2025 — 2030
NCATS Strategic Plan

National Center
for Advancing
Translational Sciences

Read the online version of the
NCATS Strategic Plan for 2025–2030:

ncats.nih.gov/about/ncats-overview/strategic-plan

Director's Message

Joni L. Rutter, Ph.D.

At NCATS' 10-year anniversary event in 2021, I presented a vision for the center: a future with more treatments for all people more quickly. That transformative 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, steeped in the successes of the past decade, are vital to achieving this vision. The *NCATS Strategic Plan for 2025–2030* 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, including patient and community voices. Their input came during more than 40 roundtable discussions and through a public request for information.

To translate science into practice more effectively, we must address these key needs:

1. To increase the pace of development and availability of treatments
2. To enable more individuals and communities to contribute to and benefit from translational science
3. To identify and address inefficiencies in translation that slow and even stop research efforts

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

As with the previous strategic plan, patients and communities are at the center of our mission and our work. 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 directions.

Our strategic plan is a living document. As we implement the plan, we will continuously assess and adapt our strategies, checking in with ourselves and our communities. We have built the plan to be flexible in order to handle new challenges, opportunities, and innovations as they emerge.

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.

Joni L. Rutter, Ph.D.
Director
National Center for Advancing Translational Sciences

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Overview of NCATS

NCATS' Mission and Vision

NCATS' mission is to turn research observations into health solutions through translational science. We work to develop or enhance the development, testing, and implementation of diagnostics and therapeutics for a wide range of diseases and conditions. Key approaches include understanding what's similar across diseases to spur multiple treatments at a time, developing models that better predict a person's reaction to treatment, enhancing clinical trials so results more accurately reflect the patient population, and leveraging real-world data and data science approaches to address public health needs. Facilitating these approaches are our robust partnerships with other government agencies, including other NIH institutes, centers, and offices; industry; academia; nonprofit organizations; and patients, patient advocates, and other communities.

NCATS' vision is to bring more treatments for all people more quickly.

Relevant Mandates and History

NCATS was created on December 23, 2011. Find full details on NCATS' Statutory Authority in Appendix A.

NCATS Organization

The divisions and offices within NCATS span the spectrum of preclinical and clinical translational science. (See Appendix A for the organizational chart.) Working together across the organization, we develop and implement plans for new opportunities. We build tools and technologies, support research, train scientists, and much more. As a highly collaborative organization, our work often cuts across our organizational structure, and our patient-driven focus is ever present.

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. We apply many approaches and strategies to reduce bottlenecks in the research pipeline that slow medical progress. Though we are a home for rare diseases, we do not focus on specific diseases and conditions, rather we focus on approaches that can address many diseases at a time.

Our work has sped health solutions in many ways. Examples include data platforms that connected existing information in useful ways, technologies that aided a national response to a public health crisis, and innovative approaches that moved treatments into the clinic. (See Appendix B for more information about our progress, as well as key accomplishments mapped to our 2016 *NCATS Strategic Plan*.)

NCATS' Culture and Core Values

NCATS values a culture that works hard towards achieving its mission. We invest in initiatives to improve health and address health disparities. Our programs leverage NCATS resources to collaboratively 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 core values (Figure 1). 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.

Figure 1. NCATS' core values.

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.

Translational science is the field that generates scientific and operational innovations that overcome long-standing challenges along the translational research pipeline. By advancing translational science, we make research more efficient and impactful. NCATS identified seven principles that underpin effective translational science and are seen across the center's projects and programs (Figure 2). These NCATS Translational Science Principles are intentionally broad and foundational to NCATS' efforts — such as tissue chips, drug repurposing, gene-targeted therapy approaches for rare diseases, and the Clinical and Translational Science Awards (CTSA) Trial Innovation Network (TIN).

Figure 2. The Translational Science Principles developed by NCATS, which characterize effective translational science approaches.

NCATS' Audacious Goals

In 2021, NCATS Director Joni Rutter, Ph.D., announced audacious goals that align with the vision of more treatments for all people more quickly (Figure 3).

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

Figure 3. NCATS' audacious goals, which are aspirational and intended to inspire us to be bold with our ideas and solutions over the next 10 years.

Overview of the *NCATS Strategic Plan for 2025–2030*

This new strategic plan sets the direction to further innovate and advance translational science in order to meet public health needs. The plan's five goals provide a way for us to collectively and tangibly make progress. Each goal is followed by several objectives and approaches for implementing them. Goals 1, 2, and 3 align directly with the three elements of NCATS' vision: More Treatments, For 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.

Strategic Planning Process

The strategic planning process included deliberate, extensive engagements with multidisciplinary constituents on how and what NCATS should prioritize to support the center's vision. We engaged over 1,150 individuals from academic institutions, advocacy organizations, industry, and the general public as well as patients through more than 44 roundtable discussions over a year of both external community and internal staff engagements. Find details on the engagement activities undertaken during the strategic planning process in Appendix C.

Approach to Priority Setting

Priority setting at NCATS, as a whole, is essential to recognize programs, projects, and other activities that are already addressing or ready to address gaps and opportunities. It also enables us to identify projects that should sunset or transition away from federal funding for further development or application — by industry, for example.

NCATS sets strategic priorities in a transparent and inclusive manner. This is illustrated by the strategic planning process that enabled inclusive discourse through multiple feedback opportunities. External community members and internal staff provided valuable perspectives about important areas for NCATS to prioritize in its strategic plan. Recommendations included sunsetting or transitioning NCATS activities and identifying opportunities derived from successful programs and projects, new directions, and translational challenges that have yet to be addressed.

In addition to the strategic planning process, part of our ongoing operational priority setting includes regularly engaging our communities to understand their needs and ways our translational science approaches could solve

them. Activities include convening workshops, workgroups, or listening sessions; gathering input across NCATS, NIH, and external communities; and soliciting input from the public. Our approaches to prioritization span NCATS intramural and extramural programs and include a robust internal operational planning process that occurs approximately four times each year. 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. Extramural research undergoes a two-stage peer review process, which further informs how the priorities outlined in a strategic plan are implemented. For intramural activities, our Division of Preclinical Innovation systematically identifies opportunities for new and innovative approaches within its research programs. The intramural *ad hoc* scientific review involves external expert input every four years on NCATS' progress and direction.

