

2023 — 2024

Biennial Report



National Center
for Advancing
Translational Sciences

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This report contains active web links. For full access to linked resources, please view the online PDF version at <https://ncats.nih.gov/about/NCATS-Biennial-Reports>.

Executive Summary

Since its establishment in 2011, NCATS has strived to fulfill its mission to turn research observations into health solutions through translational science. NCATS works to advance the development, testing, and implementation of diagnostics and therapeutics for a wide range of diseases and conditions. Under Section 479 of the Public Health Service Act, NCATS is required to publish a biennial report on the center's research activities. The first report, covering 2012–2013, focused on NCATS' earliest successes. The purpose of these reports is to showcase NCATS' scientific and operational innovations in translational science that have helped accelerate the journey from research discoveries to real-world health solutions.

The *NCATS 2023–2024 Biennial Report* is structured in alignment with the five goals of the *NCATS Strategic Plan 2025–2030*, which was released in September 2024. The strategic plan was thoughtfully developed to articulate the work NCATS is already doing while also understanding how the center can better fill critical gaps in translational science in future years. The plan's five goals are intended to build upon NCATS' core mission and values:

- Goal 1: Advance Development of and Access to More Treatments, Particularly for Diseases With Unmet Needs
- Goal 2: Empower Everyone to Contribute to and Benefit From Translational Science
- Goal 3: Accelerate Translational Science by Breaking Barriers and Boosting Efficiency
- Goal 4: Leverage Crosscutting Strategies to Enhance Translational Science
- Goal 5: Champion Effective Stewardship of Translational Science Through Transparency, Integrity, and Accountability

These five goals reflect the culmination of a two-year effort that was carried out under the leadership of Joni L. Rutter, Ph.D., NCATS director. The strategic planning process was overseen by an internal team and included input from across NCATS, as well as from other NIH institutes, centers, and offices. NCATS also sought input from members of academic institutions, patient communities and advocacy organizations, private industry, and the public. This input, articulated in the strategic plan, will help guide future NCATS activities.

This biennial report showcases how the center is transforming the landscape of translational science by highlighting some of the work aligned with NCATS' strategic goals and objectives. Moving forward, NCATS will continue to play a critical role within the biomedical research landscape, ultimately fulfilling its vision to develop more treatments for all people more quickly. Within these pages are examples of how NCATS has —

- Focused on diseases with unmet needs, including rare diseases, to increase the number of treatments and improve the well-being of patients.
- Leveraged feedback from the community to increase participation in research development and clinical trials and gather more real-world data.
- Produced streamlined workflows to enhance translational research efforts and reduce the treatment timeline from laboratory bench to bedside using various approaches, including drug repurposing.
- Utilized cross-disciplinary team science and initiated strategic partnerships to enhance resource accessibility and gain input from various experts.
- Prioritized effective stewardship that will increase transparency, reproducibility, and rigor and encourage public trust in groundbreaking discoveries and treatments.



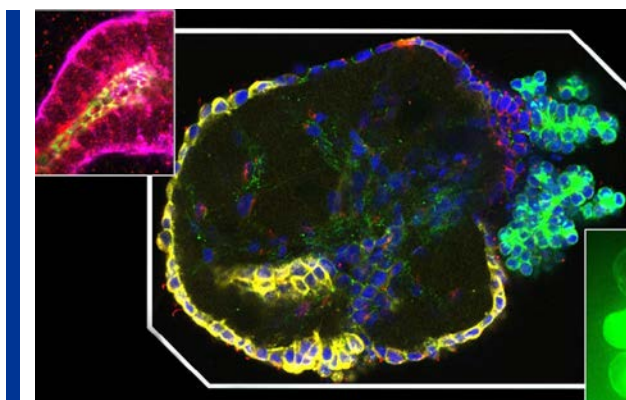
Joni L. Rutter, Ph.D.
NCATS Director



Goal 1: Advance Development of and Access to More Treatments, Particularly for Diseases With Unmet Needs

