PREPARED STATEMENT OF CHRISTOPHER P. AUSTIN, M.D.
DIRECTOR, NATIONAL CENTER FOR ADVANCING TRANSLATIONAL SCIENCES

Mr. Chairman and Members of the Subcommittee: I am pleased to present the President's fiscal year (FY) 2016 budget request for the National Center for Advancing Translational Sciences (NCATS) of the National Institutes of Health (NIH). The FY 2016 budget request for NCATS is $660,131,000, which is $27,421,000 more than the FY 2015 level. The request includes $25,835,000 for the Cures Acceleration Network.

Recent and rapid discoveries of mechanisms, the human genome, and advances in technology have led to greater scientific opportunities that have the potential to substantially improve human health. NCATS is working to improve the process for transforming these discoveries into cures so that we can bring more treatments to more patients more quickly.

NCATS defines *translation* as the process of turning observations in the laboratory and clinic into interventions that improve the health of individuals and the public – from diagnostics and therapeutics to medical procedures and behavioral changes. *Translational science* is the field of investigation focused on understanding the scientific and operational principles underlying each step of the translational process. NCATS studies translation on a system-wide level. NCATS' translational science efforts focus on the entire spectrum of translational research – basic research, pre-clinical research, clinical research, medical practice, and public health. At all stages of the spectrum, NCATS develops new approaches, demonstrates their usefulness, and disseminates the findings. Patient involvement is a critical feature of all stages in translation.

INNOVATION IN METHODS AND TOOLS

The translational science approach addresses common problems in order to make translation more efficient and effective. NCATS looks for innovative, catalytic strategies, not only to address longstanding problems, but also to accelerate the process of developing solutions. One technological innovation is a bioengineered system that represents human organs, more commonly known as a tissue chip. Through the NCATS Tissue Chip for Drug Screening program, a collaborative effort with the Defense Advanced Research Projects Agency (DARPA) and the Food and Drug Administration, tissue chips are being developed with the intent to test drug safety and effectiveness at a reduced risk and more cost-effectively than current methods. NCATS is building on its initial success in developing chips with single tissues or organs by funding projects to integrate several of the organ-specific chips into a human system. Once completed,

these integrated systems can be used to predict whether a drug, vaccine or biologic agent is toxic to, or effective in, humans.

NCATS shares its unique research approaches and resources, and demonstrates that they can be broadly applied to translational science efforts. In a recent collaboration with the National Institute of Neurological Disorders and Stroke, NCATS scientists incorporated a new approach to find a compound that could enhance the activity of the parkin protein, which is implicated in Parkinson's disease. Parkin is suspected of playing an important role in the removal of faulty mitochondria (a cell's "powerhouse") in neurons, but for patients with Parkinson's disease, this maintenance mechanism is disrupted. NCATS researchers designed a test (called an assay) to measure the activity of the gene for parkin. With this assay, the research team is now conducting high-throughput screens using the NCATS' chemical libraries to identify compounds that increase parkin activity. While specifically designed to address this problem, the screening and assay methods designed by NCATS researchers can be used by other scientists to solve many other translational research problems.

COLLABORATION AND PATIENT ENGAGEMENT

NCATS also applies innovative approaches to the study of rare diseases by fostering collaboration and patient engagement. NCATS' Rare Disease Clinical Research Network (RDCRN) is a highly collaborative network of 22 clinical research consortia and a data management center. The network is composed of approximately 2,600 researchers, including NIH scientific program staff, academic investigators, and members of 98 patient advocacy groups. Scientists from multiple disciplines at hundreds of clinical sites around the world work together with patient advocacy groups to study more than 200 rare diseases. Since its launch, nearly 29,000 patients have been enrolled in network clinical studies. Ninety-one studies are under way.

The NCATS Therapeutics for Rare and Neglected Diseases (TRND) program also establishes robust collaborations among NIH, academic scientists, nonprofit organizations, and pharmaceutical and biotechnology companies to support faster translation of drug discovery and development. When successful, these projects can be acquired by research partners for further development toward treatments for patients.

A NATIONAL NETWORK FOR CLINICAL AND TRANSLATIONAL RESEARCH

The NCATS Clinical and Translational Science Awards (CTSA) program focuses its efforts on addressing the inefficiencies and roadblocks in clinical and translational research, from scientific discovery to improved patient care. The 62 CTSA sites serve as research hubs to support a national network for clinical and translational studies. The hubs support collaborations in education and training initiatives, share best

practices and methods, promote team science, and conduct multi-site clinical studies through a shared infrastructure. A good example of such collaboration happened in the wake of the April 2013 Boston Marathon bombing. Doctors from several local hospitals quickly formed a team to design a high-quality multi-site study to examine blast-related ear injuries. Harvard's CTSA hub had an Institutional Review Board (IRB) reliance agreement in place that enabled these institutions to rely on a single committee to review, approve, and monitor the study. Therefore, a study involving seven sites was launched within weeks rather than the more typical months. This ability to streamline the review of multi-site clinical research studies enables NIH-funded research to generate results more quickly without compromising the protection of human participants. NCATS has announced plans to support the evolution of the CTSA program by soliciting innovative approaches to increasing clinical trial efficiency and effectiveness, addressing the roadblocks common to clinical studies recruitment of research study participants, and supporting collaborative innovative research in both translational science and its methods.

CONCLUSION

NCATS' mission is to catalyze the generation of innovative methods and technologies that will enhance the development, testing and implementation of diagnostics and therapeutics across a wide range of human diseases and conditions. NCATS looks forward to building on its recent successes to bring more treatments to more patients more quickly.

Christopher P. Austin, M.D.
Director, National Center for Advancing Translational Sciences

Christopher P. Austin, M.D., is director of the National Center for Advancing Translational Sciences (NCATS) of the National Institutes of Health (NIH). Dr. Austin leads NCATS's work to improve the translation of observations in the laboratory, clinic and community into interventions that reach and benefit patients – from diagnostics and therapeutics to medical procedures and behavioral changes. Under his direction, NCATS researchers and collaborators are developing new technologies, resources and collaborative research models; demonstrating their usefulness; and disseminating the data, analysis and methodologies for use by the worldwide research community.

Dr. Austin's career has spanned the spectrum of translational research in the public and private sectors. He joined NIH in 2002 as the senior advisor to the director for translational research at the National Human Genome Research Institute (NHGRI), where he was responsible for conceptualizing and implementing research programs to derive scientific insights and therapeutic benefits from the results of the newly completed Human Genome Project. While at NHGRI, Dr. Austin founded and directed the NIH Chemical Genomics Center (now the NCATS Chemical Genomics Center), Therapeutics for Rare and Neglected Diseases program, Toxicology in the 21st Century initiative, and NIH Center for Translational Therapeutics. When NCATS launched in late 2011, Dr. Austin became the inaugural director of the Center's Division of Pre-Clinical Innovation, and then was appointed as NCATS Director in 2012. Before joining NIH, Dr. Austin worked at the pharmaceutical company Merck, where he directed programs on genome-based discovery of novel targets and drugs, with a particular focus on treatments for schizophrenia and Alzheimer's disease.

Dr. Austin is trained as a clinician and geneticist. He earned an M.D. from Harvard Medical School and an A.B. *summa cum laude* in biology from Princeton University. He completed a research fellowship in developmental neurogenetics at Harvard, studying genetic and environmental influences on stem cell fate determination. Dr. Austin also trained in internal medicine and neurology at the Massachusetts General Hospital in Boston, after which he practiced medicine in academic and community hospitals, providing primary care in urban settings and in rural Alaska and Africa.