Implementation of the Strategic Plan

The *NCATS Strategic Plan for 2025–2030* sets forth five goals, each with specific objectives and high-level approaches that will guide implementation. Implementation will be coordinated across the center through the immediate Office of the NCATS Director. It will include prioritizing activities to pursue, as well as putting in place a strategy to track progress, develop metrics, and keep our communities informed as we evaluate our progress. For details on implementing the strategic plan, see Appendix D.

Strategic Priorities

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 very few treatments exist. In fact, of the approximately 10,000 rare diseases, only 5% have treatments. Health care expenses for Americans with rare diseases are three to five times greater than for those without rare diseases, resulting in a total economic burden approaching $1 trillion annually. By advancing the development of more treatments, NCATS is on a mission to change the direction of research, particularly for rare diseases and diseases with unmet needs.

We're committed to expanding capacity to develop faster and more accurate diagnoses, along with new treatments to help offset this public health challenge. We aim to revolutionize research, treatment, and diagnostic tools for diseases with unmet needs, especially rare and intractable conditions. We use a variety of approaches, including enhancing preclinical screening, refining clinical research methods, finding new indications for existing medications, and advancing personalized medicine. We are tackling common research challenges with innovative standardized platform approaches that streamline research and development processes and can be applied to multiple diseases. A platform approach or platform technology is intentionally built to be used in multiple ways. NCATS-enabled platforms include human cell–based models to better predict drug response (e.g., tissue chips and organoids), gene-targeted therapies using delivery approaches that can address many rare diseases at a time, and high-throughput screening to find new uses for existing drugs.

Our goal is clear: Increase the number of treatable diseases by increasing knowledge about their underlying causes, supporting the development of new treatments, and improving the reach, accessibility, and uptake of existing treatments. Imperative in this work is pursuing effective dissemination and implementation strategies that aim to bridge the gap between research and clinical practice.

Objective 1-1: Prioritize efforts to advance diagnosis and targeted interventions for multiple diseases, particularly rare diseases and others with unmet needs.

Developing one drug for one disease is inefficient, particularly with the extremely small number of approved treatments reaching patients per year.

We can address this challenge by investing in efforts to develop scientific platforms and tools that can address multiple diseases simultaneously. By creating diagnostic or research tools or interventions for one disease that can be easily adapted for others, we aim to make the research process more efficient and broadly applicable.

Strategies to accomplish this objective include finding new or additional uses for existing compounds and approved drugs, also called repurposing. We will also advance methods that connect different types of data in meaningful ways, resulting in identifying more treatment options. We will also explore novel treatment approaches, such as gene- and cell-targeted therapies, that can be used as a platform to study multiple diseases.

Objective 1-2: Advance biological and chemical discovery to identify new molecules and targets for potential treatments.

The development of potential medications is a long and difficult process that often results in only a few treatments for the many diseases that need them. Many diseases and conditions are currently untreatable because they involve complex issues, like proteins that don't work correctly, or because the places to treat in the body where they are treated are difficult to reach by certain cell components and small molecule therapeutics. NCATS will create novel approaches for these challenging conditions, opening the door to new possibilities beyond traditional drug discovery methods.

In the NCATS laboratories, we aim to make processes in drug discovery more rigorous, reproducible, data driven, and semiautonomous. Doing so allows effective chemical compound discovery, testing, and development for more diseases more quickly. As part of this objective, we will combine multidisciplinary expertise, automation, and artificial intelligence (AI)/machine learning (ML) to advance biological and chemical discoveries and translate them for therapeutic impact, leveraging both NCATS labs and support of external research organizations. Also, through our NCATS labs and other research programs and activities we support, we will explore quantum methods in AI-based drug discovery, aiding in the rapid and accurate design of new therapeutic compounds from start to finish.

Objective 1-3: 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.

Clinical research and trials are a critical step to getting more treatments to those who need them. However, the barriers to success can be high. Barriers include the time to develop a trial, get approval to conduct it, and identify, recruit, and retain participants.

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 ready and able to innovate in study design and recruitment strategies. We will support, coordinate, and bolster our flagship CTSA Program and the Rare Diseases Clinical Research Network (RDCRN). Both networks enable data sharing, collaboration, patient and community engagement, and training, which are all critical to turning promising 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 is built upon CTSA institutions'

relationships at the community level, which allows them to rapidly address public health priorities and conduct impactful clinical research. For example, the Pain Management Effectiveness Research Network, a multisite research cooperative program through the NIH Helping to End Addiction Long-term (HEAL) Initiative®, is using CTSA TIN infrastructure to support clinical trials that compare the effectiveness of existing nonaddictive therapies and novel approaches for prevention and management of pain to advance new treatments for the opioid crisis. This CTSA infrastructure also includes developing and using innovative trial designs, real-world data, and analytics, such as electronic health records research; prioritizing research to address rural health and women's health; and developing ways to apply what is learned across the CTSA network and in primary care settings. Importantly, the CTSAs train the next generation of clinical and translational scientists to meet the research needs ahead.

Led by NCATS and with support from multiple NIH institutes and centers and the Office of the Director, RDCRN researchers and patient advocacy groups (PAGs) enhance clinical trial readiness in the rare diseases research community. They support data sharing and leverage the regulatory flexibility to conduct research, such as biomarker development and natural history studies. These efforts will be leveraged for future clinical trials and contribute to new drug approvals for rare diseases.

Objective 1-4: Support tools and technologies for preclinical testing and drug development.

Nearly 90% of promising treatment candidates that enter clinical trials fail. To improve success rates, we need more robust and predictive preclinical approaches, ensuring potential therapies that are likely to fail do so earlier, while those with real promise have a better chance of succeeding in late-stage clinical trials.

We strive to improve preclinical research models by developing more human cell–based, physiologically relevant tissue and other non-animal models. This approach will increase the potential of those models to better predict the safety and efficacy of potential treatments in humans. In addition, an early step in drug development is creating assays, or test systems, where researchers can study the effects of compounds of interest or identify underlying molecular causes of disease. For example, using induced pluripotent stem cells either from patients with rare diseases or normal cells CRISPR-edited with rare disease mutations, we can create models that help improve our understanding of the causes of diseases, identify new targets and biomarkers, and test potential therapeutics to find possible new treatments. Another example is the use of AI/ML for predicting novel molecular structures as drug candidates, assessing safety early by analyzing chemical structures and biological data, and identifying adverse effects early in the process. These AI tools can serve as reliable indicators in preclinical data and aid further development stages.