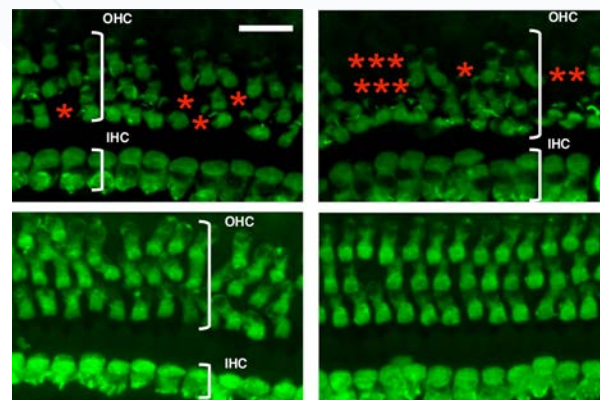
Thousands of diseases, many of which are rare, affect millions of people, and more treatments are needed. NCATS is committed to expanding capacity to develop faster and more accurate diagnoses, along with new treatments to help offset this public health challenge. NCATS aims to revolutionize research, treatment, and diagnostic tools for diseases with unmet needs, especially rare and hard-to-treat conditions. The center uses a variety of approaches, including enhancing preclinical screening, refining clinical research methods, finding new indications for existing medications, and advancing personalized medicine. NCATS' goal is clear: Increase the number of treatable diseases by identifying their underlying causes; supporting the development of new treatments; and improving the reach, accessibility, and uptake of existing treatments.

Supporting Examples



New 3-D Model Offers Insights Into the Role of Glucose in a Deadly Kidney Disease

NIH-supported researchers combined organ-in-a-dish and organ-on-a-chip technologies to better understand polycystic kidney disease (PKD) and show the role of glucose in forming PKD cysts.



With Gene Editing, Mice With a Form of Inherited Deafness Can Hear Again

A gene editing approach restored hearing in adult mice with a type of inherited hearing loss. This approach may eventually lead to potential treatments for people with the same condition.



Electrifying Invention Can Save Young Lives and Ease Chronic Lung Conditions

NCATS awardees used a Small Business Innovation Research (SBIR) grant to develop a compact wearable device that makes nitric oxide on the spot instead of storing pre-made gas. This removes the costs of producing, distributing, and storing compressed-gas cylinders.



Scientists Reveal a Potential New Approach to Treating Liver Cancer

Scientists at NIH and Massachusetts General Hospital uncovered a potential new approach against liver cancer that could lead to the development of a new class of anticancer drugs.



NCATS Enables IND Clearances and Drug Approvals

NCATS plays a pivotal role in progressing new therapeutics to market, directly benefiting patients. Through the end of 2024, 54 Investigational New Drug (IND) clearances and 4 New Drug Application (NDA) approvals have resulted from research conducted through NCATS' Intramural Research Program.



Goal 2: Empower Everyone to Contribute to and Benefit From Translational Science

For disease burden to be reduced, all people must have the opportunity to contribute to and benefit from translational science. NCATS' commitment to including different perspectives spans every area the center supports. This involves designing research with participant and community input. NCATS strives to engage with communities to understand their needs. Furthermore, as a values-based, people-centric organization, NCATS fosters a bold, creative, and innovative environment where staff can grow, thrive, and contribute to the center's mission. Members of the translational science workforce at NCATS and beyond receive training, mentorship, and development across the career spectrum to reflect these values.

Supporting Examples



Rare Disease Day at NIH 2024: Showcasing Voices and New Opportunities

The rare diseases community came together at Rare Disease Day at NIH to share inspiring stories, impactful research advances, and exciting new possibilities for the future.



Tox21 Team Finds Environmental Chemicals That Could Trigger Early Puberty in Girls

A chemical commonly used in fragranced hygiene products may trigger the onset of premature puberty in girls. NCATS researchers in the Toxicology in the 21st Century (Tox21) program tested thousands of environmental chemicals to investigate this topic.



NCATS N3C Study Shows Health Factors Before COVID-19 Infection May Predict Long COVID Risk

NCATS researchers found the strongest predictor of getting long COVID may be factors related to the use of health care services before COVID-19 infection.



NCATS Report Highlights Decentralized Clinical Trials' Challenges, Opportunities

A new report offers an approach to strengthen and expand decentralized clinical trials, potentially reaching more participants and delivering real-world research findings more quickly.



