

Draft NCATS Strategic Plan for 2024–2029

NCATS Predecisional Draft for Public Comment

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Director's Message

At the 10-year anniversary of the National Center for Translational Sciences (NCATS) in 2021, I presented a vision for the center: a future with more treatments for all people more quickly. That vision included a [set of audacious goals](#) outlining what NCATS, along with the broader translational science community, should be striving to achieve during the next decade.

Bold ideas, impactful programs, and new partnerships are vital to achieving this vision. The *NCATS 2024–2029 Strategic Plan* is a tool to guide us.

This strategic plan represents a thorough, dynamic approach to advancing our mission of turning research observations into health solutions through translational science. The plan's goals and objectives were shaped by input from staff and external partners. Their input came during more than 40 listening sessions and through a public request for information.

The plan addresses three key translational science challenges that are reflected in our vision:

1. The limited number of diseases with treatments
2. The need to bring more individuals and communities into the translational science space
3. The inefficiencies in translational science that slow efforts

To overcome these challenges, we have set forth five strategic goals. These goals rest on core values that emphasize working in a highly collaborative team-science culture, learning and practicing translational science, making data available, and developing a diverse and accountable workforce.

As with the previous strategic plan, patients are at the center. Over the last 10 years, our Clinical and Translational Science Awards (CTSA) Program and Rare Diseases Clinical Research Network (RDCRN) have shown the power of engaging patients and communities throughout the entire research process. This approach led to innovations and interventions that are now saving lives and improving health. These initiatives — and many others — will play a big role under our new strategic direction.

Our strategic plan is a living document. We will continuously assess and adapt our strategies, checking in with ourselves and our constituent communities. We will monitor our progress toward removing translational roadblocks and paving the way to better health. In addition, the plan highlights areas where NCATS has the flexibility to leverage resources and opportunities as they emerge.

As we implement the strategic plan, we will engage the global translational science community and integrate new technologies, sustainability goals, feedback opportunities, and risk management tactics. I also plan to share updates that show progress toward our strategic vision.

I look forward to the work ahead, and I thank you for your continued partnership. Together, we can realize a future of more treatments for all people more quickly.

Strategic Plan Framework

NCATS Mission

Turn research observations into health solutions through translational science

NCATS Vision

More Treatments

for All People

More Quickly

NCATS Strategic Goals



Goal 1: Apply Approaches to Foster the Identification of, Development of, and Access to More Treatments



Goal 2: Enable All People to Contribute to and Benefit From Translational Science



Goal 3: Accelerate Translation by Addressing Both Scientific and Operational Challenges



Goal 4: Broadly Utilize Research and Operations That Cut Across Translational Science Efforts



Goal 5: Work Together as Stewards for Advancing Translational Science to Promote Transparency, Integrity, Accountability, and Social Responsibility

Overview of NCATS

Relevant Mandates and History

NCATS was created on December 23, 2011. Find full details on NCATS' Statutory Authority in [Appendix A](#).

NCATS at a Glance

NCATS conducts and supports research on the science and operation of translation to bring more treatments for all people more quickly. The center applies many approaches and strategies — designed to address more than one disease at a time — to reduce bottlenecks or other roadblocks in translation. NCATS is not focused on specific diseases and conditions, but we are a home for rare diseases.

NCATS Organization

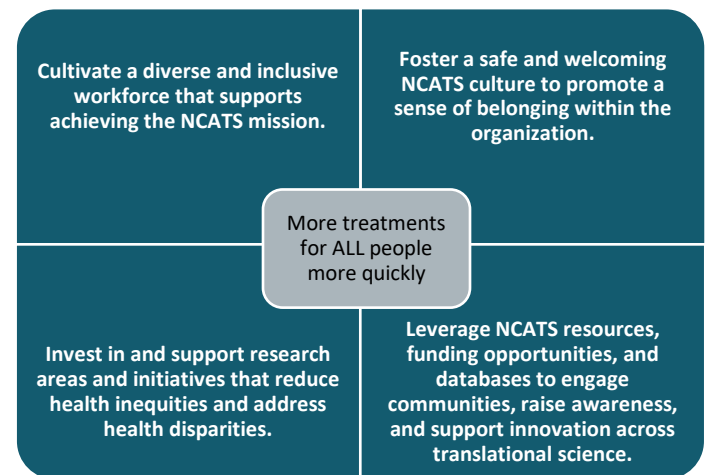
The work done by NCATS [divisions and offices](#) spans the spectrum of preclinical and clinical translational science. (See [Appendix A](#) for the Organizational Chart). Working together across the organization, we identify and devise plans for new opportunities. We build tools and technologies, support research, train scientists, and do so much more. We value innovation, collaboration, and acceleration, and our patient-driven focus is ever-present.

NCATS' Culture and Core Values

NCATS values a diverse and inclusive culture that supports achieving its mission. Our person-driven focus underlies all that we do. We invest in and support initiatives to reduce health inequities and address health disparities. Our programs leverage NCATS resources to engage communities and support innovation across translational science. In doing so, we foster a safe and welcoming environment to promote a sense of belonging within the organization and the communities we support. NCATS' culture is reflected in our draft core values:

- Impactful Innovation
- Collaboration and Team Science
- Open Communication and Discourse
- Diversity, Equity, Inclusion, Accessibility, and Belonging
- Growth and Sustainability
- Efficient, Generalizable Solutions
- Transparency and Stewardship

These core values form the foundation of NCATS' culture and identity, act as a compass for decision-making, strengthen employee motivation and satisfaction, and help establish trust and credibility with our community.



Translational Science and Translational Science Principles

Translation is the process of turning observations in the laboratory, clinic, and community into interventions that improve the health of individuals and the public — from diagnostics and therapeutics to medical procedures and behavioral changes.

NCATS focuses its efforts on translational science. This field generates scientific and operational innovations that overcome long-standing challenges or bottlenecks along the translational research pipeline. We looked at successful initiatives that NCATS has led or supported to identify the principles of successful translation. The [NCATS Translational Science Principles](#) are broad and apply to research along the translational spectrum. We apply these principles to make translational research faster, more efficient, and more impactful.