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 across every area we support. It involves designing research with participant and community input. We want to engage with communities to understand their needs. It is imperative we foster inclusion of research participants who adequately reflect the community with the disease or condition being studied. Programs like the CTSA TIN and the RDCRN support research networks that involve patients and patient advocates in research activities. The TIN also works across the CTSA Program to innovate in trial design to ensure broader access to participate in research.

NCATS is a values-based, people-centric organization committed to creating a workplace where everyone can contribute to solving major health challenges. We foster a bold, creative, and innovative environment where our staff can grow, thrive, and contribute to our mission. We work to ensure that the translational science workforce at NCATS and beyond is trained, mentored, and developed across the career spectrum to reflect these values.

Objective 2-1: Broaden inclusion of participants in translational science research.

Having research participants who do not adequately reflect the population with the disease or condition under study can create barriers to successful translation. Challenges in the recruitment of participants in research can lead to new treatments that don't work for the people who need them most. Research that reflects the real world is critical to achieving our vision. Through new and existing programs, we will explore ways to increase health literacy among potential participants and convey the importance of taking part in research. Through the CTSA Program, and specifically the TIN, we will continue to identify opportunities to build relationships at the local level and increase awareness of research opportunities within communities and through clinical care. Novel clinical trial designs, such as decentralized clinical trials, will be used to increase access to research.

Much work remains to be done to bring the individual participant perspective to the table. More involvement in preclinical research and in clinical research and clinical trials is essential to ensuring impactful findings and rigorous, reproducible research that translates to different health care settings so that needed treatments can reach people faster.

Objective 2-2: Expand engagement of relevant communities as partners in translational science to develop shared understandings of needs that inform research approaches that benefit these communities.

Lack of community engagement also negatively impacts the translation of research observations into health solutions. We can change that by engaging communities on how translational research may benefit them and how they can inform translational science to ensure research outcomes are more meaningful. To nurture trust and trustworthiness, we must engage communities with respect and humility. The CTSA TIN engages patients and communities to inform clinical research design and participation so that the results can benefit communities. Each CTSA Program institution has established a community advisory board (CAB) to understand what is needed locally to improve health in their communities and neighborhoods. For example, many CTSAs and their CABs have teamed with local faith-based and social services leaders in their communities. These collaborations increase trust and participation in clinical trials because the research community becomes a consistent and dependable resource, demonstrating a sustained commitment. Another example is inviting patient communities to participate in workshops, funding announcements, or prize competitions and other programs and activities NCATS undertakes.

In rare diseases research, unique approaches to engaging communities are needed, particularly given the size of patient populations and the challenges in diagnosis. In the NCATS laboratories, we plan to build on successful efforts that have engaged rare disease patients, families, and care providers to inform the development of treatments starting at the preclinical stage. Also, the RDCRN actively includes patients and PAGs as part of each research team, where they directly represent the perspectives and interests of patients with rare diseases. The PAGs act as a coalition to advance rare diseases research and improve patient outcomes by sharing rare diseases information with the research community and educating them. We will explore ways external communities can use our existing databases, tools, and resources to build capacity and advance their priorities.

Objective 2-3: Accounting for health disparities in designing and implementing translational science.

Many factors contribute to health disparities, including socioeconomic status and geographic location. We consider health disparities in the development of solutions for targeted and effective interventions to improve overall population health.

NCATS will support the development of strategies that address health disparities across the translational spectrum to ultimately benefit populations underserved or underrepresented in biomedical research or at higher risk for specific diseases or conditions. We must also consider the disparities experienced by individuals with rare diseases. Data sets, cell lines, and disease registries that represent the population, either of the United States or those affected by a given disease, are critical for our research to be broadly applicable. They also minimize bias of results and enable rigorous research designs. NCATS' clinical research networks, such as the CTSAs and RDCRNs, will be important for developing and applying strategies to address health disparities through community-informed research, improve recruitment approaches, and increase access to clinical trials.

For example, one important approach will be to use digital health technologies to support decentralized clinical trials, particularly to reach rural populations. CTSA-supported institutions have the capacity to test and apply solutions locally, regionally, and nationally, all of which were necessary to meet the changing needs at each of those population levels. The RDCRN Program has the expertise to address issues of study enrollment 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: Cultivate a multifaceted and highly skilled translational science workforce.

The representative translational science workforce fosters a culture of belonging and feeds innovation and progress. We are committed to developing and maintaining a highly skilled translational science workforce, both within NCATS and more broadly through the training and career development programs we support. Establishing on-ramps into the workforce and encouraging team science that includes all allied health professionals creates more opportunities for individuals to contribute to successful translational science. Training and educating a strong translational science workforce also involves conducting strategic outreach and recruitment, implementing retention initiatives, and enhancing awareness of overall workforce opportunities in translational science.

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, we 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. Our approach includes expanding and disseminating templated agreements to enable faster study start-up and providing resources like the Toolkit for Patient-Focused Therapy Development to involve patients and communities in research. We will automate routine tasks to save time and reduce errors, as well as invest in advanced collaboration tools to enhance communication and coordination among research teams. Additionally, we will optimize data management processes to ensure high-quality, FAIR (findable, accessible, interoperable, reusable) data and develop standardized protocols to maintain consistency and efficiency across studies. Working closely with regulatory bodies will further streamline approval processes and reduce delays.

Objective 3-1: Innovate to address scientific and operational processes that slow translation.

Numerous scientific and operational challenges can slow translation. They include inefficient processes, technologies, and inflexible clinical trial designs that cannot meet the challenges of the clinical trials of today, much less tomorrow.

We will streamline and improve research operations to speed translation to many diseases and conditions at a time and enhance rigor and reproducibility. Expanding collaborations and partnerships with research, manufacturing, and regulatory scientists will reduce roadblocks associated with translating findings to health solutions. For example, partnerships and collaborations are critical for certain aspects of gene- and cell-targeted therapy research, such as finding solutions to the adeno-associated virus (AAV) platform for manufacturing and regulatory challenges. We will also continue to work closely with the rare diseases and CTSA Program research communities to harmonize and streamline operations, develop standardized master protocols to enhance clinical trial comparability and rigor, and enable faster activation of clinical trials and reporting to deliver clinically relevant information to health care providers sooner than traditional methods. 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 speed translation.

Traditional methods for accumulating and accessing data are siloed and inefficient, which can slow translation. Leveraging data science approaches is key to speeding translation.

