November 10, 2021

The Honorable Patty Murray
Chair
Senate Labor, Health and Human Services, Education, and Related Agencies Appropriations Subcommittee
S-128, The Capitol
Washington, D.C. 20510

The Honorable Roy Blunt
Ranking Member
Senate Labor, Health and Human Services, Education, and Related Agencies Appropriations Subcommittee
S-128, The Capitol
Washington, D.C. 20510

The Honorable Rosa DeLauro
Chair
House Labor, Health and Human Services, Education, and Related Agencies
H-307, The Capitol
Washington, D.C. 20515

The Honorable Tom Cole
Ranking Member
House Labor, Health and Human Services, Education, and Related Agencies
H-307, The Capitol
Washington, D.C. 20515

Dear Chair Murray, Chair DeLauro, Ranking Member Blunt, and Ranking Member Cole:

We, the undersigned organizations, representing tens of millions of Americans with rare diseases, thank you for your long-held commitment to the rare disease patient population. As you know, there are an estimated 7,000 rare diseases, which affect between 25 and 30 million Americans. Unfortunately, around 90% of rare diseases lack an FDA-approved treatment, and patients need your support in funding scientific breakthroughs that can lead to treatments for their diseases. Therefore, we write today to urge you to adopt the House-passed funding level for the National Center for Advancing Translational Sciences (NCATS) at the National Institutes of Health (NIH) for Fiscal Year 2022 (FY22) as outlined in H.R. 4502.

H.R. 4502 provides an increase of more than $42 million for the important work NCATS is doing, from bench to bedside, to advance solutions to the most challenging aspects of therapy development in order to deliver new treatments and cures to patients across the full spectrum of diseases. Furthermore, we ask that you ensure that a majority of the $13 million that is currently not directed in H.R. 4502 to the Center's rare disease programs. These rare disease programs currently lack the funding needed to bring about the kind of transformative advancements called for in NCATS' mission.

NCATS Background and Budget

NCATS was established in 2011 and has three major components: Reengineering Translational Science (RTS) activities, the Cures Acceleration Network (CAN), and the Clinical and Translational Science Awards (CTSA) program. NCATS is critical to the rare disease

community as the technologies being developed and the programs conducted at NCATS have enormous potential to revolutionize medicine for many rare and common diseases. Over the past decade, NCATS has supported projects that led to 44 successful Investigational New Drug (IND) applications, for both rare and common diseases, at the Food and Drug Administration (FDA).

NCATS funding is primarily directed spending. Congress has traditionally appropriated a bulk of NCATS appropriations to the CTSA program, which NCATS administers to fund projects at a consortium of American medical research centers. The two NCATS programs containing its rare disease efforts – the RTS program and CAN – receive far less funding.[1] In the past few budget cycles, of the total of NCATS appropriations, the RTS programs have received approximately 23%, CAN has been capped at approximately 7%, and CTSA received approximately 70%. Historically, appropriations increases for NCATS have been proportional to the overall funding percentage of NCATS programs themselves, perpetuating the cycle of limited funding for rare disease programs.

In FY21, the rare disease programs under RTS and CAN received approximately $40 million of NCATS' overall funding, representing less than 5% of NCATS total appropriation. Therefore, we believe that a majority of any non-directed increase in funding for NCATS in FY22 should be targeted towards programs that impact the rare disease community.

Reengineering Translational Science

Many of the rare disease programs within NCATS' RTS component are housed in the Office of Rare Disease Research (ORDR)[2] and the Therapeutics for Rare and Neglected Diseases (TRND) program.[3] TRND's goal is to close the gap that exists between basic research discoveries and the testing of new drugs in humans. The TRND program supports the preclinical development of therapeutic candidates intended to treat rare or neglected disorders, with the goal of enabling an Investigational New Drug (IND) application.[4]

ORDR oversees a majority of the rare disease-related activities at NCATS, including the Genetic and Rare Diseases Information Center (GARD), a central repository of information on rare diseases for patients and practitioners.[5] Additionally, ORDR is responsible for the Rare Diseases Clinical Research Network (RDCRN), which supports clinical studies for research on rare diseases.[6] ORDR also works collaboratively across all NCATS rare disease programs and administers grants and awards, including clinical trial readiness grants for rare diseases. Finally, ORDR also oversees a program called the Multidisciplinary Approaches to Shortening the Diagnostic Odyssey, which aims to reduce the time between when a patient starts to experience

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[1] Report. H.R. 4502, 117th Congress (2021). https://www.congress.gov/117/crpt/hrpt96/CRPT-117hrpt96.pdf
[2] NCATS. Divisions & Offices. NIH. Accessed 9/1/21 https://ncats.nih.gov/about/center/org
[3] NCATS. Therapeutics for Rare and Neglected Diseases (TRND). NIH. Accessed 9/1/21. https://ncats.nih.gov/trnd
[4] Ibid.
[5] NCATS. Genetic and Rare Diseases Information Center (GARD). NIH. Accessed 9/1/21. https://ncats.nih.gov/gard
[6] NCATS. Rare Diseases Clinical Research Network (RDCRN). NIH. Accessed 9/1/21. https://ncats.nih.gov/rdcrn

symptoms and when they obtain a correct diagnosis, which currently averages over five years. This odyssey is a tremendous burden on rare disease patients and often results in adverse health consequences and prolonged delays to accessing appropriate care.