NCATS' Mission

NCATS' mission is to turn research observations into health solutions through translational science. We work to enhance the development, testing, and implementation of diagnostics and therapeutics for a wide range of diseases and conditions. Key approaches include understanding what is similar across diseases to develop multiple treatments at a time and building models that better predict a person's reaction to a treatment. Clinical trials are enhanced so that the results reflect the full diversity of the population. NCATS partners with other government agencies, including other NIH institutes and centers (ICs); industry; academia; and patient support organizations.

NCATS' Vision and Audacious Goals

Our vision is *more treatments for all people more quickly*. In 2021, Dr. Joni Rutter matched this vision with [audacious goals](#) for the center.



These audacious goals are aspirational. They provide direction and focus our efforts on addressing big challenges in translation.

Overview of the NCATS Strategic Plan

In December 2021, we celebrated our 10-year anniversary and the many successes of NCATS scientists, grantees, and collaborators across the translational science spectrum. (See [Appendix B](#) for more information about our progress, as well as key accomplishments mapped to our 2016 *NCATS Strategic Plan*.) At our celebration, we highlighted our efforts to advance treatments for all diseases. We heard stories from patients and their families of how NCATS tools and collaborations had helped them. Now, with the solid foundation established over our first decade, NCATS looks to the future.

This new strategic plan sets the direction to further innovate and advance translational science principles. Each goal is followed by several objectives and NCATS' approaches for pursuing them. Goals 1, 2, and 3 align directly with the three elements of NCATS' vision: More Treatments, All People, More Quickly. Goal 4 addresses common approaches and strategies that work across many of our efforts. Goal 5 reflects our commitment as stewards for NCATS and translational science. The strategic plan emerged from numerous discussions with our constituents about the vision and their feedback on how and what NCATS should prioritize within that vision.

Strategic Planning Process

NCATS engaged over 1,100 individuals through over 40 roundtable discussions over a year of public stakeholder and internal engagements. Find details on the engagement activities undertaken during the strategic planning process in [Appendix C](#).

Approach to Priority Setting

NCATS takes a holistic view to priority setting and engages in a transparent and inclusive process. During our strategic planning engagements, we heard much about the importance of setting priorities, including sunseting or handing off activities, with intention and transparency. Priority setting at NCATS incorporates many of the steps set forth in the NIH strategic plan. We seek to balance community health needs and understand the opportunities available. We also recognize programs, projects, and other activities that are already addressing or ready to address gaps and opportunities.

Our robust planning processes enable us to develop, prioritize, implement, and sunset or transition our programs. Our approaches to prioritization span NCATS intramural and extramural programs and include a robust internal operations planning process. Further outside review of extramural concepts and program updates occurs during open sessions of the NCATS Advisory Council meetings and through consultation with the Cures Acceleration Network (CAN) Review Board. For our intramural activities, our Division of Preclinical Innovation systematically identifies opportunities to enable staff to explore new and innovative approaches within their research programs. In addition, the intramural *ad hoc* scientific review includes obtaining extensive external expert input on a 4-year basis on the progress and direction of the intramural research program.

In addition to the center's regular priority-setting discussions, we routinely host formal and informal listening sessions that enable our constituents to share their priorities, identify gaps and opportunities in translational science, and discuss our current plans.

Implementation of the Strategic Plan

The 2024 *NCATS Strategic Plan* sets forth five goals, each with specific objectives and high-level approaches to consider for implementation. By continuously evaluating and adapting our strategies and sharing success stories, we will have a significant impact on advancing translational science that has the potential to improve health outcomes. Full details on implementing the strategic plan can be found in [Appendix D](#).

Strategic Priorities

Goal 1: Apply Approaches to Foster the Identification of, Development of, and Access to More Treatments

Of more than 10,000 known diseases, many are rare, and although many have known causes, only a few hundred have safe, effective treatments. This significant unmet need must be matched with strategies that expand the ability and capacity to develop new and effective treatments. We also need strategies that lead to accurate diagnoses, an important step toward improving patient care as well as helping us learn more about a disease. NCATS applies techniques and technologies across the entire translational science spectrum to advance the research, treatments, and diagnostic capabilities for these diseases with unmet needs. We focus on diseases that are rare, intractable, and without treatment. We look for solutions to research challenges common across diseases and for platform approaches that can have multiple uses. We aim to increase the number of diseases with a treatment and improve the reach and uptake of available treatments. We will identify methods that enable treatment development. We will also improve preclinical screening, clinical research approaches, and the application of personalized medicine. Dissemination and implementation approaches will be important to promote access and adherence.

Objective 1-1: Develop strategies to advance diagnosis and targeted interventions that address multiple diseases at a time, particularly for rare diseases and others with unmet needs.

Developing one drug for one disease is slow and inefficient. We can address the extremely small number of approved treatments by expanding the ability and capacity to develop new or more effective treatments more quickly and efficiently.

We invest in efforts designed to create platforms that can address more than one disease at a time or to develop an intervention for one disease that can be readily adapted to apply to other diseases. Strategies will include finding new or additional uses for existing compounds and approved drugs, also called drug repurposing. We will also develop methods that improve the research process, such as connecting different types of data in meaningful ways. We can move toward this objective by using computational modeling and artificial intelligence (AI) and machine learning (ML) and by exploring gene- and cell-targeted therapies.

Objective 1-2: Support and leverage existing national clinical and translational networks to conduct high-impact clinical research, clinical trials, and translational science and disseminate and implement successful interventions and treatments into the clinic and the community.

Translating a discovery into a therapy efficiently and working to ensure it reaches the people who need it benefits from a national ecosystem of clinical and translational research networks.

We will continue to support and coordinate the Clinical and Translational Science Awards (CTSA) Program and Rare Diseases Clinical Research Network (RDCRN). The CTSA and RDCRN continue to use their wide-reaching communities to develop, test, and share solutions for even broader-reaching impact. Both networks enable data sharing, collaboration, and training, which are all critical to enabling the translation of research observations into health interventions.

The CTSA Program includes a collaborative and efficient network of over 60 institutions and their partners working at the local, regional, and national levels. The CTSA Program infrastructure allows them to rapidly address public health priorities and conduct impactful clinical research, including developing and using innovative trial designs. It also allows them to support clinical trial readiness and train the next generation of clinical and translational scientists.

With support from multiple NIH ICs, and the Office of the Director, the RDCRN researchers partner with patient advocacy groups (PAGs) to conduct research and natural history studies and to develop clinical trial readiness studies for more than 280 rare diseases.