Our data science approaches support more rapid data aggregation, exploration, reuse, linkage, and interpretation, and we apply them across the translational research spectrum. For example, in the rare diseases community, broader data sharing enables translation of learnings from one rare disease to another. It also enables the ability to aggregate knowledge from many small communities to maximize insights and knowledge. These insights include learning about the genetic and cellular mechanisms that are the same in different diseases, improving care for patients with different diseases that have similar characteristics, and identifying potential drug repurposing opportunities. In addition, expanding the use of real-world data, such as electronic health records (EHRs), will inform different aspects of clinical research, including biomarker identification, trial design, and participant recruitment. Expanding informatics and data strategies that make data sources more interoperable allow researchers to harness real-world evidence to speed the understanding of disease onset and progression and possible prevention and treatment opportunities.

Objective 3-3: Develop innovative technologies and models in translation to achieve faster diagnosis and treatment.

Generating data, tools, and technologies needed for treatments to get regulatory approval is critical and time-consuming. Harnessing new scientific discoveries and innovation can help streamline translation and shorten the time it takes to get a diagnosis, find the right patient cohorts, or identify a potential treatment, thereby preventing or reducing the impact of rare and chronic diseases on patients. This will also enable the application of platform technologies to many diseases and conditions.

We consistently work to develop and automate technologies that can speed the creation, analysis, screening, or testing of different compounds or drugs for multiple diseases. By applying innovative statistical and computational methods to link data from EHRs, digital and mobile technologies, and other sources, researchers can discover new, previously unexplored, causal relationships that enable the identification of prevention or treatment approaches. By combining clinical consultation with AI/ML and -omics analyses, we will shorten the diagnostic odyssey of hard-to-diagnose, often rare, diseases. Also, by using human cell–based models as predictive tools and working with the FDA and others during the development and validation process, we can streamline regulatory acceptance of new approaches and new treatments. In line with Goal 1, we can also speed up prevention, diagnosis, and access to effective treatments by identifying commonalities among diseases.

Goal 4: Leverage Crosscutting Strategies to Enhance Translational Science

Translational science faces challenges and opportunities in several areas that cross other strategic plan goals and serve as the foundation used to achieve other objectives. NCATS will continue to leverage cross-cutting approaches to enhance the 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 our reach and resources. Embracing data science and facilitating FAIR data will unlock new insights and accelerate discoveries. Finally, our commitment to education and outreach ensures that we are continuously learning, growing, and engaging with the communities we serve.

Objective 4-1: Support and promote team science.

As a principle of translational science, team science brings together individuals with different areas of expertise across professions and different backgrounds to work on a shared problem and produce research that advances translation. Team science is a core part of NCATS' culture that has enabled highly collaborative efforts, such as the RDCRN, National Clinical Cohort Collaborative (N3C), Tissue Chip for Drug Screening Program, Biomedical Data Translator, Accelerating Medicines Partnership® (AMP®) Bespoke Gene Therapy Consortium (BGTC), and the Platform Vector Gene Therapy (PaVe-GT) program, to name a few. Team science also provides a fertile ground for enhancing training and mentorship opportunities. We will promote these opportunities through training and career development activities at NCATS and partner institutions, such as those funded through the CTSA Program.

We will continue to foster collaborative opportunities that bring people with different areas of expertise and backgrounds together to successfully address translational roadblocks. For example, successful clinical trials require a range of roles, such as nurses, pharmacists, coordinators, project managers, statisticians, and community workers, in addition to patient and community perspectives. Cross-disciplinary teams and partnerships leverage all partners' expertise to solve complex translational science problems as expanded on in Objective 4-2. Hiring from or engaging with our communities and incorporating their input is also needed to further the field of translational science and, more importantly, ensure the results can benefit all.

Objective 4-2: Develop effective partnerships and collaborations.

Without partnerships and collaboration, research can be stifled and siloed, slowing translation and resulting in missed opportunities. We can break down siloes in translation through innovative and strategic collaborations with partners across the biomedical ecosystem.

We will continue to develop effective partnerships and collaborations through numerous channels, including formal strategic alliances, research agreements, and other types of partnerships. Collaborating with patients,

caregivers/care partners, patient advocates, advocacy and professional nonprofit groups, communities, academia, industry, and other federal agencies will offer broad perspectives to advance translational science, find efficiencies, and ultimately benefit patients.

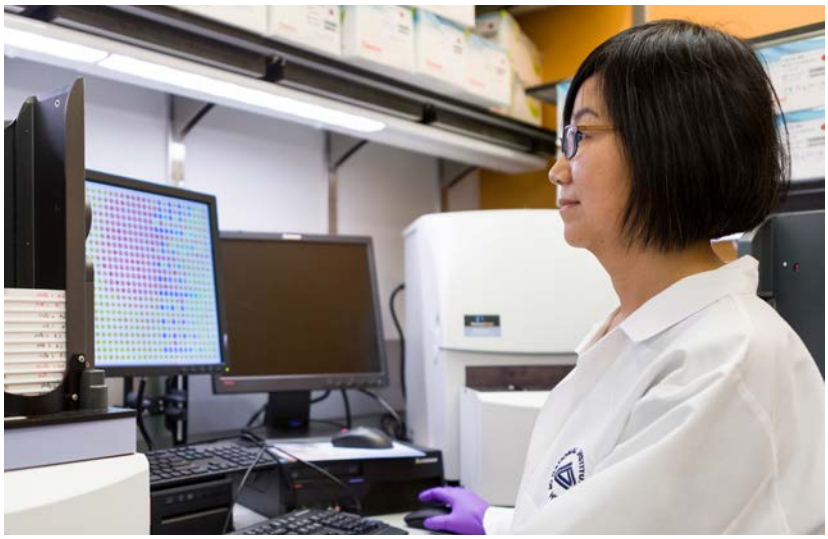
For example, NCATS led the ACTIV-6 decentralized platform clinical trial, which involved numerous partners and leveraged the CTSA Program infrastructure and collaborations to enroll participants from all 50 states and territories. Enrollment of hard-to-reach groups increased steadily over time. The study completed enrollment with unprecedented speed: Each arm completed enrollment in less than 10 months, and results were posted within 15 months from day one of a given treatment opening for participant enrollment. As a result, clinically relevant information reached doctors and patients more quickly than traditional methods. We will learn from and expand on this success.

