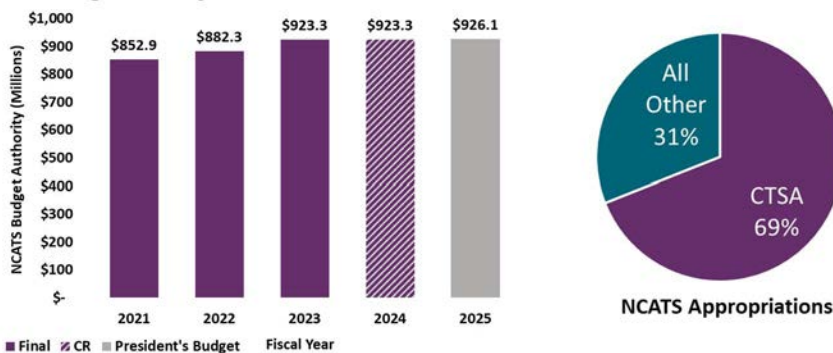NIH》 National Center for Advancing Translational Sciences

More Treatments for All People More Quickly

NCATS' Approach

NIH's National Center for Advancing Translational Sciences (NCATS) works with patient organizations, researchers, industry, clinicians, and other partners 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 Budget Authority (Millions)

Fiscal Year	Value
2021	$852.9
2022	$882.3
2023	$923.3
2024	$923.3
2025	$926.1

■ Final ▨ CR ■ President's Budget

NCATS Appropriations
- All Other 31%
- CTSA 69%

NCATS by the Numbers

🏛 **>60 medical research institutions** in NCATS' Clinical and Translational Science Awards (CTSA) program network

👥 **240 intramural collaborations** active across all sectors

📇 **395 patents** issued to NCATS since 2010, including **94** U.S. and **301** foreign patents

🗒 **55 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 in the United States, for whom a lack of treatments and diagnostic tools leads to annual direct medical costs of **$400 billion and nearly $1 trillion total costs**. To overcome these formidable challenges, NCATS is speeding the translation of scientific discoveries into health solutions.*

More Treatments

- New Approach Methodologies (NAMs), 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 more therapies for rare diseases.
- 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 builds and supports organizational cultures that support diversity, equity, inclusion, and accessibility.
- NCATS' CTSA institutions are increasing participation among underrepresented groups in clinical research and the translational science workforce. CTSA institutions collaborate with many partners, including minority-serving institutions and rural organizations, across the United States.

More Quickly

- The Streamlined, Multisite, Accelerated Resources for Trials (SMART) Institutional Review Board (IRB) Platform and the Trial Innovation Network (TIN) overcome roadblocks in clinical trials to cut study start-up time.
- Rapid screening of the NCATS Pharmaceutical Collection of approved drugs is enabling researchers to find leads for rare and common diseases, to move quickly into preclinical and clinical testing.

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 consists of 20 consortia that include physicians, scientists, and patient advocacy groups 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.

Audacious Goals

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

More treatments
Increase the number of treatments in development by fivefold.

For all people
Dramatically increase diversity, equity, inclusion, and access across every area NCATS supports.

More quickly
Enable diagnostics and therapeutics to reach people twice as fast.

Emerging Public Health Needs

We develop and support research initiatives to meet urgent public health demands, including:

➢ Clinical trials conducted through NCATS' nationwide CTSA Program network: By reaching people where they are, NCATS makes research participation easier through innovations in clinical trial recruitment, design, and management.

➢ Drug repurposing and human cell-based screening: NCATS' already-established capabilities have been pivoted towards emerging infectious disease concerns (e.g., Zika, SARS-CoV-2), rare diseases, as well as novel paths for addressing pain and addiction (NIH HEAL Initiative collaborations).

➢ Clinical data harmonization in cloud-based repositories: Moving towards a clinical data resource for studying many diseases, based on the National COVID Cohort Collaborative, or N3C, that harmonizes data from electronic health records to reveal critical insights into Long COVID, and how COVID-19 health outcomes vary across ages, races, chronic conditions, and treatment regimens.

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 and relevant health needs, 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.

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