A Framework for Assessing Clinical Trial Site Readiness

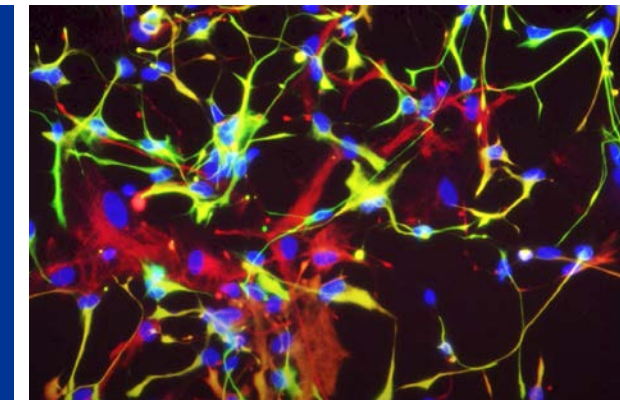
NCATS-supported clinical trial experts developed a framework for determining clinical trial site readiness. This framework may help new sites enter the clinical trials enterprise and improve reach to underserved communities.



Goal 3: Accelerate Translational Science by Breaking Barriers and Boosting Efficiency

Turning discoveries into health solutions takes too long. NCATS aims to improve both scientific and operational processes to make translation more effective and efficient, ultimately reducing the time it takes for treatments to reach patients. By streamlining scientific and operational processes and integrating data science more effectively, researchers can reveal new knowledge and spur advances more broadly. Making processes easier and faster will allow teams to focus on scientific goals that deliver impactful results. NCATS' approach includes expanding and disseminating templated agreements, providing resources to involve patients and communities in research, automating routine tasks, and optimizing data management processes.

Supporting Examples



Efficient Approach Creates Astrocytes Faster From Stem Cells for Drug Development and Regenerative Medicine

NCATS scientists used stem cells and a novel shortcut to create large supplies of astrocytes to speed up research on therapies for neurodegenerative diseases.



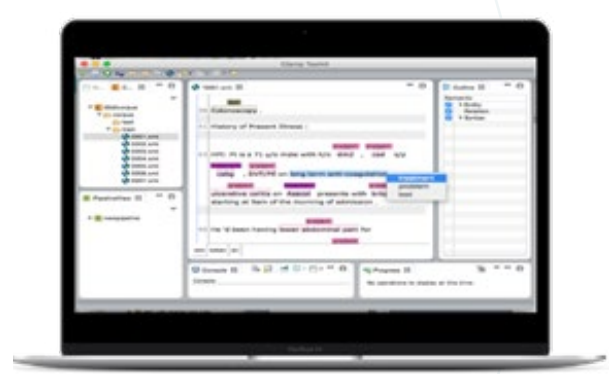
Study Suggests Most People May Have Gene Variants That Impact Drugs' Effectiveness

An NCATS-funded study showed four out of five people took medicines that could have been affected by their genes. This work highlights the potential benefits over a person's lifetime of using genomic information to personalize medical treatments.



As Flights End, Tissue Chips in Space Projects Offer Glimpses Into the Biology of Aging

The NCATS-led Tissue Chips in Space program sent its final projects to the International Space Station to help researchers learn about aging-related diseases and conditions and potentially translate results into treatments.



Artificial Intelligence Is Advancing Clinical Research and Data Quality

NCATS awardees used SBIR funding and Clinical and Translational Science Award (CTSA) Program grants to commercialize a customizable natural language processing software.



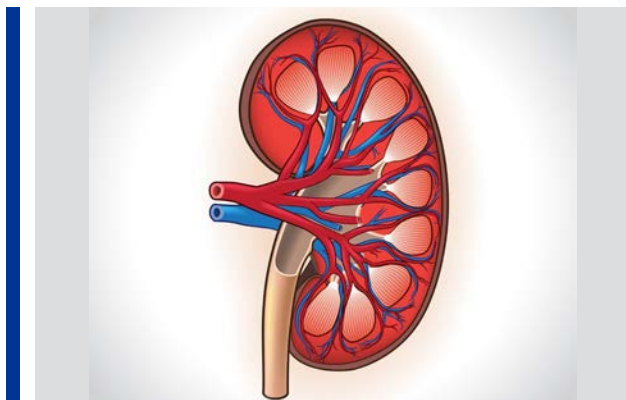
Faster Cures, More Genetic Treatments

Using NCATS' Biomedical Data Translator, clinicians identified a drug that was repurposed to treat SHINE syndrome, a rare neurodevelopmental disorder.

