

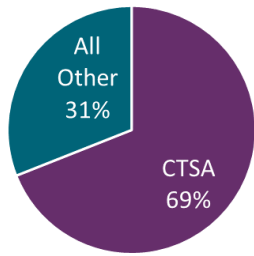


# More Treatments for All People More Quickly

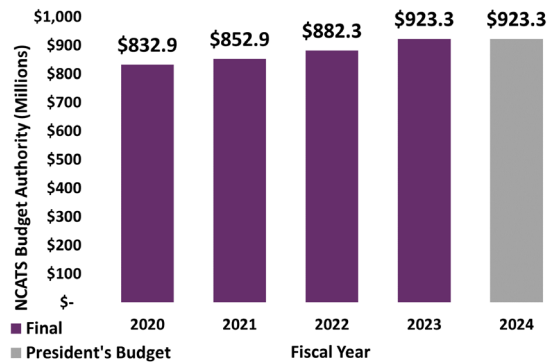
## NCATS' Approach

NIH's National Center for Advancing Translational Sciences (NCATS) works with patient organizations, researchers and clinicians to focus on what's common across diseases. Together, we develop tools, technologies and approaches that overcome roadblocks slowing medical progress — from high failure rates in the therapy development pipeline to inequities in clinical outcomes. We support a range of initiatives to achieve a future that offers **more treatments for all people more quickly**.

## Funding History



NCATS Appropriations



## NCATS by the Numbers

- >60** medical research institutions in NCATS' Clinical and Translational Science Awards (CTSA) program network
- 243** intramural collaborations active across all sectors
- 356** patents issued to NCATS since 2010, including **88** U.S. and **268** foreign patents
- 48** approved Investigational New Drug (IND) applications built on a decade of NCATS' advances

*Of the more than 10,000 known rare diseases, only about 500 have treatments. That means that millions of people with illnesses or diseases are waiting for safe and effective treatments. The therapeutic challenge is particularly great for the 30 million people living with a rare disease, for whom a lack of treatments and diagnostic tools leads to annual direct medical costs of \$400 billion in the United States. To overcome these formidable challenges, NCATS is speeding the translation of scientific discoveries into health solutions.*

## More Treatments

- Advanced tools, such as 3-D tissue bioprinting and tissue chips, are designed to better predict efficacy, improve drug development success rates and lower the number of costly therapies.
- The Platform Vector Gene Therapy (PaVe-GT) program and the Bespoke Gene Therapy Consortium (BGTC) will lead to many more gene therapies for rare diseases.
- Such initiatives as the Rare Diseases Clinical Research Network (RDCRN) and the Biomedical Data Translator seek solutions that can be applied across conditions and diseases.

## All People

- NCATS programs and initiatives tackle health disparities and build organizational cultures that support diversity, equity, inclusion and accessibility.
- NCATS' CTSA Program institutions are leading the way with strategies that increase participation among underrepresented groups in clinical research and the workforce.

## More Quickly

- The National COVID Cohort Collaborative (N3C) harnesses real-world data to quickly explore and test critical clinical research questions as the pandemic evolves.
- The Streamlined, Multisite, Accelerated Resources for Trials (SMART) Institutional Review Board (IRB) Platform and the Trial Innovation Network (TIN) overcome time-consuming and failure-causing roadblocks in clinical trials.

## Rare Diseases Research Innovation

**NCATS is the heart of rare diseases research at NIH. We support programs that find solutions for rare diseases, including:**

- The **RDCRN** of physicians, scientists and patient advocacy groups, which is delivering insights into more than 200 rare diseases. The network plays a pivotal role in developing therapies, including a U.S. Food and Drug Administration–approved drug to treat acute intermittent porphyria.
- The **Impact of Rare Diseases on Patients and Healthcare Systems (IDeaS) pilot study**, which mapped the lengthy and arduous diagnostic odyssey many people with rare diseases face. The study revealed that the annual direct medical costs for people in the United States with rare diseases are \$400 billion.
- The **PaVe-GT** program, which uses the same gene therapy delivery system and manufacturing methods in multiple gene therapy trials. The trials are targeting rare liver and neuromuscular conditions.

## COVID-19

**We are developing and supporting initiatives to meet the urgent public health demands of the COVID-19 pandemic rapidly and flexibly, including:**

- The **N3C**, a nationwide electronic health records data platform. N3C has revealed critical insights into long COVID, breakthrough infections, and how COVID-19 risks vary across ages, races, chronic conditions and treatment regimens.
- Clinical trials conducted through NCATS' **CTSA Program** network. The trials tested convalescent plasma, immune modulators for hospitalized patients, and repurposed drugs for mild-to-moderate COVID-19.
- The **OpenData Portal (ODP)**, which shares COVID-19-related drug effectiveness data and at-a-glance summaries of how individual SARS-CoV-2 variants may respond to known treatments.

## Audacious Goals

As NCATS advances into its second decade, we have set audacious goals for the next 10 years:



### **More treatments**

Have a treatment in the pipeline for 25% of known diseases.



### **For all people**

Enable a workforce and research enterprise that inclusively benefits health for everyone



### **More quickly**

Cut the average time for diagnostics and therapeutics to reach people



## Diversity, Equity, Inclusion and Access to Research

**We are committed to greater inclusion in our research and workforce to improve the health of all communities through the following initiatives:**

- The CTSA **TIN**, which develops innovative approaches to boost diversity in clinical trials. The TIN's **Recruitment Innovation Center (RIC)** brings diverse groups of collaborators into the planning and implementation of clinical research.
- The expansion of research tools, such as tissue chips for drug screening and data sets to include underserved populations, in the drug discovery process.
- The NCATS **Gaining Research Equity and Advancement in Translational Sciences (G.R.E.A.T.S) Program**, which supports the career development of a diverse group of undergraduate and graduate students.