Objective 4-3: Promote data science strategies and standards.

While specific aspects of data science will directly speed translation (see Objective 3-2), more broadly, a variety of data science approaches underlie and cut across much of NCATS' work. Improving and promoting strategies in data science that facilitate knowledge generation is a key objective of the next five years.

Six core themes in data science will serve as a roadmap and drive the approaches we utilize and support: (1) data stewardship and sustainability; (2) data ingestion, management, and governance; (3) new technologies for expanded knowledge; (4) data reuse and interpretation; (5) open science and a collaborative culture; and (6) data science capacity building and workforce development. The privacy of individuals, their control and ownership of their data, and the security of that data will always be a top priority in our data science efforts. To support growing interest in AI/ML for research, NCATS will apply and foster the development of standardized practices and data formats to ensure that data sets are valuable and predictive, all with the overarching goal of ensuring good-quality data sets, analytic methods, and the interpretation and use of results. We will use state-of-the art AI technology (including large language models), biostatistics, informatics, and newer efforts in quantum science to aid in every step of the translational process. Specific areas include the innovative design of preclinical and clinical studies and preventative care strategies.

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



Translational science is an engine that drives scientific research to clinical practice, yet it is not well understood by those outside the field. More awareness of translational science is needed to convey its value in addressing long-standing research challenges.

We will enhance translational science efforts by creating and sharing resources, methodologies, and impact stories. We will increase knowledge about translational science through scientific publications, as well as other communication and outreach activities intended to reach audiences beyond the research community. We also will build relationships through different types of engagement. We welcome every opportunity to inform research communities and the public about how our research and support addresses long-standing challenges in translational research so that new treatments and other health solutions reach all people more quickly.

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

Effective stewardship is key to our success and the achievement of our strategic goals. NCATS is dedicated to the innovative and responsible use of resources to advance our 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. We focus on internal and external collaboration to overcome scientific, operational, and policy challenges. By working ethically and responsibly, we foster a collaborative environment with our communities. Together, we can ensure that transparency, integrity, and accountability drive our efforts, propelling translational science forward.

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

Effective translation is slowed when research and research findings are not disseminated within the biomedical research and clinical care ecosystems. The lack of transparency can also erode public trust. Transparency and open science are ways to promote reproducibility, progress, trust in research, and trustworthiness of researchers.

We will expand internal and external processes for transparent priority setting, including the launch, assessment, and sunset of activities (see the Approach to Priority Setting section). We will foster an open and collaborative working environment with flexible approaches to best address and manage scientific, operational, and administrative change. 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. Our outreach and communications will be more informative and accessible.

Objective 5-2: Support robust, reproducible, and ethical research.

Rigor, reproducibility, and ethical approaches to designing and conducting experiments are the cornerstones of scientific research. We have provided the translational science community with guidelines and best practices in these areas, from assay development to regulatory development.

As highlighted in the translational science principles, we apply these principles to generate reproducible, high-quality findings that contribute to advancing successful translation. We strive to ensure that planning, development, award, and implementation of all research programs and activities we support are conducted in a rigorous, robust, and data-driven manner. In addition, we will continue to develop and share standard guidelines and best practices for researchers. NCATS and the translational science community will look for ways to increase support for ethical, legal, and societal impacts of its research and activities.

Objective 5-3: Assess policies and provide guidance that impacts translational science programs and activities.

Different types of policies can enable or impede translation. Proper stewardship around policy is critical in developing and implementing research-related programs and activities to foster effective translation.

Relevant legal and policy frameworks must be taken into consideration, such as privacy protections when using patient data, operational processes when conducting clinical trials, procedures when screening and sequencing for rare diseases, and when drafting data sharing and management plans. As part of this strategic plan, we will continue to identify and understand how policies impact translation and provide our perspective on these impacts. Additionally, we will consider how data generated from research and related activities can inform policy development. From there, we will support and build activities that enable us to address potential barriers either directly or through our role as a convener bringing together different groups, including consultation with Tribal Nations, patients and communities, scientific societies, and other affected organizations. We will also seek ways to increase awareness throughout the translational science research community around the meaning and implications of various policies.

Objective 5-4: Optimize resources and infrastructure to supercharge the impact of translational science.

There are many factors to consider and address when developing and initiating translational science projects or programs. Addressing these factors — which include project scope, appropriate expertise, funding, and logistical operations — will improve start time and reduce time to results. Leveraging complementary expertise and tools ensures that research is being conducted in the most efficient manner.

We will continually assess and optimize organizational, operational, and business practices to advance translational science. This effort includes streamlining, digitizing, and automating business operations to align with relevant leading practices. We will ensure our funding opportunities address cutting-edge research needs and seize new opportunities. Our processes for grants, contracts, and other engagements will continue to be creative and robust. Regular monitoring and evaluation of our programs, activities, and priorities will ensure they align with and advance the NCATS mission.

Appendices

Appendix A: Additional Details on NCATS' Organization

Statutory Authority

Establishment of NCATS

NCATS was created on December 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 December 13, 2016, subsequently modified NCATS' authorization language.