Goal 4: Leverage Crosscutting Strategies to Enhance Translational Science

NCATS will continue to leverage crosscutting approaches to enhance its work, bringing more treatments to all people more quickly. Promoting team science will bring together different types of expertise to tackle complex problems. Initiating strategic partnerships will extend NCATS' reach and resources. Embracing data science and facilitating FAIR (findable, accessible, interoperable, and reusable) data will unlock new insights and accelerate discoveries. NCATS' commitment to education and outreach ensures that the center is continuously learning, growing, and engaging with its communities.

Supporting Examples



NCATS-Funded Rare Disease Researchers Provide Key Data, Help Build New Kidney Atlas

The Nephrotic Syndrome Study Network (NEPTUNE) has helped researchers learn more about the biology of both rare and common kidney diseases. It has also led to better ways to diagnose and treat these conditions.



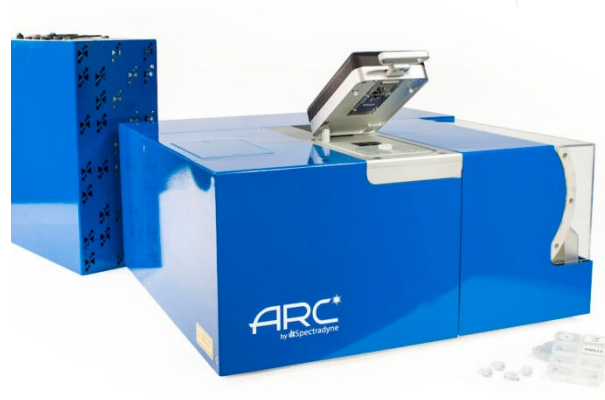
New Path for a Gene Therapy Trial at NIH for a Rare Metabolic Disease

Scientists at NCATS and other NIH institutes and centers teamed up to advance a gene therapy treatment for MMA, a rare and potentially life-threatening genetic disorder.



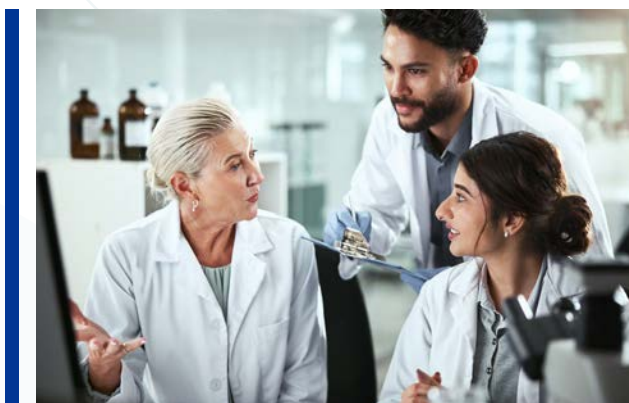
NCATS Researchers Help Gene Therapy for AADC Deficiency Gain FDA Approval

NCATS' Therapeutics for Rare and Neglected Diseases (TRND) program helped develop a recently approved gene therapy for a rare genetic brain disorder.



Spectradyne: SBIR Funding Spurs Advances in Nanoparticle Technology

NCATS SBIR funding helped Spectradyne, LLC, commercialize an improved nanoparticle analyzer critical to research and drug development.



Training Tomorrow's Translational Scientists to Address Neonatal Opioid Withdrawal

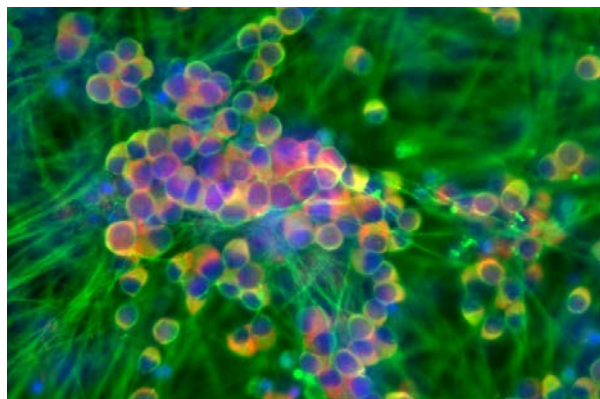
NCATS provides national leadership to strengthen the translational science workforce and accelerate the path from discovery to health solutions. Through CTSA training, researchers developed a safer buprenorphine treatment for neonatal opioid withdrawal syndrome, which advanced into a startup gaining national recognition.