Objective 1-3: Support the discovery, development, and use of tools, technologies, models, methods, and assays for preclinical testing, drug development, and preclinical screening to enable identification of disease and to treat disease progression.

Many promising research discoveries in the laboratory face failure points along the therapeutic development path, resulting in a nearly 90% failure rate. Areas of risk leading to high failure rates include poor disease identification and targeting, assay sensitivity, and poor model prediction of toxicity and effectiveness.

We strive to increase the predictive value of research models by supporting the development and dissemination of more human, cell-based, physiologically relevant tissue and other nonanimal models. An early step in drug development is creating assays, or test systems, where researchers can study the effects of compounds of interest. Using existing assay technologies and developing new types of assays and tools and technologies to perform these assays can expand our understanding of diseases and aid in finding possible treatments. We will also further explore preclinical targets, methods and tools for testing, and screening approaches to complement assay research to identify more treatments or treatment avenues to pursue.

Objective 1-4: Advance biological and chemical discovery to identify new molecules and targets for potential treatments.

The development of potential drugs is traditionally a slow, nonautomated process that limits the number of avenues that can be explored and exploited for new treatment development.

We aim to make processes in drug development more rigorous, reproducible, data driven, and semiautonomous. These improvements will increase the likelihood of developing compounds that may treat diseases and will allow the creation of variations of effective chemical compounds more quickly. They also will expand treatments for the many diseases and conditions that lack therapies. We will call on multidisciplinary expertise, automation, and AI/ML to advance biological and chemical discoveries and translate them for therapeutic impact. We will also explore adding approaches that model molecular interactions at the quantum level to AI-based drug discovery to aid in the rapid and accurate design of new pharmaceutical compounds.

Goal 2: Enable All People to Contribute to and Benefit From Translational Science

Enabling all people to contribute to and benefit from translational science crosscuts everything we do at NCATS. Involving *all people* in translational science is a multidimensional strategy addressing health

disparities, inclusion in research and research design, broader community engagement, and translational science workforce training and development. This goal ensures that we engage with different communities to understand their health challenges and what is important to them. We will address issues of health disparities, inclusion, equity, access, and belonging in all aspects of our work. We will continue to encourage NCATS staff to participate in activities that raise our collective awareness and create a workplace where all individuals feel valued and secure in contributing to our mission. Embracing all backgrounds and perspectives is crucial to our collaborative and innovative methods.

Objective 2-1: Work toward broader inclusion of patients, their families and caregivers, and care providers as participants in translational science.

To ensure we achieve the NCATS mission and vision, it is crucial that we include our patients and communities as partners.

We will engage with patients, families, advocacy groups, and communities to ensure that study designs and goals reflect their perspectives and are meaningful to them. This engagement will begin during the preclinical stages and continue to the clinical space. We will continue to implement novel approaches to increase the inclusion of different individuals and perspectives in research and research design. Much work remains to be done, particularly in the preclinical space, to bring the patient perspective to the table. More inclusivity and broader representation in clinical research and clinical trials is essential to ensuring rigorous, reproducible research that translates to broader health care settings. NCATS will support strategies that respect the input and the value of the research participant.

Objective 2-2: Broaden engagement efforts and create more inclusive approaches that build trust and engage people at different levels to benefit translational science.

The participation of different types of communities and participants in research is valuable. The involvement of each requires different expectations, and each should be approached differently.

We will connect and build relationships to inform, shape, and improve translational science. While we already work in many ways to engage our constituents, we will strive to reach communities that have not been a part of our outreach and engagement efforts. Our CTSA program, particularly the Trial Innovation Network, engages patients and communities, bringing in perspectives to inform design and participation in clinical research, as well as meaningful outcomes that can impact patients at local, regional, national, and even global levels. The RDCRN include patients and PAGs as part of each research team, where they directly represent the perspectives and interests of patients with rare diseases. The PAGs also act as a coalition to advance the cause of rare diseases research and improve patient outcomes by sharing rare diseases information across the network and educating the community. We also will explore how to take our existing databases, tools, and resources to external communities in ways that build capacity and access to NCATS and its programs and activities. We will build trust by engaging at all levels with respect and humility.

Objective 2-3: Apply translational science to address health disparities and health inequities.

Many challenges contribute to health disparities and health inequities in biomedical research. Frequently, diseases can disproportionately affect specific populations. Also, social determinants

of health — such as socioeconomic status, demographic location, and gender — can lead to health inequities, leaving populations vulnerable and often with limited access to care.

We will improve the development of strategies that address health inequities in both the clinical and preclinical phases of translational research to ultimately benefit underserved or underrepresented populations. These groups include women, individuals across the life span, underrepresented minorities, rural residents, and individuals with disabilities. We must also consider the disparities experienced by individuals with rare diseases. Data sets, cell lines, and disease registries that represent the diversity of the population, either of the United States or those affected by a given disease, are critical for our research to be broadly applicable and minimize bias of results. NCATS' clinical research networks will be important for developing and applying strategies to address health disparities through research and increasing access to clinical trials. One important approach will use digital health technologies to support decentralized trials. CTSA-supported institutions have the capacity to test and apply solutions locally, regionally, and nationally. The RDCRN Consortia has the expertise to address issues of representation and access to care for its rare disease patient populations. Its approach may be adaptable to other programs and research activities in rare diseases.

Objective 2-4: Continue to develop and support efforts to cultivate a multifaceted, highly skilled, representative, and inclusive translational science workforce and help provide a clear and sustainable career path for translational scientists.

Building the translational science workforce at all stages of career development is essential to ensuring that all people are able to contribute to and benefit from translational science. A representative workforce contributes to the sharing of multiple perspectives, experiences, and backgrounds and fosters a culture of belonging that will enable NCATS and the translational science community to innovate and make progress.

We will continue to develop a highly skilled, representative, and inclusive translational science workforce, both within NCATS and more broadly. A core aspect of training in translational science is ensuring researchers and those working in the field understand the [principles of effective translational science](#). NCATS values cross-disciplinary training opportunities that foster team science. (For more on team science, see Goal 4.) We will look for opportunities to bring more individuals into the translational science pipeline and enable them to advance in their careers at NCATS or through our programs to the broader field of translational science. NCATS will also strive to include trainees and individuals in professions in clinical or scientific areas that may be new to translational science. Training and educating a representative workforce will include conducting strategic outreach and recruitment, implementing retention initiatives, and enhancing awareness of overall workforce opportunities.

