



The Honorable Susan Collins  
Chair  
Committee on Appropriations  
United States Senate

The Honorable Patty Murray  
Vice Chair  
Committee on Appropriations  
United States Senate

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

The Honorable Tammy Baldwin  
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 16, 2026

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

The NCATS Alliance (the Alliance) recommends Congress allocate no less than \$51.303 billion for the National Institutes of Health (NIH) and no less than \$1.228 billion for the National Center for Advancing Translational Sciences (NCATS) as part of the Fiscal Year 2027 Labor, Health and Human Services and Education (L-HHS) Appropriations legislation. The Alliance represents patients living with rare diseases, clinicians, academic medical centers, researchers, and biotechnology companies committed to getting more novel diagnostics, treatments, and cures to all Americans more quickly by ensuring NCATS has the resources and policies in place to accelerate translational science. We applaud the bipartisan work of the committees for passing

the bipartisan Fiscal Year 2026 L-HHS bill that included a \$415 million increase for NIH and \$14 million increase for NCATS.

Established by Congress in 2011, NCATS is the only NIH institute with a singular mission to advance translational science and accelerate treatments and cures for all diseases — rare and common. Wise federal investment will enable NCATS to continue developing clinical and translational technologies that benefit tens of millions of Americans and save the economy billions of dollars annually. The Alliance urges support for increased funding in the following areas:

**Gene Therapy Research (+\$50M)** Scientists are making considerable progress in gene therapy, though significant challenges remain in bringing effective therapies to market. The PaVe-GT program is now publicly sharing regulatory packages and IND application materials to accelerate development of other rare disease gene therapies — with peer-reviewed findings published in 2025 and FDA designations granted for an investigational therapy for propionic acidemia. NCATS is a critical partner with the FDA and FNIH on the Bespoke Gene Therapy Consortium (BGTC), a public-private partnership to develop platforms and standards that speed the delivery of customized gene therapies for rare diseases with no approved treatments. Finally, NCATS helps lead the Somatic Cell Gene Editing Program (SCGEP), an initiative that heavily contributed to the successful dosing of Baby KJ with a life-changing n-of-1 gene therapy, helping to write the playbook within the recently published FDA draft Plausible Mechanism Framework. The expanded funding will spur the development and research of more gene editing platforms, standardization, and integration into the PaVe-GT, BGTC, SCGEP, etc.

**Human-Based Preclinical Translational Science (+\$50M)** NCATS funds humanized preclinical tools — including tissue chips and 3D bioprinting models — that replicate human tissues and organs. In 2025, NCATS launched Tissue Chips in Space 2.0, selecting six research teams to study age-related diseases like Alzheimer's and cardiac dysfunction using the microgravity environment of the International Space Station. NCATS programs have also supported fifty-six investigational new drug applications and helped accelerate regulatory approval of therapies for rare conditions including Duchenne muscular dystrophy. This research is helping NIH move away from expensive and difficult animal models to human relevant systems that contain only human cells. The proposed increase in funding will spur further creation and integration of rare disease organoid models, 3D printed tissues, and tissue chips research.

**Rare Disease Research (+\$30M)** Ten percent of Americans — roughly 30 million people — live with a rare disease, costing the healthcare system approximately \$400 billion annually. On average, patients wait over six years for a correct diagnosis after seeing more than seven specialists. NCATS leads rare disease research within NIH through the Rare Diseases Clinical Research Network (RDCRN), which now supports 21 research consortia studying over 280 diseases at 273 clinical sites, alongside the GARD Information Center and the Rare Diseases Registry Program. NCATS' drug repurposing initiative is also accelerating the development of

new treatments by leveraging automation and advanced screening technologies to rapidly identify existing approved drugs that may be effective against rare and common diseases — dramatically shortening the time and cost it takes to bring new therapeutic options to patients who have long waited for answers. This funding will spur the linkage of the RDCRN to the National Clinical Cohort Collaborative (N3C), centralize rare trial data from the RDCRN using similar security and governance controls as the N3C, and improve GARD with AI driven user exploration and integration.

**National Clinical Cohort Collaborative — N3C (+\$70M)** N3C is an open-science, privacy-preserving data platform that combines electronic health records, imaging, genomic, and Medicare/Medicaid data from nearly 100 clinical centers representing over 23 million patients. In January 2026, NCATS held a community forum to outline new capabilities and governance processes, reflecting N3C's maturation into a sustained national research infrastructure. N3C now supports research across any disease domain, inclusive of rare diseases. Continued investment ensures researchers get access to critical linked data sets (environmental exposures, insurance claims, etc.), improved governance and security controls, and state-of-the-art AI powered data quality pipelines. This investment enables researchers to focus on science rather than time-consuming legal agreements and data startup activities, while improving research reproducibility and replicability by having national visibility and validation into data cleaning, harmonization, and standardization pipelines.

**Clinical and Translational Science Awards — CTSA (+\$30.5M)** The CTSA Program supports a national network of more than 60 leading medical research institutions across 30 states, speeding the translation of discoveries into improved patient care. Current initiatives include AI-powered research models, reproducibility science, and wearable technology — including a 2025 open-source smartwatch algorithm for more accurately tracking caloric expenditure in people with obesity. SMART IRB 3.0, launched in 2025, is now in use by nearly 1,400 institutions including the Department of Defense and VA. Additional funding for the CTSA's will extend engagement with rural and community systems to further improve the health of all Americans.

**Indirect Cost Protections** NIH funding is foundational to the U.S. research enterprise. The research NCATS funds extramurally is often incredibly complex due to the serious and rare, often ultra-rare, nature of these diseases. The Alliance supports the extension of the current prohibition on changes to NIH Facilities & Administrative cost reimbursement policy in the FY27 appropriations bill.

Congress recognized the need for improved translational science when it created NCATS in 2011. Despite being grossly 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. At a moment of unprecedented pressure on NIH and the broader federal research enterprise, the work of NCATS has never been more critical to patients, the U.S. leadership in

NCATS Alliance

April 16, 2026

Page 4

life sciences, and American competitiveness in commercial biotechnology innovation. The NCATS Alliance urges Congress to provide NCATS with the funding it needs to accomplish this critical mission.

Sincerely,

Alliance for Regenerative Medicine  
American Society of Gene and Cell Therapy  
Axle Informatics

Consortium of Rural States (CORES)

- Dartmouth SYNERGY Clinical and Translational Science Institute
- Penn State Clinical and Translational Science Institute
- South Carolina Clinical & Translational Research Institute
- University of Arkansas for Medical Sciences Translational Research Institute
- University of Iowa Institute for Clinical and Translational Science
- Frontiers Clinical and Translational Science Institute at the University of Kansas
- University of Kentucky
- University of New Mexico & University of Arizona Southwest Center for Advancing Clinical and Translational Innovation
- Utah Clinical and Translational Science Institute

Datavant

EveryLife Foundation for Rare Diseases

Friedreich's Ataxia Research Alliance

FSHD Society

Global Genes

Milken Institute FasterCures

Muscular Dystrophy Association

Myotonic Dystrophy Foundation

Neuroimmune Foundation

National Organization for Rare Disorders

Physicians Committee for Responsible Medicine

PTC Therapeutics

Regenstrief Institute