

September 15, 2021

The Honorable Patty Murray
Chair
Senate Labor, Health and Human Services,
Education, and Related Agencies
Appropriations Subcommittee
Room 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
Room S-128, The Capitol
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Chair Murray and Ranking Member Blunt,

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 to 30 million Americans. We write today to ask for your full support in appropriating increased funding for the National Center for Advancing Translational Sciences (NCATS) at the National Institutes of Health (NIH) in Fiscal Year 2022 (FY22). We request that the Senate provide \$955 million for NCATS in FY22, which represents an increase of approximately 12% from the FY 2021 appropriation of \$855 million. Furthermore, we ask the Committee to explicitly direct a significant portion of any funding increase above the FY21 funding level to rare disease programs at NCATS.

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 administering 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 Investigational New Drug (IND) approvals, for both rare and common diseases, at the Food and Drug Administration (FDA).

While most rare disease programs at NCATS fall under RTS, CAN also focuses on rare diseases, but to a lesser extent. NCATS funding is largely directed spending, with Congress appropriating specific dollar amounts to the CTSA and CAN programs.¹ In the past few budget cycles, the RTS programs have received approximately 23% of NCATS funding, CAN approximately 7%, and CTSA approximately 70% of NCATS appropriations.

¹ Report. H.R. 4502, 117th Congress (2021). <https://www.congress.gov/117/crpt/hrpt96/CRPT-117hrpt96.pdf>

The rare disease programs that are considered part of RTS and CAN received approximately \$40 million of NCATS overall funding, which represents less than 5% of NCATS total appropriation in FY21. Appropriations increases for NCATS have historically been proportional to the overall funding percentage of NCATS programs themselves, which has perpetuated the cycle of limited funding for rare disease programs. Therefore, we believe that a significant portion of any overall FY22 proposed increase for NCATS should be targeted towards programs that have impacts on 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 a basic research discovery and 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 houses 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, some of which include clinical trial readiness grants. 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 symptoms and 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 the 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 RTS rare disease projects. NCATS has the ability to help address thousands of rare diseases by developing treatments, diagnostics, and improving drug development processes. Ensuring RTS is adequately funded will have a tremendous impact on rare disease patients.

We request that RTS programs relating to rare diseases receive a substantial portion of any increase in appropriated funds for NCATS in FY22.

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>

Cures Acceleration Network

The CAN program was established to bring cures to high need patient populations by minimizing hurdles between research and clinical trials by utilizing Defense Advanced Research Projects Agency-like authorities.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

For rare and common genetic diseases, one of the most promising projects within CAN 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 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 request the Senate appropriate up to \$60 million in funding for CAN consistent with what was allocated in the House-passed FY22 Labor-HHS appropriations bill.

Clinical and Translational Science Awards Program

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 are aimed at improving 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 The Common Metrics Initiative is working to develop uniform standards for enhanced data sharing.

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>

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>

Finally, the Trial Innovation Network (TIN) is developing methods for better, more efficient clinical trials, a key issue for patients with rare diseases in small clinical trials.¹⁴

We request the Senate appropriate \$616 million in funding for CTSA consistent with what was allocated in the House-passed FY22 Labor-HHS appropriations bill.

While the work of all three components of NCATS is vital, we believe that a significant portion of any proposed increase would be most effective if allocated to programs with rare disease applications under CAN and RTS.

We urge the Senate to provide \$955 million in funding for NCATS, with a significant portion of any increase dedicated 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

CC: Members of the Senate Labor, Health and Human Services, Education, and Related Agencies Appropriations Subcommittee

¹⁴ NCATS. *CTSA Program Projects & Initiatives*. NIH. Accessed 9/1/21. <https://ncats.nih.gov/ctsa/projects>