NCATS Strategic Plan

National Center
for Advancing
Translational Sciences

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Contents

Welcome from the Director

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Five years ago, the National Center for Advancing Translational Sciences (NCATS) was established to catalyze a transformation in the way health interventions are developed and to bring more treatments to more patients more quickly. This required new fundamental principles and approaches to define the burgeoning field of translational science. Just as the understanding of cells and organs created a basis for the development of interventions for the diseases that affect them, so will the understanding of the translational process create a basis for more science-driven, predictive and effective intervention development for the prevention and treatment of all diseases.

This new science of translation is distinct in content, operations and culture. While this presents unique challenges in uncharted territory, it also provides us with unprecedented opportunities within the larger context of the biomedical enterprise. Encompassing many disciplines of science and operations, including biology, chemistry, informatics, pharmaceutics, engineering, medicine, public health, project management, team science, collaboration development and patient engagement, translational science defines the scientific and operational relationships among these fields, builds bridges, and creates a network that leverages all stakeholders to more effectively develop and deliver interventions that benefit the health of the public.

NCATS has engaged in a broad internal and external consultation process to establish a strategic plan that conveys not only what we aspire to accomplish, but also the enormous potential and excitement of our field. This has itself been a learning process, and our Center is much richer for the hundreds of contributors who have given us their best ideas, many of which you will read here.

We have organized this Strategic Plan according to conceptual themes of translational science, collaborations, training and stewardship. Within these themes are NCATS' goals and specific objectives and strategies that exemplify best approaches. We are proud of what has been accomplished during the first five years of NCATS, as described in our Annual Reports. This Strategic Plan sets the stage for the Center's future and will be a living document that will be adapted over time and as relevant to the changing needs in translational science.

NCATS is by design a different kind of scientific organization with a different kind of mission, epitomized by the word "translation" in our name, which is derived from Latin meaning "to carry across." The science of carrying across from one field to another, from one part of the research ecosystem to another, to bring more treatments to more patients more quickly — this is the NCATS mission. We welcome all to join us in making this bold, collaborative and forward-looking plan a reality.

Introduction

Overview of NCATS

The National Center for Advancing Translational Sciences (NCATS), one of the 27 Institutes and Centers (ICs) at the National Institutes of Health (NIH), is transforming translational science to get more treatments to more patients more quickly. NCATS relies on the power of data, new technologies and teamwork to develop, demonstrate and disseminate innovations that reduce, remove or bypass costly and time-consuming bottlenecks in translational research.

Rather than targeting a particular disease or area of fundamental science, NCATS focuses on what is common across diseases and the translational process. The Center conducts and supports research on both the scientific and operational aspects of translation to lead to more predictive and successful development of new medical interventions, such as drugs, diagnostics, and medical devices, for all human diseases.

NCATS' efforts by design complement and empower those of other NIH ICs, academic scientists, the private sector and the nonprofit community. By emphasizing collaboration, innovation, deliverables and team science, the Center serves as a catalyst to enable others in the translational research ecosystem to work more effectively.

Translation and Translational Science

Defining Translation and Translational Science

- **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 of investigation focused on understanding the scientific and operational principles underlying each step of the translational process.

NCATS studies translation on a system-wide level as a scientific and operational problem.

Translational Science Spectrum
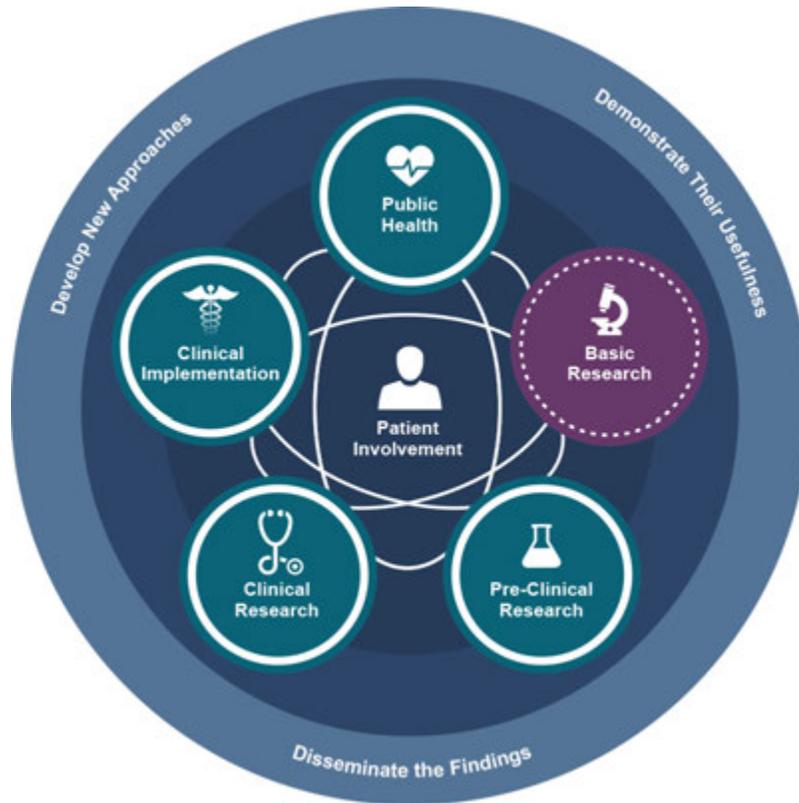
The translational science spectrum, as illustrated in the graphic above, represents each stage of research along the path from the biological and chemical basis of health and disease to interventions that improve the health of individuals and the public. The spectrum is not linear or unidirectional; each phase builds upon and informs the others. At all stages of the spectrum, NCATS develops new approaches, demonstrates their usefulness, and disseminates the findings. Patient involvement is a critical feature of all phases in translation.

