December 1, 2022

The Honorable Charles Schumer
Majority Leader
United States Senate
Room S-221, the Capitol
Washington, DC 20510

The Honorable Nancy Pelosi
Speaker of the House
United States House of Representatives
H-232, the Capitol
Washington, DC 20515

The Honorable Mitch McConnell
Minority Leader
United States Senate
Room S-230
Washington, DC 20510

The Honorable Kevin McCarthy
Minority Leader
United States House of Representatives
H-204, the Capitol
Washington, DC 20515

The Honorable Patrick Leahy
Chair
Committee on Appropriations
United States Senate
Room S-128, the Capitol
Washington, DC 20510

The Honorable Rosa DeLauro
Chairwoman
Committee on Appropriations
U.S. House of Representatives
H-307, the Capitol
Washington, DC 20515

The Honorable Richard Shelby
Vice Chairman
Committee on Appropriations
United States Senate
S-146A, the Capitol
Washington, DC 20510

The Honorable Kay Granger
Ranking Member
Committee on Appropriations
U.S. House of Representatives
1036 Longworth HOB
Washington, DC 20515

Leader Schumer, Speaker Pelosi, Leader McConnell, Leader McCarthy, Chairman Leahy, Vice Chairman Shelby, Chairwoman DeLauro, and Ranking Member Granger:

We are writing to express our support for increased appropriations in Fiscal Year 2023 (FY 23) for the National Center for Advancing Translational Sciences (NCATS) to ensure that NCATS has the resources required to accomplish the important mission assigned to it by Congress and the National Institutes of Health (NIH). We are encouraged by the approximately 3% proposed increase for NCATS' Clinical and Translational Science Awards (CTSA) Program which has served an important part of the COVID-19 pandemic response effort and has supported health equity and innovative care in rural communities. As you know, the non-CTSA programs such as the Rare Disease Clinical Research Network (RDCRN) and the National COVID Cohort Collaborative (N3C program)[1] are also invaluable assets to biomedical

[1] *See* H.R. Rep. No. 117-403, at 134 (2022) ("The Committee encourages NCATS to consider expanding the platform to accelerate research and cures for other high-priority diseases such as cancer and other rare diseases."); *see also* EXPLANATORY STATEMENT FOR DEP'TS OF LABOR, HEALTH AND HUMAN SERVS., AND EDUC., AND RELATED AGENCIES APPROPRIATIONS BILL, 2023, 117th CONG., at 136 (appropriating $90 million to the Cures Acceleration Network. A $30 million increase from FY 22).

innovation and public health. For these reasons, we are concerned that the proposed funding for the non-CTSA portion of NCATS' budget – which falls below the average 3% across NIH's other institutes and centers – will slow progress for urgent public health research priorities.

Beyond the CTSA program, NCATS' programs play a critical role in advancing medical science and our nation's health. For example, the N3C program, which contains the largest repository of COVID-19 data in the U.S., has enabled more than 3,500 researchers from over 300 institutions to answer research questions, accelerating our understanding of the COVID-19 virus and contributing to the implementation of curative treatments. Additionally, NCATS' portfolio of rare disease research programs has catalyzed innovation and advancements for the 30 million Americans with a rare disorder. And finally, since its inception, the Therapeutics for Rare and Neglected Diseases (TRND) program has helped to bring 44 investigational new drugs to market, providing tangible results that directly benefit patients most in need.

NCATS' budget, as proposed by both the House and Senate, threatens to impede innovation. Without additional funds, the N3C program will not only be unable to expand to other disease areas, but will likely sunset in 2024. Additionally, the TRND program will likely have to halt additional projects aimed at the most promising therapeutic candidates, such as leveraging RNA-directed therapies for rare genetic diseases. Also at risk are cutting edge programs like the Platform Vector Gene Therapy program aimed at establishing a platform approach to conducting gene-therapy clinical trials for multiple diseases simultaneously, and the Tissue Chips program that is developing specialized chips that model human tissue to provide alternative platforms for research that are more effective in determining a treatment's potential therapeutic benefit.

To prevent debilitating cuts to these important programs, we strongly urge Congress to increase NCATS' non-CTSA appropriations by approximately 3%, a level commensurate with other NIH institutes and centers as well as NCATS' own CTSA program. By doing this, Congress would affirm the critical role NCATS plays, empowering the Center to accomplish its mission of advancing innovative treatments and cures.

Sincerely,

FasterCures, a center of the Milken Institute
Friedreich's Ataxia Research Alliance (FARA)
Muscular Dystrophy Association (MDA)
National Organization for Rare Disorders (NORD)
PTC Therapeutics
St. Jude Children's Research Hospital
Vesalius Therapeutics