Goal 3: Accelerate Translation by Addressing Both Scientific and Operational Challenges

Despite much progress, it still takes too long to turn an early discovery into a treatment, and even longer for that treatment to be adopted in clinical practice. Innovations in translational science and operations can make translational research faster, more effective, and more accessible. Scientific innovations, including broader incorporation of data science into research processes, can reveal new knowledge and spur advances more broadly. Operational efficiencies can reduce long-standing bottlenecks and help

teams align goals and objectives. This goal focuses on improving scientific and operational processes to enable more effective and efficient translation, which, in turn, will reduce the time required for treatments to reach patients. Our strategies include developing better predictive models, patient-centric designs, innovative trial designs for rare diseases, new approaches and methods to speed up the timeline for correct diagnoses, and streamlined research operations.

Objective 3-1: Remove, reduce, or bypass scientific and operational barriers that slow translation.

Numerous scientific and operational challenges in both preclinical and clinical areas can inhibit translation, including insufficient tools and technologies, one-size-fits-all approaches, and inflexible clinical trial designs.

We will use our scientific expertise in robotics and automation to streamline and speed laboratory operations. Doing so also will enhance rigor and reproducibility and allow us to apply platform technologies to many diseases and conditions. We will continue to work with collaborators in research, manufacturing, and regulatory oversight to address known roadblocks associated with gene- and cell-targeted therapy research and manufacturing. This cooperation will support applications that can be scaled to address multiple rare diseases and disorders. NCATS will continue to work closely with the CTSA Program research network to harmonize and streamline operations and enable faster activation of clinical trials and reporting. Together, we will develop and implement novel clinical trial designs and demonstrate network-wide readiness to address emerging public health needs.

Objective 3-2: Apply data science approaches to directly speed translation.

Data science and complementary approaches crosscut and underpin much of NCATS' work, removing and reducing the barriers in translation (Objective 4-2).

Our data science approaches use large and complex data set computation, connect different data sets, and support sharing and access to speed research. Approaches that support more rapid data exploration, reuse, linkage, and interpretation apply to many science disciplines. In the rare diseases community, in particular, broader data sharing enables the ability to learn as much as possible about specific rare diseases. These insights include learning about the genetic and cellular mechanisms that are the same in different diseases. Expanding the use of real-world data, such as electronic health records (EHRs), will inform clinical research, including trial design and recruitment. Another important area for NCATS to continue developing is informatics and data strategies that enable researchers to harness and bring together different data sources to speed understanding of natural history, diagnosis, and disease onset and progression and to point to possible treatment options and approaches.

Objective 3-3: Use innovative approaches to develop technologies and models that can accelerate diagnosis, support prevention, or minimize the impact of disease.

Access to and analysis of data, such as sequencing data and real-world data, can shorten the length of the diagnostic odyssey, which currently averages 6 years, experienced by many rare disease patients.

We will develop and apply innovative statistical and computational methods to linked data from EHRs, digital and mobile technologies, and other sources to discover new, previously unexplored, causal relationships that may prompt the identification of interventions. We will provide more rapid and accurate diagnosis of patients with hard-to-diagnose diseases by combining clinical consultation with machine assistance and genomic analyses. Different human-based cell models will be explored as better predictive models of health and disease, including rare diseases. These models also will be used in efforts to prevent disease and to create pathways that provide the data and knowledge needed to gain regulatory approval of treatments. In line with Goal 1, identifying commonalities among diseases and applying that knowledge when testing for and treating multiple diseases and conditions may speed prevention, diagnosis, and access to the right treatment(s).

Goal 4: Broadly Utilize Research and Operations Strategies That Cut Across Translational Science Efforts

Many challenges and opportunities in translational science are not unique to only one of the first three strategic goals in this plan. Several of our research objectives bridge all aspects of the work we do to reduce or eliminate translational science roadblocks. This goal highlights key crosscutting objectives that span all aspects of NCATS: team science, partnerships and collaborations, broader leveraging of data and data science, and education and outreach.

Objective 4-1: Support translation through team science.

[Team science](#) brings together individuals from different disciplines to work on a shared problem. This approach is integral to the field of translational science, is central to NCATS' culture and success in overcoming translational research barriers, and has enabled NIH-wide activities, such as the RDCRN, Tissue Chip for Drug Screening Program, and Accelerating Medicines Partnership® (AMP®) Bespoke Gene Therapy Consortium (BGTC).

NCATS will continue to foster collaborative opportunities that bring different disciplines together to advance translational science. This commitment includes engaging with our communities (both internal and external) to understand what additional expertise is needed to further the field of translational science and successfully address translational roadblocks. Cross-disciplinary teams, including those established through different types of partnerships (see Objective 4-2), build on the expertise of all partners to address complex translational science problems.

Objective 4-2: Develop novel and effective partnerships and collaborations in a variety of settings to foster translational science advances.

Innovative and strategic collaborations with scientists and other partners across the biomedical ecosystem support the translation of early-stage discoveries into medical interventions in meaningful ways.

We will continue to develop effective partnerships and collaborations through formal alliances, research agreements, and partnerships. Collaborating across our organization enables us to innovate by breaking down silos to foster more communication, finding efficiencies, and incorporating diverse perspectives. Collaborating externally with different partners — such as patients, caregivers/care partners, patient advocates, advocacy groups, impacted communities, the academic community, industry, other federal agencies, and

international organizations — will provide broad perspectives to advance translational science and ultimately benefit patients. Streamlining approaches to these partnerships and collaborations also helps speed translational science (Goal 3).

Objective 4-3: Apply data science strategies across the translational science spectrum to improve public health.

Strategies in data science advance the development of tools and technology, facilitating knowledge generation. Specific aspects of data science directly speed translation (Objective 3-2) and underlie much of the work NCATS tackles.

Five core themes in data science will drive the approaches we utilize and support: (1) data ingestion, management, and governance; (2) new technologies and new knowledge; (3) data reuse and interpretation; (4) open science and a collaborative culture; and (5) data science capacity building and workforce development. The privacy of individuals and the security of the data will always be a prime consideration in our data science efforts. To support the explosion of interest in AI/ML, NCATS will foster the development of good algorithmic practices and standardization of data formats to ensure that data sets are valuable, predictive, and representative, all with the overarching goal of minimizing bias. We will use state-of-the-art AI technology (including large language models), biostatistics, informatics, and newer efforts in quantum science to aid in innovative design of preclinical and clinical studies, and we will explore evidence and data to generate novel research approaches.

