Statement by the Ad Hoc Group for Medical Research on
FY 2016 Appropriations for the National Institutes of Health
Submitted for the record on April 3, 2015, to the
Subcommittee on Labor, Health and Human Services, Education and Related Agencies
Committee on Appropriations
United States Senate

The Ad Hoc Group for Medical Research is a coalition of more than 200 patient and voluntary health groups, medical and scientific societies, academic and research organizations, and industry. We appreciate the opportunity to submit this statement in support of strengthening the federal investment in biomedical, behavioral, social, and population-based research conducted and supported by the National Institutes of Health (NIH).

The Ad Hoc Group is deeply grateful to the Subcommittee for its long-standing and bipartisan leadership in support of NIH. We continue to believe that science and innovation are essential if we are to continue to improve our nation's health, sustain our leadership in medical research, and remain competitive in today's global information and innovation-based economy.

Despite increases provided in each of the past two fiscal years, the NIH budget remains lower than it was in FY 2012 in actual dollars. Additionally, since 2003, NIH funding has declined by 23 percent after adjusting for biomedical inflation. While the President's FY 2016 budget represents a much needed next step by increasing NIH funding above biomedical inflation, the Ad Hoc Group's members believe that the ongoing and emerging health challenges confronting the United States and the world, and the unparalleled scientific opportunities to address these burdens demand a funding level of at least $32 billion in FY 2016. We look forward to working with Congress and the Administration to achieve this goal through the annual appropriations process.

The Ad Hoc Group also urges Congress and the Administration to work in a bipartisan manner to end sequestration, the continued cuts to medical research that squander invaluable scientific opportunities, discourage young scientists, threaten medical progress and continued improvements in our nation's health, and jeopardize our economic future.

NIH: A Public-Private Partnership to Save Lives and Provide Hope

The partnership between NIH and America's scientists, medical schools, teaching hospitals, universities, and research institutions is a unique and highly-productive relationship, leveraging the full strength of our nation's research enterprise to foster discovery, improve our understanding of the underlying cause of disease, and translate this knowledge into the next generation of diagnostics, therapeutics, and other clinical innovations. Nearly 84 percent of the NIH's budget is competitively awarded through more than 55,000 research and training grants to more than 300,000 researchers at over 2,500 universities and research institutions located in every state.

The federal government has an essential and irreplaceable role in supporting medical research. No other public, corporate or charitable entity is willing or able to provide the broad and sustained funding for the cutting edge basic research necessary to yield new innovations and technologies of the future.

Research funded by NIH has contributed to nearly every medical treatment, diagnostic tool, and medical device developed in modern history, from a new treatment for cystic fibrosis to an awareness campaign that resulted in a dramatic decrease in the number of infants lost to Sudden Infant Death Syndrome to a vaccine to

prevent cervical cancer. We are all enjoying longer, healthier lives thanks to the federal government's wise investment in this lifesaving agency. Examples of recent breakthroughs made by NIH-supported scientists include:

