that what is funded by our government is deemed to be valuable by those outside of academia. While the mission of the NIH is not to make life easier for industry, it is to apply knowledge to enhance, lengthen life and reduce illness and disability. As a vital cog of the larger research ecosystem, NIH has a role to play in ensuring the fruits of its scarce resources are seen as valuable. As demonstrated in other diseases states (e.g., cancer, HIV/AIDS), industry must be recognized as a partner in advancing American innovation. Looking to prioritize NIH in a fashion that will yield industry-ready outputs will only help to further our ability to advance American biomedical innovation – catapulting our nation's ability to remain the world's top innovator.

Thank you, again, for calling this important hearing.