Objective 4-4: Communicate and raise awareness of the value and applications of translational science and its principles.

Although progress has been made in translational science, more awareness and understanding of translational science is needed. Many outside the field — both other researchers and the public — are unsure of what translational science is and are unclear of its value to them.

We will maximize the impact of the center's translational science efforts by creating and sharing resources, methodologies, and impact stories. We will increase public knowledge about translational science and its principles through scientific publications and communications outreach. We also will build relationships through different types of engagement. We welcome every opportunity to inform the public and research communities about how our research and support address long-standing challenges in translational research so that new treatments and other health solutions reach people faster.

Goal 5: Work Together as Stewards for Advancing Translational Science to Promote Transparency, Integrity, Accountability and Social Responsibility

Stewardship involves responsible planning and management of resources and is integral to our success, as well as meeting the goals in this strategic plan. NCATS applies stewardship to all activities and efforts to responsibly use funds and resources in support of achieving our mission and vision. It underpins our culture and all that we do. We are intentionally naming stewardship as a goal, with objectives that highlight additional areas integral to successful stewardship. Many stewardship efforts are internal to ensure that NCATS works efficiently, ethically, and responsibly to address scientific, operational, and policy barriers to translation.

Objective 5-1: Approach translational science research, operations, and other activities in an open, shareable, and transparent manner.

Transparency and open science are ways to promote reproducibility, progress, and trust in research and the people who conduct it.

We will expand internal and external processes for transparent priority setting, including the launch and sunset of activities (see the Priority Setting section below). We will also continue to actively partner with external organizations to promote a culture of openness, sharing, and transparency, including identifying synergies and collaborating to achieve shared missions. We will ensure our data science activities follow findable, accessible, interoperable, and reusable (FAIR) principles. We also will pursue a variety of strategies to disseminate information about our mission and activities, planning, priorities, methods, and best practices, with a focus on making outreach and communications more inclusive, informative, and accessible. We will foster an open and collaborative working environment with flexible approaches to best address and manage scientific, operational, and administrative change.

Objective 5-2: Support robust, reproducible translational science research and operations that consider societal and ethical impacts.

Rigor, reproducibility, and ethical approaches to designing and conducting experiments are the cornerstones of scientific research.

As reflected in the translational science principles, NCATS uses rigorous and robust methods to generate reproducible findings that contribute to advancing translation. We will strive to ensure that all scientific programs and activities are conducted in a rigorous, robust, and data-driven manner. We also must consider the ethical, legal, and societal impacts of supported research and other activities.

Objective 5-3: Advance understanding and solutions regarding how science policies can impact translation.

Barriers to translation can include policies at organizational, state, and government levels, such as operational processes for conducting clinical trials or screening and sequencing for rare diseases. The data generated from research and related activities can also inform policy development.

We will support, build, and disseminate strategies to foster policies for sharing information to enable research, while also establishing security protections that ensure individual privacy and consider cultural respect. Policies may need to be adapted to address intellectual property, technology transfer, and partnerships to enable translational science. We will seek to increase awareness among the translational science research community around the meaning and implications of various policies, and we will facilitate the transition from research to regulatory acceptance by understanding and providing information on research and regulatory policies.

Objective 5-4: Optimize resources and infrastructure to ensure efficient and effective stewardship in meeting the NCATS mission and vision and furthering the field of translational science.

Leveraging complementary expertise and tools reduces redundancy and ensures that the best science is being conducted in the most efficient manner.

We will continually assess and optimize operational and business practices internally to improve translational science. This effort includes implementation of strategic and operational planning activities and routine evaluation of activities, resources, and priorities to ensure their alignment with the NCATS mission.

Appendices

Appendix A: Additional Details on NCATS Organization

Statutory Authority

Establishment of NCATS

NCATS was created on Dec. 23, 2011, by the Consolidated Appropriations Act, 2012 (P.L. 112-74), which amended the Public Health Service (PHS) Act by including authorization language for NCATS. The 21st Century Cures Act (P.L. 114-255), which became law on Dec. 13, 2016, subsequently modified NCATS' authorization language.

The current PHS Act [authorization language for NCATS](#) includes the purpose of NCATS, the phases of clinical trials that may be supported, the NCATS biennial report, and the previously existing NIH programs that were moved to NCATS, such as the [Cures Acceleration Network \(CAN\)](#).