Goal 5: Champion Effective Stewardship of Translational Science Through Transparency, Integrity, and Accountability

Effective stewardship is key to NCATS' success and the achievement of the center's strategic goals. NCATS is dedicated to the innovative and responsible use of resources to advance its mission, aligning with NIH's commitment to fostering a culture of integrity and efficiency. At NCATS, stewardship goes beyond resource management. It means building relationships, embracing innovation, streamlining processes, and improving efficiencies to ensure successful translation. NCATS focuses on internal and external collaboration to overcome scientific, operational, and policy challenges. By working ethically and responsibly, NCATS fosters a collaborative environment with its communities. NCATS will ensure that transparency, integrity, and accountability drive the center's efforts, propelling translational science forward.

Supporting Examples



NCATS Scientists Turn Stem Cells Into Vast Supply of Neurons to Better Predict Pain Therapies' Effectiveness

NCATS researchers and collaborators are testing possible new pain-relieving drugs in the laboratory on nerve cells. The team created a protocol that is highly reproducible and efficient, addressing a major roadblock in the field of stem cell research.



Forgoing One Food Treats Eosinophilic Esophagitis as Well as Excluding Six

Researchers report that removing animal milk alone from the diet of adults with eosinophilic esophagitis is as effective at treating the disease as eliminating animal milk plus five other common foods. This multi-site, randomized trial supports robust research and will help improve transparency regarding treatment options for patients.



NIH Gene Therapy Team Reveals Its Path to FDA Orphan Drug and Rare Pediatric Disease Designations

NCATS received an Orphan Drug Designation for an investigational gene therapy to treat a form of propionic acidemia, a rare metabolic disorder. NIH's Platform Vector Gene Therapy (PaVe-GT) researchers are sharing what they have learned in hopes of speeding others' work in this field.



Using AI to Shape the Future of Treatment Personalization

An NCATS SBIR grantee used artificial intelligence to predict how diseases and different treatments will affect individual patients. The tool uses robust data to create a learning health system that will potentially allow clinicians to predict disease progression and treatment efficacy for individual patients.



Areas of Strength and Opportunities for Growth in Translational Science Education and Training

NCATS conducted a scoping review of translational science education and training literature. The findings from this review will inform the development of a highly qualified translational science workforce.

Appendix

Appendix: Statutory Language on Biennial Report

Public Health Service Act

Section 479 NATIONAL CENTER FOR ADVANCING TRANSLATIONAL SCIENCES

(c) BIENNIAL REPORT. — The Center shall publish a report on a biennial basis that, with respect to all research supported by the Center, includes a complete list of —

- (1) the molecules being studied;
- (2) clinical trial activities being conducted;
- (3) the methods and tools in development;
- (4) ongoing partnerships, including —
 - (A) the rationale for each partnership;
 - (B) the status of each partnership;
 - (C) the funding provided by the Center to other entities pursuant to each partnership, and
 - (D) the activities which have been transferred to industry pursuant to each partnership;
- (5) known research activity of other entities that is or will expand upon research activity of the Center;
- (6) the methods and tools, if any, that have been developed since the last biennial report was prepared; and
- (7) the methods and tools, if any, that have been developed and are being utilized by the Food and Drug Administration to support medical product reviews.

(d) INCLUSION OF LIST. — The first biennial report submitted under this section after the date of enactment of the 21st Century Cures Act shall include a complete list of all of the methods and tools, if any, which have been developed by research supported by the Center.

(e) RULE OF CONSTRUCTION. — Nothing in this section shall be construed as authorizing the Secretary to disclose any information that is a trade secret, or other privileged or confidential information subject to section 552(b)(4) of title 5, United States Code, or section 1905 of title 18, United States Code.

