

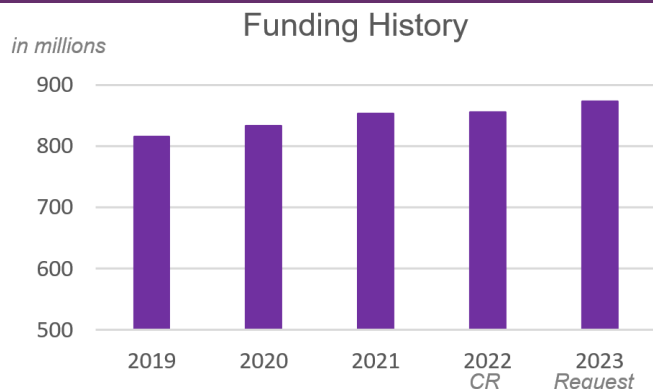


NCATS: A “Unique” Mission

Since its establishment in Fiscal Year 2012, NCATS focuses on **shortening the journey from scientific observation to clinical intervention, so that more treatments and cures can reach more patients more quickly**. NCATS supports development of innovative research tools, technologies, and strategies that can quickly pivot to address emerging needs or unaddressed, long-standing problems. NCATS undertakes research projects which cut through scientific and operational roadblocks. These robust yet nimble approaches are leveraged to address rare diseases, clinical trials, and public health emergencies, including the opioid crisis and COVID-19 pandemic, among other research areas.



Joni L. Rutter, Ph.D., became the NCATS Acting Director in April 2021. She is recognized internationally for her work in basic and clinical research in human genetics.



Research Highlights

- Leveraging expertise in forming collaborative teams, engaging the academic community, and building partnerships with private sector experts to benefit all diseases, including rare diseases
- Platform-based approaches to address translational research needs, such as gene-targeted therapies which can be used for many genetic and rare diseases
- Addressing COVID-19 needs and future pandemic preparedness through drug repurposing and screening
- Active involvement in four COVID-19 clinical trials: expansion of two convalescent plasma trials, immune system modulation therapy trials, and trials to test several existing drugs for mild-to-moderate COVID-19
- Home to a centralized national data enclave and analytics platform to systematically collect clinical data on COVID-19-tested patients for research

Facts and Figures:

- ~ 3,000 drugs and compounds in the NCATS Pharmaceutical Collection (NPC) for drug screening
- 61 Clinical and Translational Science Awards (CTSA) hubs
- 12 million+ individuals represented by data derived from electronic health records (EHRs) in the NCATS National COVID Cohort Collaborative (N3C) research enclave
- RDCRN: 20 consortia studying 200 rare diseases
- 146 intramural projects
- 28 patents licensed
- (CY 2018-2021)
- 14 INDs (CY2018-2021) from NCATS intramural advances





Focusing on Rare Diseases and Harnessing Cross-cutting Research Approaches and Collaborations

The concept of “further together” is demonstrated through the concept of platform-based approaches to rare diseases research. With the myriad of rare diseases in need for cures and treatments, only cross-cutting approaches will result in the dramatic impacts to enhance the livelihood of people affected by rare diseases. Through the Bespoke Gene Therapy Consortium (BGTC) and the Platform Vector Gene Therapy (PaVe-GT) program, NCATS is actively leading highly collaborative efforts to more-readily advance gene-directed therapies for rare disease treatment needs, as well as demonstrate the potential of gene therapies for broader use. NCATS is also leveraging informatics capabilities and approaches to harness data to understand rare diseases in new ways.

Catalyzing the Community for Rapid Implementation of Clinical Trials

NCATS’ CTSA Program is a national network of clinical trial-ready institutions, capable of addressing complex clinical research needs. NCATS and the CTSA Program have deployed the resources and expertise to rapidly engage in clinical trials for a variety of interventions. Most recently, NCATS oversees a large clinical trial to test up to seven over-the-counter and repurposed drugs for self-administered home treatment of mild-to-moderate COVID-19. NCATS takes an active involvement in patient recruitment for these efforts.

Making Data Accessible to Support Research

For example, NCATS’ National COVID Cohort Collaborative (N3C) enables COVID-19 studies on a nation-wide harmonized data set derived from electronic health records (EHRs) from over 12 million COVID-19 tested patients, with demographic representation similar to that of the American population.

Identifying and Testing Potential Therapies Faster

Many NCATS efforts support the repurposing of existing drugs and molecules as a path towards discovering new treatments for disease and identifying ways to overcome technical and operational barriers. Advanced technological approaches and team science approaches allow NCATS’ scientists to rapidly test over 10,000 compounds and identify their biological interactions with the COVID-19 virus. NCATS built the OpenData Portal to openly and quickly share data from these COVID-19-related drug repurposing experiments. Through participation in the Antiviral Program for Pandemics (APP), they will continue using rapid screening and dissemination models, and collaboration with academic scientists and the private sector, to advance drug discovery and development in preparedness for future infectious disease needs.

TRANSLATIONAL SCIENCE IS IMPROVING THE PROCESS:



Understanding what’s similar across diseases to help develop multiple treatments at a time



Developing models that better predict a person’s reaction to a treatment



Enhancing the design and conduct of clinical trials so the results more accurately reflect the patient population



Learn more at:
ncats.nih.gov