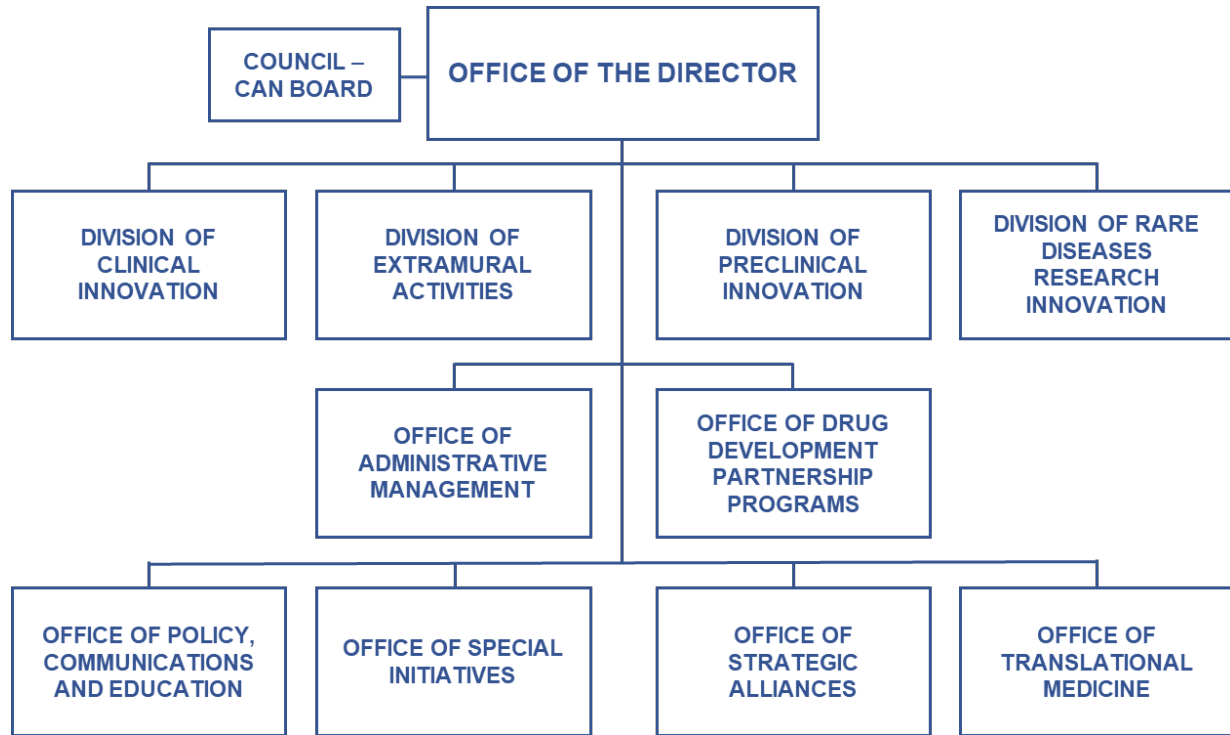
Cures Acceleration Network

CAN was established within NIH on March 23, 2010, by the Patient Protection and Affordable Care Act (P.L. 111-148), but it was not appropriated any funds. Several stakeholders wrote a [letter to Congress](#) (PDF — 35KB) on May 14, 2010, asking Congress to provide funding for CAN.

On Dec. 23, 2011, the Consolidated Appropriations Act, 2012, appropriated \$10 million for CAN and moved CAN to NCATS.

The purpose of CAN is to award grants and contracts to eligible entities to accelerate the development of high-need cures, including through the development of medical products and behavioral therapies.

Organizational Chart



Learn more about our staff at <https://ncats.nih.gov/about/our-staff>.

Research and related activities often cut across our organizational structure.

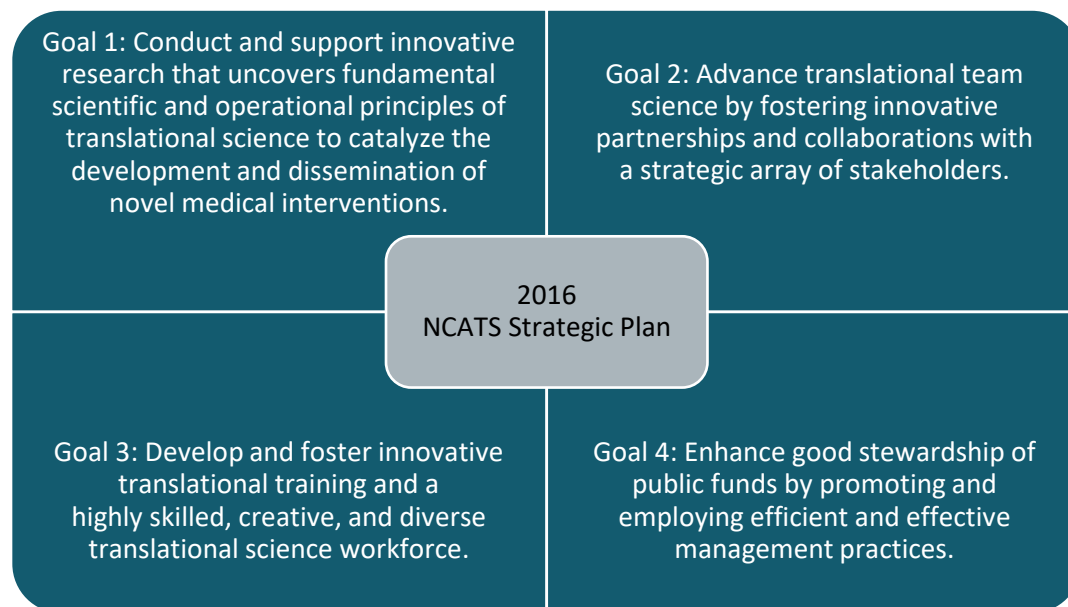
For more information on NCATS divisions and offices, visit <https://ncats.nih.gov/about/divisions-and-offices>.

For more information on NCATS research activities, visit <https://ncats.nih.gov/research/research-activities>.

Appendix B: Accomplishments From the 2016 NCATS Strategic Plan

The 2016 *NCATS Strategic Plan* was released five years after NCATS was established and set a roadmap for the center’s early activities. Over the subsequent years, NCATS has realized the potential envisioned in that strategic plan, becoming a recognized leader and innovator in the space of translational science. The release of the 2024 *NCATS Strategic Plan* offers an opportunity to reflect on and recognize the many ways in which the 2016 *NCATS Strategic Plan* goals were accomplished and how NCATS led the way in advancing translation science.

NCATS 2016 Strategic Plan Goals



NCATS has made progress in meeting the goals of the 2016 *NCATS Strategic Plan* in several key areas, highlighted below. Find more details and impact examples from these activities in the NCATS Congressional Justifications (found on the [Budget](#) page), [NCATS biennial reports](#), [NCATS Impact webpage](#), and [NCATS News & Events webpage](#).

Innovation in Clinical Trials (Goals 1, 2, and 3)

NCATS has played a leading role as an innovator in the conduct and efficiency of clinical trials. The Institutional Review Board (IRB) and recruitment and operations processes have been revitalized through the [Clinical and Translational Science Awards \(CTSA\) Streamlined, Multisite, Accelerated Resources for Trials IRB \(SMART IRB\)](#) Reliance Platform and the [Trial Innovation Network](#). Leveraging the scope and capabilities of the CTSA Program network enabled NCATS to rapidly respond to the COVID-19 pandemic through the Accelerating COVID-19 Therapeutic Interventions and Vaccines (ACTIV) trials ([ACTIV-1](#) and [ACTIV-6](#)). The [Rare Diseases Clinical Research Network \(RDCRN\)](#) has built a collaborative rare disease research network that has resulted in 12 U.S. Food and Drug Administration (FDA)–approved treatments for 11 of the diseases studied in this network.

