The Honorable Patty Murray
Chair
Committee on Appropriations
United States Senate

The Honorable Susan Collins
Vice Chair
Committee on Appropriations
United States Senate

The Honorable Tammy Baldwin
Chair
Committee on Appropriations
Subcommittee on Labor, Health and Human Services, Education, and Related Agencies
United States Senate

The Honorable Shelly Moore Capito
Ranking Member
Committee on Appropriations
Subcommittee on Labor, Health and Human Services, Education, and Related Agencies
United States Senate

The Honorable Tom Cole
Chair
Committee on Appropriations
U.S. House of Representatives

The Honorable Rosa DeLauro
Ranking Member
Committee on Appropriations
U.S. House of Representatives

The Honorable Robert Aderholt
Chair
Subcommittee on Labor, Health and Human Services, Education, and Related Agencies
U.S. House of Representatives

April 17, 2024

Dear Senators Murray, Collins, Baldwin, and Capito and Representatives Cole, DeLauro, and Aderholt:

The NCATS Alliance (the Alliance) recommends Congress allocate at least $51.303 billion for the National Institutes of Health (NIH) and no less than $1.194 billion for the National Center for Advancing Translational Sciences (NCATS) as part of the Fiscal Year 2025 Labor, Health and Human Services and Education Appropriations legislation. Founded in 2023, the NCATS Alliance is a coalition of patient advocates, clinicians, academic medical centers, researchers, and biotechnology companies with a shared vision of educating the public and policymakers on the important science being advanced by NCATS and ensuring Congress provides NCATS with the necessary funding to deliver on its scientific mission.

In 2011, Congress established NCATS to accelerate the development of new diagnostics, therapeutics, and cures for common and rare diseases using translational and clinical science. NCATS is the only NIH center or institute charged by Congress with a singular mission to advance translational science to accelerate treatments and cures for ALL diseases, rare and common, and regardless of which organs or tissues are affected. Increased federal funding will enable NCATS to make rapid advances in the development of new clinical and translational science and technologies to speed the development of safe, effective treatments for diseases that affect tens of millions of Americans and cost the American economy billions of dollars each year. In pursuit of this mandate, the NCATS Alliance urges your support of increased funding for NCATS in the following high impact areas of research.

- **$50 Million Increase For Gene Therapy And Gene Editing Research.** Scientists are making considerable progress in the transformational field of gene therapy and gene editing, yet there remain considerable challenges in developing and bringing to market effective therapies using these extremely promising genetic approaches. The Alliance continues to support the NCATS Platform Vector-Gene Therapy (PaVe-GT) pilot program which aims to develop technologies to simplify and reduce the cost of developing gene therapies for multiple rare diseases simultaneously. NCATS also continues to lead an effort with the Food and Drug Administration -- a public-private partnership known as the Bespoke Gene Therapy Consortium, to develop platforms and standards that will speed the development and delivery of customized, or "bespoke," gene therapies to treat multiple rare diseases that have no FDA approved treatment.

- **$50 Million Increase For Pre-Clinical Translational Science.** NCATS is developing humanized preclinical tools, Tissue Chips and 3D Bio-Printing models for example, designed to replicate much more closely human tissues and organs and better assess the impact a therapeutic agent will have on them. These exciting technologies hold the potential to increase the success rate of clinical trials and reduce the time it takes to complete them by delivering data that is far more predictive of positive results in human trials. NCATS runs programs like the Therapeutics for Rare and Neglected Diseases (TRND), in collaboration with industry and other stakeholders, that helped accelerate regulatory approval of a gene therapy for the rare pediatric condition aromatic L-amino acid decarboxylase (AADC) deficiency and a modified glucocorticoid with a novel mechanism of action for Duchenne muscular dystrophy. NCATS is helping move promising preclinical candidates into clinical trials, enabling the submission of 55 investigational new drug (IND) applications for diverse conditions, shepherding the projects through the so-called "valley of death."

- **$30 Million Increase For Rare Disease Research**. Nearly 10% of people in the U.S. have a rare disease, approximately 30 million people. Medical care costs total approximately $400 billion, which is on par with common diseases like cancer, heart failure and Alzheimer's disease. For individuals living with a rare disease, it can take almost 5 years to receive a diagnosis after seeing more than 7 medical specialists. NCATS is the leader

of rare disease research within the NIH. The NCATS Division of Rare Diseases Research Innovation facilitates and coordinates NIH-wide rare disease programs and oversees the NIH Genetic and Rare Diseases (GARD) Information Center, the nation-wide Rare Diseases Clinical Research Network (RDCRN), and the Rare Diseases Registry Program. NCATS is advancing multiple scientific initiatives to develop new diagnostics to reduce the time it takes to diagnose a rare disease and develop technologies to accelerate the approval of new treatments.

- **$50 Million Increase For The National Clinical Cohort Collaborative (N3C).** N3C is an open-science, privacy-preserved data-sharing platform designed to accelerate biomedical research and discovery. It combines electronic health-record data with imaging, mortality, viral genome sequences, and Medicare and Medicaid data from the Centers for Medicare and Medicaid Services (CMS) to answer key research questions on a variety of diseases. NCATS provides a reusable, real world data research infrastructure that allows scientists to focus on advancing science instead of spending valuable time and costs on startup activities. Continued investment in a common, reusable infrastructure for translational science for the assembly, harmonization, usability, and analysis of multi-modal clinical data is fundamental to accelerating the pace of discoveries, particularly for rare disease research.

- **$50 Million Increase For A Specialized Platform for Innovative Research (ASPIRE)**. ASPIRE represents a pivotal cross-sector initiative uniting academic researchers, government agencies, small businesses, and industry powerhouses to pioneer an innovative robotics and digital platform that will transform chemistry into an information-based science and significantly enhance our ability to discover and develop new drugs for currently undrugged targets. ASPIRE aims to serve as a blueprint to revolutionize drug discovery and development processes through laboratory automation by integrating state-of-the-art Artificial Intelligence (AI), cutting-edge life sciences technologies, and advanced robotics that will advance U.S. large-scale manufacturing needs through innovation, faster design, and production of therapeutics. As a national asset, ASPIRE's framework for broad collaboration will provide a model lab that others can utilize, further enhance, and innovate upon. This investment will fast-track the creation of new therapies by enabling more widely distributed discovery as well as manufacturing of therapeutics. ASPIRE will also link physically separated laboratories to act in concert and quickly respond to large-scale manufacturing needs for urgently needed therapeutics, including countermeasures, and the tools and technologies developed and disseminated through ASPIRE initiative will help reduce U.S. dependence on pharmaceuticals sourced from foreign countries. ASPIRE will elevate the speed and caliber of drug research breakthroughs for the betterment of all.

- **$36 Million Increase For Clinical and Translational Science Awards.** Under NCATS' leadership, the Clinical and Translational Science Awards (CTSA) Program supports a national network of medical institutions that speeds the translation of research discoveries into improved care. Currently, more than 60 leading medical institutions

across the nation receive CTSA Program funding. The institutions offer expertise, resources, and partnerships at the national and local levels to improve the health of individuals and communities. The CTSA Program also nurtures the field of translational science through education, training, and career support at all levels.

Conclusion

Congress recognized the need for improved translational science when it created NCATS in 2011. Despite being seriously under-resourced since its inception, NCATS has made tremendous strides in advancing the translational science and technologies needed to move all biomedical research and development much faster toward effective treatments for rare and common diseases. The NCATS Alliance urges Congress to provide NCATS with the funding it needs to accomplish even more and achieve its goal of getting more treatments to all people more quickly.

Sincerely,

NCATS Alliance