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

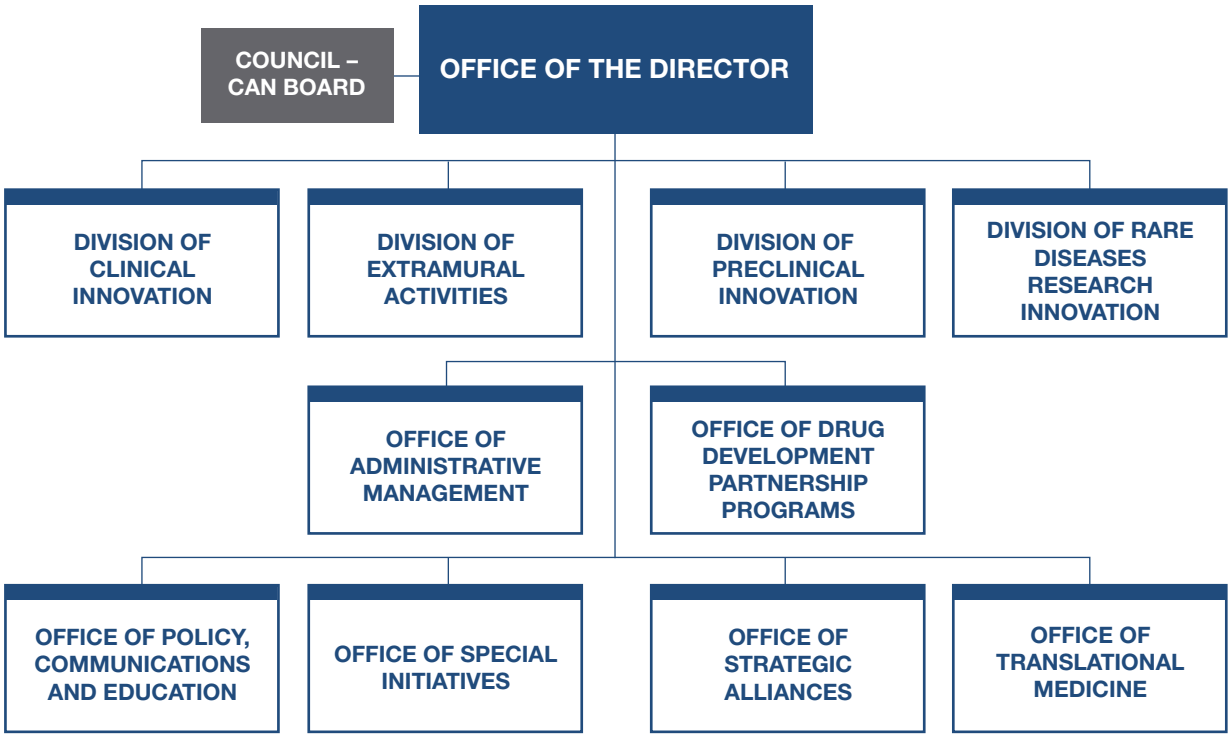
Cures Acceleration Network

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 interested parties wrote a letter to Congress (PDF — 35KB) on May 14, 2010, asking Congress to provide funding for CAN.

On December 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



To learn more about NCATS, visit our website:

- NCATS Divisions and Offices
- NCATS 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. The release of the *NCATS Strategic Plan for 2025–2030* 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 (Figure B-1).

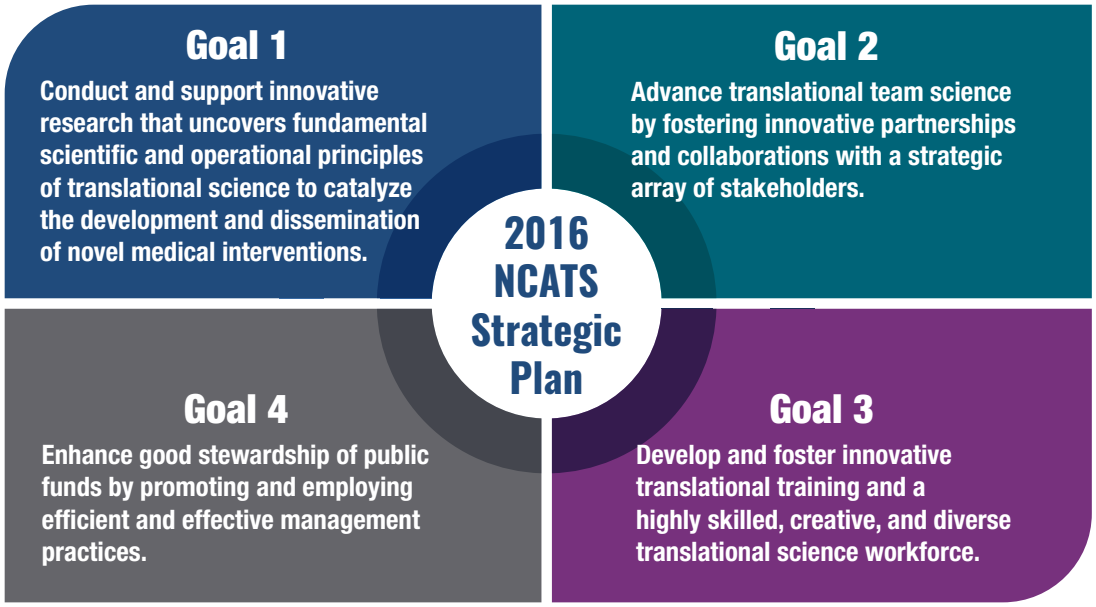


Figure B-1. The four goals for the 2016 NCATS Strategic Plan.

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 our Budget webpage), NCATS biennial reports, NCATS Areas of Impact, and NCATS News & Events.

Innovation and Impacts 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) 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 diseases 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 at a time.

Expansion of Training Opportunities in Translational Science (Goal 3)

NCATS provides robust training experiences for new translational scientists through our numerous internal fellowship opportunities and various awards to our external collaborators. Additionally, the NCATS Office of Strategic Alliances 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 Clinical 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 Translation Laboratory and 3-D Tissue Bioprinting Program, and developed tools to work with these systems, like Somatic Cell Genome Editing. In particular, our Tissue Chip 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 (Figure B-2). Tissue chips have allowed the testing of treatments for many diseases or conditions, including addiction and pain and COVID-19, as well as understanding aging by sending tissue chips into space.

