

# Speeding More Treatments for All

Of the more than 10,000 known 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 diagnostic tools and treatments leads to annual direct medical costs of \$400 billion in the United States. To overcome these formidable challenges, NIH's National Center for Advancing Translational Sciences (NCATS) is speeding the translation of scientific discoveries into health solutions.

## NCATS' Approach

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 the roadblocks slowing biomedical 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.

**More treatments:** Advanced tools, such as [3-D tissue bioprinting](#) and [tissue chips](#), are designed to better predict drug development efficacy, improve drug development success rates and lower the number of costly failures. 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. Initiatives such 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. For example, the [Clinical and Translational Science Awards \(CTSA\) Program](#) institutions are leading the way with strategies that increase participation among underrepresented and disproportionately affected 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 Platform](#) and the [Trial Innovation Network \(TIN\)](#) seek to overcome time-consuming and failure-causing obstacles in clinical trials.

## NCATS by the Numbers

- ✓ More than 60 medical research institutions in the [CTSA Program](#) national network
- ✓ 243 active intramural research collaborations across all sectors
- ✓ 356 patents issued to NCATS, including 88 U.S. patents and 268 foreign patents
- ✓ 48 approved Investigational New Drug applications built on a decade of NCATS' intramural advances
- ✓ \$923 million in fiscal year 2023; CTSA Program receives 68%

## NCATS in Focus

### *Rare Diseases*

NCATS is the heart of rare diseases research at NIH. The programs that NCATS supports to find solutions for rare diseases include the following:

- The [RDCRN](#) of physicians, scientists and patient advocacy groups, which delivers insights into more than 200 rare diseases. The RDCRN plays a pivotal role in therapy development, 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 that so many people with rare diseases face. IDeaS revealed that the annual direct medical costs for people in the United States with rare diseases may total \$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*

NCATS is developing and supporting initiatives to meet the urgent public health demands of the COVID-19 pandemic rapidly and flexibly. These initiatives include the following:

- 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 treating mild-to-moderate COVID-19.
- The [OpenData Portal](#), 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.

### *Diversity, Equity, Inclusion and Access to Research*

NCATS is committed to greater inclusion in its research and its workforce to improve the health of all communities through the following initiatives:

- The CTSA Program's [TIN](#), which develops innovative approaches to boost diversity in clinical trials. The TIN's [Recruitment Innovation Center](#) brings diverse groups of stakeholders into the planning and implementation of clinical research.
- Expansion of research tools, such as [tissue chips for drug screening](#) and data sets, to include underserved or vulnerable 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 through intramural research internships at NCATS.

### Audacious Goals

As NCATS enters its second decade, it has 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.