- A Phase I clinical trial to assess the safety, efficacy, and immunogenicity of an intramuscular Ebola vaccine co-created by NIH and GlaxoSmithKline. Results indicated this vaccine was well-tolerated and elicited anti-Ebola antibody responses in healthy adult volunteers. Another promising vaccine candidate began Phase I trials in October 2014 in thirty-nine healthy volunteers. The vesicular stomatitis virus (VSV) Ebola vaccine studies are being conducted in collaboration with the U.S. Department of Defense and NewLink Genetics Corp. A parallel study is ongoing at the Walter Reed Army Institute of Research to evaluate in real time the vaccine's safety when provided at different dosages and compare the immune responses induced by one injection versus two.
- NIH-supported scientists contributed to the first comprehensive 3-D atlas of gene expression in the developing human brain as part of a larger project to profile gene expression throughout the course of brain development. The results provide a powerful map to link areas of the brain to genes tied to neurodevelopmental disorders and human-specific brain functions. This resource will help reveal the early roots of brain-based disorders, such as autism and schizophrenia.
- A new technology called CRISPR (clustered regularly interspaced short palindromic repeats) is allowing scientists to specifically target genes for deletion, addition, activation, or suppression in what amounts to performing their own genetic microsurgery. Using this system, NIH-supported researchers have altered DNA in human cells, rats, mice, zebrafish, bacteria, fruit flies, yeast, nematodes, and crops. This wide-ranging applicability makes the technology valuable for numerous applications, including conducting large-scale genetic screens in mammalian cells (recently validated by NIH-funded scientists), as well as the promise of new treatments for genetic diseases.
- The Recovery After an Initial Schizophrenia Episode (RAISE) initiative aims to prevent long-term disability in individuals with serious mental illness through early intervention. RAISE comprises two complementary efforts: the Early Treatment Program, which is continuing to follow patients for an additional three to four years to investigate the long-term impact of early intervention; and, the Connection Program, which successfully integrated team-based, multi-element services targeting the first episode of psychosis (FEP) in mental health systems in New York and Maryland, and is now evaluating promising strategies for reducing the duration of untreated psychosis among persons experiencing FEP. NIH has collaborated with the Substance Abuse and Mental Health Services Administration to translate early RAISE findings into guidance for states regarding evidence-based approaches to FEP treatment, and assembled a broad range of training resources developed through RAISE for use by state-supported Community Mental Health Centers.
- Current treatments for Hemophilia, a rare bleeding disorder in which the blood fails to clot normally, require a lifetime of frequent injections, often twice a week, of an expensive clotting factor called factor IX to restore normal clotting. A recent NIH-funded clinical trial used gene therapy to reprogram the body's own cells to produce factor IX using special viruses that have been engineered not to cause diseases. When adult men with hemophilia were given an intravenous dose of the therapy, patients who received the higher dose improved markedly, with the effects lasting for the entire 4-year period of the study.
- NIH-funded researchers developed a 3-D scaffold that guides the development of stem cells into specialized cartilage-producing cells, an approach that could allow for the creation of orthopedic implants to replace cartilage in patients with arthritis. This approach could allow for implants that restore function to a joint immediately and drive development of a mature, viable tissue replacement.

- Lung cancer solid tumors are particularly difficult to detect. NIH-funded scientists used genetic data from the Cancer Genome Atlas (TCGA) database to develop a molecular signature for non-small-cell lung cancers. Using this signature and samples from patients with non-small-cell lung cancer, researchers designed a highly sensitive DNA-based blood test that accurately identified all patients with advanced lung cancer, as well as half of patients whose lung cancer was in its earliest stage. This simple blood test was shown to detect solid tumors rapidly and accurately, track their progression over time, and could possibly predict their response to treatment. Efforts are now underway to conduct clinical trials to measure this technique and its potential to improve the detection of many different kinds of solid tumors.
- TB is treated with antibiotic drugs, but the bacteria that cause TB have evolved to become resistant to these medications. An NIH-funded research team analyzed the structure of an existing antibiotic and made various chemical modifications to create a new class of agents that were active against both multidrug-resistant (MDR) and extensively drug-resistant (XDR) bacteria. These compounds were not toxic in laboratory assays or in animals, and a subset of the compounds was highly effective against TB infections in mice. This work represents an initial step in the development of a new class of drugs to treat TB.
- Sickle cell disease is a genetic blood disorder that causes defective hemoglobin, the protein in red blood cells that carries oxygen. It affects millions worldwide, including approximately 100,000 people in the United States. The disease disproportionately affects African Americans, and current treatments are largely ineffective. A recent NIH-funded study showed that a stem cell transplant from a healthy relative could reverse the disease in 87 percent of patients. NIH research also is working towards a drug therapy for sickle cell disease. Through a collaborative agreement, researchers at the National Center for Advancing Translational Sciences' (NCATS) Therapeutics for Rare and Neglected Diseases (TRND) program and AesRx, a biopharmaceutical company, developed a drug candidate to treat sickle cell disease that specifically targets the underlying disease mechanism. The success of a Phase II clinical trial to evaluate safety and effectiveness has resulted in the recent acquisition of the drug by a pharmaceutical company that will advance the clinical development activities required for regulatory approval and commercialization.