Rare Disease Advancements ([Goals 2 and 4](#))

NCATS is a home for rare disease advancements that too often get stuck in the translational pipeline. The center raises awareness of the economic burden of rare diseases through activities like the [IDeaS \(Impact of Rare Diseases on Patients and Healthcare Systems\) study](#). NCATS also disseminates information and resources through the [Genetic and Rare Diseases \(GARD\) Information Center](#) and through partnerships established in programs like the [Accelerating Medicine Partnership® \(AMP®\) Bespoke Gene Therapy Consortium \(BGTC\)](#) and [Platform Vector Gene Therapy \(PaVe-GT\)](#). These efforts increase the potential to address many diseases a time.

Expansion of Training Opportunities in Translational Science ([Goal 3](#))

NCATS prides itself on providing robust training experiences for new translational scientists, whether through our numerous internal fellowship [opportunities](#) or [through various awards](#) to our external collaborators. Additionally, the NCATS Office of Strategic Alliances Training Opportunities provides training in the business areas surrounding translational science. Activities include internal lunch-and-learn programs that are open to all NCATS staff and trainees and the internal NCATS Advancing Innovation through Mentorship (AIM) program, which is fashioned after the [National Science Foundation iCorps program](#).

Harnessing the Power of Data Science ([Goals 1, 2, and 4](#))

At NCATS, [we have invested](#) in new ways to connect, access, and learn from large and complex data sets. We also create and use data tools and methods in new ways to speed translational research. Activities span the translational pipeline, from using platforms like [Biomedical Data Translator](#) and [OpenData Portal](#) to identify promising therapeutic candidates and avenues to harnessing the power of clinical data to address urgent public health needs through the [National COVID Cohort Collaborative \(N3C\)](#) and [CURE ID](#).

Developing and Utilizing Human Cell Models ([Goals 1 and 2](#))

NCATS is providing key advances in innovative systems to mimic diseases and test potential treatments in efficient and validated human cell models. We established key infrastructure, including the [Stem Cell Translational Laboratory](#) and [3-D Tissue Bioprinting Laboratory](#), as well as tools to work with these systems, like [Somatic Cell Genome Editing \(SCGE\)](#). In particular, our [Tissue Chips for Drug Screening Program](#) has led the development of 3-D platforms designed to represent human organ systems and mimic functions of the human body. Tissue chips have allowed the testing of treatments for [many diseases](#), including [addiction and pain](#) and [COVID-19](#), as well as understanding aging by sending our [Tissue Chips into space](#).

Increasing Awareness of Translational Science as a Discipline ([Goals 3 and 4](#))

One goal of our inaugural strategic plan was to increase awareness of translational science as a discipline. To this end, the NCATS Communications Branch developed and widely disseminated new resources that describe NCATS' work in crisp, clear, and compelling language. NCATS also developed the NCATS [Translational Science Principles](#), which stem in part from in-depth case studies and build on scholarship identifying core competencies for translational science.

Engaging and Facilitating Novel Partnerships ([Goals 2 and 4](#))

NCATS has led the way in showing how to establish and conduct successful collaborations through such programs as [A Specialized Platform for Innovative Research Exploration \(ASPIRE\)](#) and leadership in NIH Common Fund programs like the [Extracellular RNA Communication Program](#). NCATS also has established a new version of the standard scientific partnership agreement with the [innovative cooperative research collaboration agreement template](#). This template speeds the implementation of research agreements. Through NCATS' facilitation of partnerships, three major exclusive licenses are now in different stages of their life cycles.

Enhancing NCATS' Internal Processes ([Goal 4](#))

Stewardship of all NCATS activities was instrumental to achieving the goals of the 2016 *NCATS Strategic Plan*. During this time, NCATS developed a user-friendly internal human resources system — the K2 ProgressivE Enterprise Personnel System and workflow automation application. It is one example of how we enabled an administrative structure to align key operational support and infrastructure in support of the NCATS mission. NCATS has also established a transparent and proactive center-wide operations planning process. To improve the awards management process, the NCATS Division of Extramural Activities (DEA) led the effort to establish [Other Transaction Awards](#) as a new business line at the NIH level, which is critical to the conduct of the [Cures Acceleration Network](#). DEA established prize competitions as a new mechanism for funding and developed best practices for engaging NIH institutes and centers as partners, instead of as a service center, as exemplified by the partnership with the National Institute on Drug Abuse on managing the Native Collective Research Effort to Enhance Wellness (N CREW) Program, under the NIH Helping to End Addiction Long-term® Initiative, or HEAL Initiative®.

Achieving Crucial Regulatory Milestones ([Goals 1 and 2](#))

Advancing treatments through the translational pipeline requires navigating the regulatory process of therapeutic approval. NCATS has engaged in NIH-wide partnerships to develop novel therapies to the point of clinical research trial readiness, and the FDA has approved 64 Investigational New Drug applications based on NCATS preclinical research. The preclinical research conducted by NCATS was also key to de-risking the clinical trials leading to the approval of three drugs.

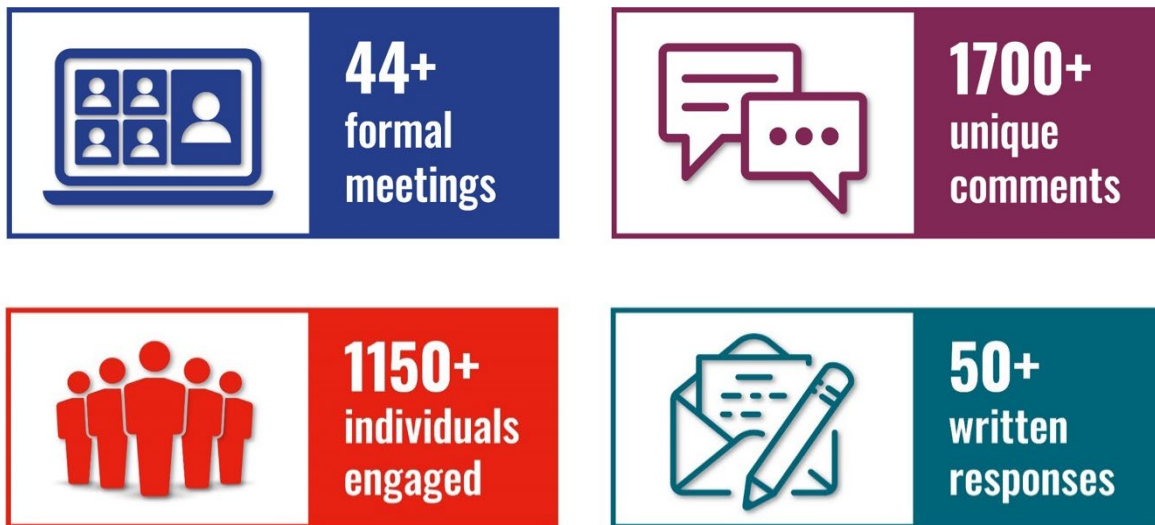
Appendix C: Strategic Planning Process

Ongoing Community Engagement

From January 2023 to August 2023, NCATS Director Dr. Joni Rutter and the Strategic Planning Team participated in over 40 meetings with more than 1,150 individuals. These discussions gathered input on the future of NCATS activities, unmet needs in translation, and opportunities to make progress toward bringing new health solutions to people faster:

- Nineteen virtual discussions were held, involving more than 350 NCATS staff members.
- NCATS hosted two virtual [strategic planning roundtable discussions](#) on May 9 and 10, 2023. During these discussions, Dr. Rutter gave an overview of her vision for NCATS and information on the strategic plan. Participants then met in breakout groups to offer their perspectives on four translational science areas: Research; Training and Education; Patient and Community Engagement; and DEIA (Diversity, Equity, Inclusion, and Accessibility). All breakout sessions answered the same four broad, open-ended questions to solicit input.
- In addition, Dr. Rutter presented the strategic plan at 24 virtual meetings as part of her engagement of the Clinical and Translational Science Awards Program committees and at two meetings with researchers and advocacy groups who are part of the Rare Diseases Clinical Research Network. These conversations included questions tailored for each particular group.
- The NCATS Advisory Council discussed and gave input on the strategic plan at their meetings in May 2023, January 2024, and May 2024.

We routinely publicly promoted an email address for additional comments or questions.



We continue to engage our constituents as part of our strategic plan implementation and will develop a specific plan for ongoing engagement to keep internal and external groups apprised of our activities and progress.

Framework Development

Based on a set of relevant, recurring themes identified from the feedback, we categorized the more than 1,700 comments received across strategic engagements. Using this semiquantitative analysis of the input received, we developed a draft framework for the 2024–2029 *NCATS Strategic Plan*. The framework presented five strategic plan goals, a brief narrative context of each goal, and a set of potential themes matched to the NCATS vision.

Framework Input

We published a request for information (RFI) ([NOT-TR-23-027](#)) in the NIH Guide Notice in September 2023. The RFI asked for feedback on the draft goals and plans presented in the framework. Fifty-one separate responses were received.

During the period the RFI was open for comments, Dr. Rutter held three small-group discussions with other NIH institute and center directors. In addition, the Strategic Planning team presented the draft framework at an NCATS town hall and held five virtual discussions, one for each goal, with NCATS staff.

Document Drafting and Refinement

Following external and internal feedback on the draft framework, the Strategic Planning team drafted objectives. The goals and objectives were further discussed with NCATS leadership. The full strategic plan draft was published for 30 days on the NCATS website and discussed with the NCATS Advisory Council in May 2024.

Appendix D: Implementation of the Strategic Plan

The 2024 *NCATS Strategic Plan* sets forth a vision with five goals, each with objectives, for implementation. By continuously evaluating and adapting our strategies, we are committed to making a significant impact on advancing translational science and improving health outcomes. We will provide regular updates on progress.

As the 2024 *NCATS Strategic Plan* was developed, all divisions and offices were involved in providing feedback, ensuring center-wide consultation. After the 2024 *NCATS Strategic Plan* is launched, divisions and offices will identify how their current and upcoming activities fit into the goals and objectives. In addition, an individual, committee, or working group will be formed to assist in implementing each objective.

To start, the 2024 *NCATS Strategic Plan* will be used as a reference point for guiding all programs and initiatives that NCATS implements going forward. For example, operations planning is an NCATS-wide internal activity during which new ideas are brainstormed and discussed by NCATS staff and leadership for transparency, collaboration, coordination, and priority setting. As part of this process, staff submitting new ideas will identify the relevant 2024 strategic goal(s) and objective(s) in their submissions. Concept clearances for the NCATS Advisory Council will also require the same information to be provided.

We have begun to identify metrics to measure the progress of the strategic plan's goals and objectives. This process will apply a variety of approaches to systematically monitor and collect accomplishments and other indicators of progress. Additionally, NCATS' activities and programs, including those published on the NCATS website, will be collected in a coordinated way and assigned to the relevant strategic plan goals and objectives. NCATS will continue discussions with our constituents to inform how we think about what is impactful in terms of progress in meeting our mission and vision.

We will continue to assess how our implementation aligns with that plan and other NIH-wide plans, including the [*NIH-Wide Strategic Plan for Diversity, Equity, Inclusion, and Accessibility \(DEIA\)*](#); [*NIH Strategic Plan for Data Science*](#); and [*NIH-Wide Strategic Plan for Women's Health Research*](#).

Appendix E: Acronym List

ACTIV	Accelerating COVID-19 Therapeutic Interventions and Vaccines
AI	artificial intelligence
AIM	Advancing Innovation through Mentorship
AMP®	Accelerating Medicines Partnership®
ASPIRE	A Specialized Platform for Innovative Research Exploration
BGTC	Bespoke Gene Therapy Consortium
CAN	Cures Acceleration Network
CTSA	Clinical and Translational Science Awards
DEA	Division of Extramural Activities
DEIA	Diversity, Equity, Inclusion, and Accessibility
EHR	electronic health record
FAIR	findable, accessible, interoperable, and reusable
FDA	U.S. Food and Drug Administration
GARD	Genetic and Rare Diseases Information Center
ICs	institutes and centers
IDeaS	Impact of Rare Diseases on Patients and Healthcare Systems
IRB	Institutional Review Board
ML	machine learning
N3C	National Covid Cohort Collaborative
NCATS	National Center for Advancing Translational Sciences
NIH	National Institutes of Health
PAG	patient advocacy group
PaVe-GT	Platform Vector Gene Therapy
PHS	Public Health Service
RDCRN	Rare Diseases Clinical Research Network
RFI	request for information
SCGE	Somatic Cell Genome Editing
SCTL	Stem Cell Translational Laboratory
SMART IRB	Streamlined, Multisite, Accelerated Resources for Trials IRB Reliance