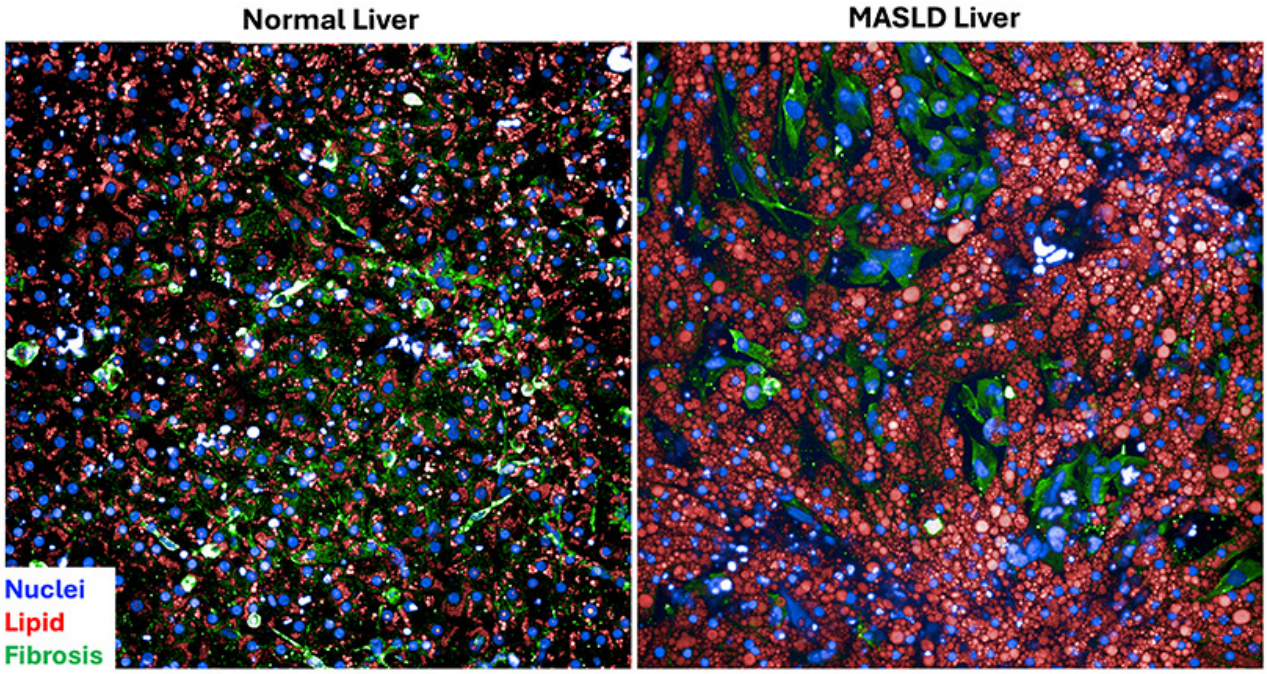


Figure B-2. Tissue chip models used to study the progression of metabolic dysfunction–associated steatotic liver disease (MASLD), which affects a quarter of the world's population. On the left, a normal liver is shown with four different types of cells. On the right, a diseased liver reveals a high amount of fatty lipids and the beginning of fibrosis, a life-threatening type of liver damage. (Dillon Gavlock, University of Pittsburgh Drug Discovery Institute)

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 and other templates speed the implementation of research agreements. Thanks to 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 for managing the Native Collective Research Effort to Enhance Wellness (N CREW) Program, under The Helping to End Addiction Long-term® Initiative, or NIH 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 readiness to conduct clinical trials for regulatory approval, and the FDA has approved 56 Investigational New Drug (IND) clearances based on research involving the NCATS Division of Preclinical Innovation. The preclinical research conducted by NCATS was also key to de-risking the clinical trials leading to the approval of four drugs (Figure B-3).

Figure B-3. Contributions of the NCATS Division of Preclinical Innovation to Investigational New Drug clearances and drug approval applications.

Appendix C: Strategic Planning Process

In November 2022, following the appointment of Dr. Joni L. Rutter as the new director, the center began the process of updating the *NCATS Strategic Plan* that was released in fall 2016. The strategic planning team formed to manage the day-to-day activities of the strategic planning process. The team was based in the NCATS' Science Policy Branch and included staff from the Office of Policy, Communications and Education and the NCATS Office of the Director. As described below, all parts of the center as well as of the external community have contributed feedback and input throughout the entire process.

The almost two-year-long process encompassed robust community engagement, development of a strategic framework, a request for public and internal input on the framework, and drafting of the plan, while incorporating feedback at each stage (Figure C-1).

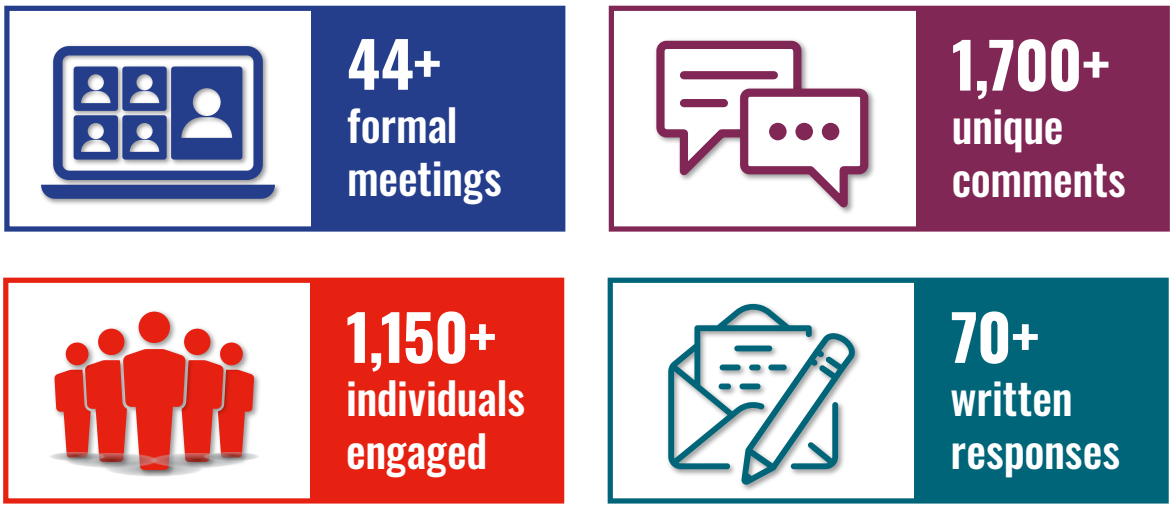


Figure C-1. By the numbers: How NCATS involved the community in the strategic planning process.

Community Engagement

From January 2023 to August 2023, Director Rutter and the strategic planning team participated in over 44 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:

- We held 19 virtual discussions involving more than 350 NCATS staff members.
- We hosted two virtual strategic planning roundtable discussions for the public 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 different translational science topics. All breakout sessions answered the same four broad, open-ended questions to solicit input.
- Dr. Rutter presented the strategic plan at 24 virtual meetings of the CTSA Program committees and at two meetings with researchers and patient advocacy groups who are part of the RDCRN. These conversations included questions tailored for each particular group. Dr. Rutter also took other opportunities through public speaking engagements to discuss and engage individuals and groups on the strategic plan.
- The NCATS Advisory Council discussed and gave input on the strategic plan at their meetings in May 2023, September 2023, January 2024, and May 2024.

We routinely promoted the strategic plan through different communications channels and solicited input through a public email address to receive additional comments or questions.

After releasing the strategic plan, we will continue to engage internal and external groups in dialogue about our activities and progress.