These programs are vital for rare disease patients, but they are not reaching their full potential. Currently, only about $30 million of the overall NCATS budget funds rare disease projects under RTS. Yet, NCATS has the potential to help address thousands of rare diseases by developing treatments, diagnostics, and improving the therapy development processes. Therefore, ensuring rare disease programs under RTS are adequately funded will have a tremendously beneficial impact on rare disease patients.

We request that RTS programs relating to rare diseases receive a majority of the $13 million in non-directed funds from H.R. 4502 for NCATS in FY22.

Cures Acceleration Network

The CAN program was established to bring cures to high-need patient populations by utilizing additional authorities helpful in assembling multi-stakeholder, public-private coalitions to advance high-value programs much faster and more effectively so as to deliver more treatments to more patients more quickly.[7] The projects are chosen with input from the CAN Review Board, a group of academic, public, and private sector representatives who report directly to the NCATS Director and are charged with "identifying significant barriers to successful translation of basic science into clinical application."[8]

One of the most promising projects within CAN, for both rare and common genetic diseases, is the Platform Vector Gene Therapy (PaVe-GT) project, which is currently studying the impact of using the same viral vector, or delivery mechanism, with different therapeutic agents inside for four rare genetic diseases.[9] This so-called "plug-and-play" model has the potential to treat thousands of known rare and common genetic disorders. PaVe-GT is just one example of how technology and research originating from NCATS rare disease projects could have a tremendous impact on broader patient populations.

We support the $60 million for CAN consistent with the House-passed H.R. 4502.

Clinical and Translational Science Awards Program

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[7] NCATS. Cures Acceleration Network (CAN). NIH. Accessed 9/1/21. https://ncats.nih.gov/funding/review/can
[8] NCATS. Cures Acceleration Network (CAN) Review Board. NIH. Accessed 9/1/21. https://ncats.nih.gov/advisory/canboard
[9] NCATS. Platform Vector Gene Therapy (PaVe-GT) Pilot Project. NIH. Accessed 9/1/21. https://ncats.nih.gov/expertise/pave-gt-pilot-project

The CTSA program is a consortium of more than 50 medical research centers across the country that are awarded grants from NCATS to fund translational science activities.[10] These centers aim to improve the translational process of turning observations made in the laboratory, clinic, and community into diagnostics and treatments that improve the health of individuals and the public.[11]

NCATS also works to leverage the power of the CTSA network by conducting several innovative programs to tackle challenges that exist across the research and development spectrum.[12] The Streamlined, Multisite, Accelerated Resources for Trials (SMART) Institutional Review Board (IRB) aims to reduce logistical burdens by establishing a centralized IRB.[13] Additionally, the Common Metrics Initiative is working to develop uniform standards for enhanced data sharing. Finally, the Trial Innovation Network (TIN) is developing methods for better, more efficient clinical trials,[14] a key issue for patients with rare diseases who are often in small clinical trials.

We support the $616 million in funding for CTSA consistent with what was allocated by the House in H.R. 4502.

While the work of all three components of NCATS is vital, we believe that rare disease patients would benefit most if a majority of the non-directed funding is allocated to programs with rare disease applications.

Therefore, we urge you to adopt the House-passed FY22 funding level for NCATS reflected in H.R. 4502 and ensure that a majority of any currently non-directed spending be allocated to rare disease programs at NCATS.

We thank you for your attention to our request and look forward to working with you to ensure that NCATS has the resources it needs in order to serve the rare disease community. If you have any questions, please do not hesitate to contact Rick White at rwhite@rarediseases.org.

Sincerely,

National Organization for Rare Disorders
Muscular Dystrophy Association
Friedreich's Ataxia Research Alliance

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[10] NCATS. About the CTSA Program. NIH. Accessed 9/1/21. https://ncats.nih.gov/ctsa/about
[11] NCATS. Translational Science Spectrum. NIH. Accessed 9/1/21. https://ncats.nih.gov/translation/spectrum
[12] NCATS. CTSA Program Projects & Initiatives. NIH. Accessed 9/1/21. https://ncats.nih.gov/ctsa/projects
[13] NCATS. NCATS SMART IRB Platform. NIH. Accessed 9/1/21. https://ncats.nih.gov/ctsa/projects/smartirb
[14] NCATS. CTSA Program Projects & Initiatives. NIH. Accessed 9/1/21. https://ncats.nih.gov/ctsa/projects