For patients and their families, NIH is the "National Institutes of Hope."

NIH is the world's premier supporter of merit-reviewed, investigator-initiated basic research. This fundamental understanding of how disease works and insight into the cellular, molecular, and genetic processes underlying life itself, including the impact of social environment on these processes, underpin our ability to conquer devastating illnesses. The application of the results of basic research to the detection, diagnosis, treatment, and prevention of disease is the ultimate goal of medical research. Ensuring a steady pipeline of basic research discoveries while also supporting the translational efforts necessary to bring the promise of this knowledge to fruition requires a sustained investment in NIH.

Stagnant Funding Threatens Scientific Momentum

Over the past decade, NIH has lost more than 23 percent of its budget after inflation, significantly impacting the nation's ability to sustain the scientific momentum that has contributed so greatly to our nation's health and our economic vitality. The leadership and staff at NIH and its Institutes and Centers has engaged patient groups, scientific societies, and research institutions to identify emerging research opportunities and urgent health needs, and has worked resolutely to prioritize precious federal dollars to those areas demonstrating the greatest promise. At the same time, a continued erosion of our national commitment to medical research

threatens our ability to support a medical research enterprise that is capable of taking full advantage of existing and emerging scientific opportunities.

Perhaps the most destructive and long-lasting impact of the decline in the NIH budget is on the next generation of scientists, who see training funds slashed and the possibility of sustaining a career in research diminished. The continued success of the biomedical research enterprise relies heavily on the imagination and dedication of a diverse and talented scientific workforce. Of particular concern is the challenge of maintaining a cadre of clinician-scientists to facilitate translation of basic research to human medicine. NIH supports many innovative training programs and funding mechanisms that foster scientific creativity and exploration. Additional funding is needed if we are to strengthen our nation's research capacity, ensure a biomedical research workforce that reflects the racial and gender diversity of our citizenry, and inspire a passion for science in current and future generations of researchers.

NIH is Critical to U.S. Competitiveness

Our country still has the most robust medical research capacity in the world, but that capacity simply cannot weather repeated blows such as persistent below-inflation funding levels and sequestration cuts, which jeopardize our competitive edge in an increasingly innovation-based global marketplace.

Other countries have recognized the critical role that biomedical science plays in innovation and economic growth and have significantly increased their investment in biomedical science. This shift in funding is creating an innovation deficit in the U.S. and raises the concern that talented medical researchers from all over the world, who once flocked to the U.S. for training and stayed to contribute to our innovation-driven economy, are now returning to better opportunities in their home countries. We cannot afford to lose that intellectual capacity, much less the jobs and industries fueled by medical research. The U.S. has been the global leader in medical research because of Congress's bipartisan recognition of NIH's critical role. To maintain our dominance, we must reaffirm this commitment to provide NIH the funds needed to maintain our competitive edge.

NIH: An Answer to Challenging Times

The research supported by NIH drives not only medical progress but also local and national economic activity, creating skilled, high-paying jobs and fostering new products and industries. According to a report released by United for Medical Research, a coalition of scientific advocates, institutions and industries, in fiscal year 2011, NIH-funded research supported an estimated 432,000 jobs all across the United States and generated more than $62 billion in new economic activity.

The Ad Hoc Group's members recognize the tremendous challenges facing our nation's economy and acknowledge the difficult decisions that must be made to restore our country's fiscal health. Nevertheless, we believe strongly that NIH is an essential part of the solution to the nation's economic restoration. Strengthening our commitment to medical research, through robust funding of the NIH, is a critical element in ensuring the health and well-being of the American people and our economy.

Therefore, the Ad Hoc Group for Medical Research recommends that NIH receive at least $32 billion in FY 2016 as the next step toward a multi-year increase in our nation's investment in medical research.