Responses to Required Information

(1) the molecules being studied	NCATS' Division of Preclinical Innovation maintains a number of compound collections for high-throughput screening and drug discovery, including several custom designed and curated libraries. For a list of NCATS' small molecule collections, visit our Compound Management page. One example is the NCATS Pharmaceutical Collection, which is a comprehensive drug-repurposing collection of all approved and investigational drugs that provides a valuable resource for both validating new models of disease and better understanding the molecular basis of disease pathology and intervention Data generated through NCATS' screening efforts are deposited in PubChem (within NIH's National Library of Medicine). See a list of NCATS-deposited compounds/data within PubChem.
(2) clinical trial activities being conducted	ClinicalTrials.gov is a database of privately and publicly funded clinical studies conducted around the world. View the studies for which NCATS is a sponsor/collaborator. View the list of clinical research studies being supported by NCATS' Rare Diseases Clinical Research Network (RDCRN).
(3) the methods and tools in development	This biennial report highlights many of the ongoing efforts of NCATS to develop methods and tools that will improve the translational research process. View the complete list of all of the active projects funded by NCATS.
(4) ongoing partnerships, including — (A) the rationale for each partnership; (B) the status of each partnership; (C) the funding provided by the Center to other entities pursuant to each partnership; and (D) the activities which have been transferred to industry pursuant to each partnership	A and B) NCATS' Division of Preclinical Innovation develops approaches that improve the efficiency and effectiveness of translation. To do so, NCATS intramural scientists leverage state-of-the-art laboratories and collaborations among government, industry, academia, and patient and rare disease communities to advance new technologies to make preclinical research more predictive and efficient. C) Investigators do not receive grant funds through NCATS' intramural program. Instead, selected researchers partner with NCATS experts to generate preclinical data and clinical-grade material through government contracts for use in Investigational New Drug (IND) applications to a regulatory authority, such as the U.S. Food and Drug Administration (FDA). Search the list of NCATS intramural projects. D) For information on any activities which have been transferred to industry, please inquire with NCATSPartnerships@mail.nih.gov.
(5) known research activity of other entities that is or will expand upon research activity of the Center	NIH investigators frequently conduct PubMed searches on areas of research interest. However, NIH does not conduct competitive intelligence on for-profit entities.

(6) the methods and tools, if any, that have been developed since the last biennial report was prepared	NCATS plays a pivotal role in progressing new therapeutics to market, directly benefiting patients. We partner with external researchers to help bridge the gap between the preclinical and clinical stages of therapeutic development by advancing promising medicinal agents through late-stage preclinical development and IND-enabling studies. Our scientists also develop small molecules that drug companies can license and further develop for clinical trials. To date, 56 IND clearances and 4 New Drug Application (NDA) approvals have resulted from research conducted at NCATS as part of our Intramural Research Program. View the updated list of all IND clearances and NDA approvals to which NCATS has contributed. In addition, NCATS also develops and shares small molecule “probes,” which are compounds with extensively validated effects on a potential disease target, and which can be used as powerful tools in the development of new therapeutics. Generating these chemical probes requires specialized expertise and facilities, and scientists within NCATS’ Division of Preclinical Innovation collaborate with disease experts to develop new screening assays and methods to find and modify screening hits into small molecule probes and potential therapies. View the list and descriptions of the assays (methods) developed by NCATS’ intramural program.
(7) the methods and tools, if any, that have been developed and are being utilized by the FDA to support medical product reviews.	Global Substance Registration System (GSRS) The GSRS resource is a registration system for the ingredients in medicinal products. This project, developed by NCATS scientists, makes it easier for FDA, regulators, and other stakeholders to exchange information about substances in medicines, supporting scientific research on the use and safety of these products. While the main goal is to assist agencies in registering and documenting information about substances found in medicines, a collaboration with the FDA also has enabled NCATS to publish a public dataset of substance records, which is updated on a regular basis and used by collaborators in regulatory science as well as industry. The EU Substance Registration System (EU-SRS) is sponsored by both the European Medicines Agency (EMA) and the German Federal Institute for Drugs and Medical Devices (BfArM). This system uses the GSRS software NCATS-hosted FDA public data to support substance curation processes for the EU. CURE ID, created through a collaboration between NCATS and the FDA, enables the crowdsourcing of medical information from health to facilitate the development of new treatments using repurposed drugs for difficult-to-treat diseases, including rare diseases, infectious diseases, and chronic conditions, among others. CURE ID is accessible through a website, smartphone, or other mobile device.



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