Framework Development

Referring to a set of relevant, recurring themes identified from the feedback, we categorized the more than 1,700 comments received across strategic engagements (Figure C-2). Using this semiquantitative analysis of the input received, we developed a draft framework for the *NCATS Strategic Plan for 2025-2030.* The framework presented five strategic plan goals, a brief narrative context of each goal, and a set of potential themes aligned to the NCATS vision.

Framework Input

In September 2023, we presented a draft framework at the NCATS Advisory Council meeting and published a request for information (RFI) (NOT-TR-23-027) via an NIH Guide Notice. The RFI asked for feedback on the draft goals and plans presented in the framework. We received 51 separate responses.

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 meeting for staff 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 on the NCATS website from May 14 through June 14, 2024. It was presented and discussed at the NCATS Advisory Council in May 2024. We received over 20 written responses on the draft and made edits to the strategic plan as applicable. Many comments were more relevant to the implementation process and have been catalogued for implementation (see Appendix D).

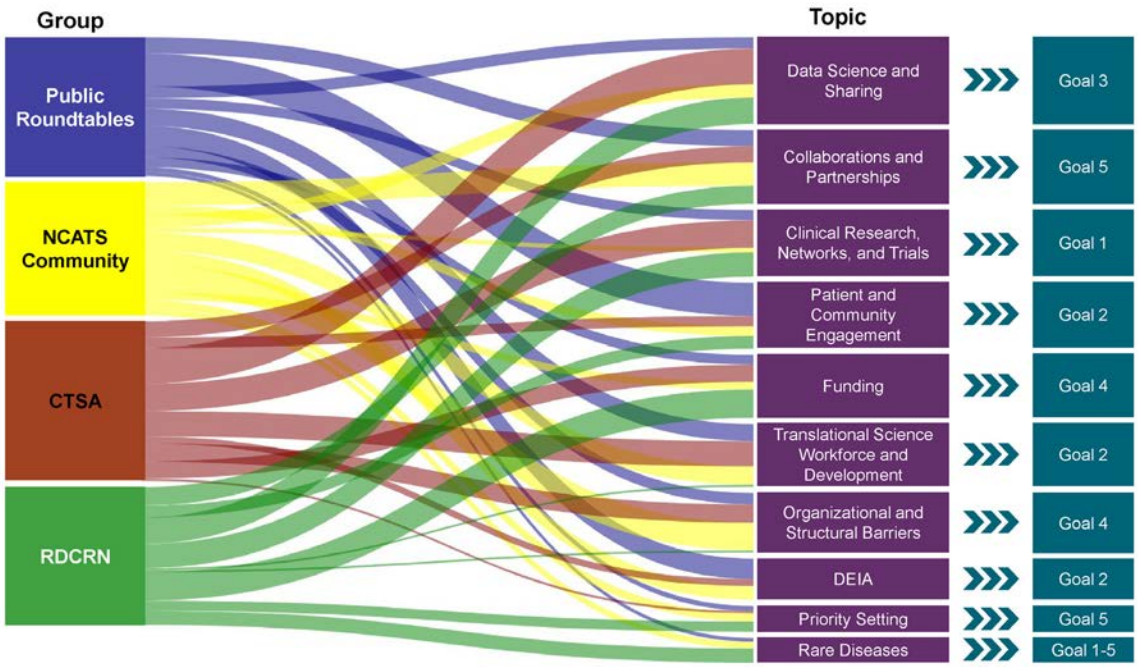


Figure C-2. Different voices focusing on similar topics. NCATS held Strategic Plan roundtable discussions in 2023 with multiple communities and groups (left column). Even with a broad set of participants, a common set of themes was identified by participants for NCATS to consider (right column). Line thickness indicates proportional feedback provided on a topic.

Appendix D: Implementation of the Strategic Plan

The *NCATS Strategic Plan for 2025–2030* sets forth a vision with five goals, each with objectives for implementation. By continuously evaluating our progress and adapting our strategies, we are committed to making a significant impact on advancing translational science and improving health outcomes.

As the strategic plan was developed, all divisions and offices provided input at multiple steps to ensure center-wide engagement. Involvement at all levels of NCATS will continue after the *NCATS Strategic Plan for 2025–2030* is released, starting with internal center leadership discussions in fall 2024.

Consideration of strategic plan implementation is already underway internally. It will be coordinated across the center through the immediate Office of the NCATS Director. Implementation will include prioritizing activities to pursue, as well as putting in place a strategy to track progress, develop metrics, and keep our communities informed as we evaluate our progress.

To start, the *NCATS Strategic Plan for 2025–2030* will be used as a reference point for guiding all programs and initiatives that NCATS implements going forward. For example, internal planning is an NCATS-wide activity that occurs two to four times per year during which new ideas are brainstormed and discussed by NCATS staff and leadership to promote transparency, collaboration, coordination, and priority setting. This includes mapping programs and activities to the relevant strategic plan goals and objectives to ensure alignment. As part of this process, starting in October 2024, staff submitting proposals for activities and programs will identify the relevant strategic plan goal(s) and objective(s). Concept clearances for the NCATS Advisory Council also will incorporate this information.

Discussion of potential metrics of the progress in achieving our strategic plan's goals and objectives and the impact of our programs and activities is ongoing. We will use a variety of approaches to systematically monitor and collect accomplishments and other indicators of progress. We will continue discussions with our constituents to inform how we think about what is impactful in terms of progress in fulfilling our mission and vision.

We will continue to ensure that our implementation aligns with White House, U.S. Department of Health and Human Services, and NIH priorities and plans. Center-specific activities recently initiated include developing roadmaps for NCATS' pillars of organizational culture and impact and center-wide data science strategies.

Appendix E: Acronyms and Abbreviations

AAV	adeno-associated virus
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
CAB	community advisory board
CAN	Cures Acceleration Network
CTSA	Clinical and Translational Science Awards
DEA	Division of Extramural Activities
EHR	electronic health record
FAIR	findable, accessible, interoperable, and reusable
FDA	U.S. Food and Drug Administration
GARD	Genetic and Rare Diseases Information Center
IDeaS	Impact of Rare Diseases on Patients and Healthcare Systems
IRB	Institutional Review Board
MASLD	metabolic dysfunction–associated steatotic liver disease
ML	machine learning
N3C	National Clinical 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
SMART IRB	Streamlined, Multisite, Accelerated Resources for Trials IRB
TIN	Trial Innovation Network

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