Basic Research — Basic research involves scientific exploration that can reveal fundamental mechanisms of biology, disease or behavior. Every stage of the translational research spectrum builds upon and informs basic research. NCATS scientists typically do not conduct basic research; however, insights gained from the Center's studies along the translational spectrum can inform basic research.

Pre-Clinical Research — Pre-clinical research connects the basic science of disease with human medicine. During this stage, scientists develop model interventions to further understand the basis of a disease or disorder and find ways to treat it. Testing of medical interventions is carried out using cell or animal models of disease; samples of human or animal tissues; or computer-assisted simulations of drug, device or diagnostic interactions within living systems.

Clinical Research — Clinical research includes studies to better understand a disease in humans and relate this knowledge to findings in cell or animal models; testing and refinement of new technologies in people; testing of interventions for safety and effectiveness in those with or without disease; behavioral and observational studies; and outcomes and health services research. The goal of many clinical trials is to obtain data to support regulatory approval for an intervention.

Clinical Implementation — The clinical implementation stage of translation involves the adoption of interventions that have been demonstrated to be useful in a research environment into routine clinical care for the general population. This stage also includes implementation research to evaluate the results of clinical trials and to identify new clinical questions and gaps in care.

Public Health — In this stage of translation, researchers study health outcomes at the population level to determine the effects of diseases and efforts to prevent, diagnose and treat them. Findings help guide scientists working to assess the effects of current interventions and to develop new ones.

Center Organization

NCATS' divisions and offices span the entire spectrum of translational science. Through programs in its Division of Pre-Clinical Innovation, the Center drives advances in early stages of the translational process, from target validation to first-in-human studies. Through its Division of Clinical Innovation, NCATS supports clinical and translational research, creating and sharing the expertise, tools and training needed to develop and deploy effective treatments in people. Cross-cutting programs in rare diseases, translational technologies, strategic alliances and other emerging areas address scientific and operational barriers to enable faster and more effective interventions that tangibly improve human health.

Bridging the Gap: Scientific and Operational Challenges and Opportunities in Translational Science

Several thousand genetic diseases affect humans, of which only about 500 have any treatment. A novel drug, device or other intervention can take about 14 years and cost $1 billion or more to develop, and about 95 percent never make it past clinical trials. Even when a new drug or other intervention is developed and shown to be effective in clinical trials, many years may pass before all patients who could benefit from it are identified and treated.

Numerous scientific and organizational roadblocks can limit the speed of progress. Obstacles along the path to translation include:

- Lack of understanding about the science of translation and insufficient rigor in conducting translational research, leading to unpredictability and frequent failure of possible interventions
- A shortage of qualified translational investigators
- Organizational structures and incentives that do not encourage the teamwork essential to translational science
- Inflexible and inefficient clinical trial implementation and low participation in studies
- A lack of data interoperability
- Insufficient tools and technologies to predict toxicology and efficacy in safety assessment
- A shortage of qualified biomarkers for application in disease diagnosis and measurement of treatment response
- Inadequate development and measurement of appropriate clinical outcome measures or endpoints, including patient reported outcomes

Strategic Plan Development

Internal Process: Strategic Principles

NCATS employees were engaged in the early stages of the Center's strategic planning process to provide their perspectives on the fundamental characteristics that guide activities at NCATS, both current and aspirational. Through a series of small group discussion sessions, the opinions and insights of nearly all NCATS employees were gathered. These diverse perspectives contributed to the development of the request for external input as well as the final strategic principles that are presented in the NCATS Strategic Principles section.

Gathering External Input: Focus Groups

To identify the key strategic planning topics on which NCATS would seek public input, NCATS created a series of focus groups composed of NCATS researchers, program leaders, other staff, and members of NCATS' two advisory committees: the Advisory Council and the Cures Acceleration Network (CAN) Review Board. Each focus group was asked to identify priorities and challenges in each of six pre-defined overarching areas of NCATS' research and operational activities. These focus areas were: Pre-Clinical Innovation — Improving the Drug Development Process; Pre-Clinical Innovation — Testing and Predictive Models; Repurposing Drugs; Re-Engineering the Clinical and Translational Process; Accelerating and Supporting Rare Diseases Research; and Building Partnerships with Stakeholder Groups. All focus groups also were asked to consider two cross-cutting questions: (1) how NCATS could more effectively engage with patients and community members in each phase of translation, and (2) how the strategic plan should address the role of "big data" and informatics in translational science.

Stakeholder Engagement: RFI and Webinars

After receiving additional input from NCATS Advisory Council and CAN Review Board members at a public meeting in early September 2015, NCATS launched the stakeholder engagement phase of the strategic planning process in October 2015 to solicit feedback from the broader public. The goal of this phase was to identify areas of opportunity, challenges and research needs in translational science to help set the Center's strategic priorities. NCATS' stakeholders include, but are not limited to, patients and members of the health advocacy community; basic, translational and clinical scientists at universities and research institutions; health care providers; biotechnology, venture capital and pharmaceutical industry members; colleagues at other NIH Institutes, Centers and Offices; partners at other government agencies (e.g., the Food and Drug Administration and other Department of Health and Human Services agencies, the Environmental Protection Agency, and the Department of Defense); policymakers; and the general public.

A public request for information (RFI) and a series of four "town hall" webinars were the principal vehicles used to solicit feedback from these individuals and groups. The RFI was publicly disseminated in October 2015 and was open for comment until February 2016, and the public informational webinars took place in October and November 2015. Through the RFI, NCATS encouraged stakeholders to comment on any issues of interest that apply across the translational science spectrum, including:

- Breaking down professional, cultural and scientific silos across the translational science spectrum
- Focusing on inter-operability of data systems (such as integrating patient data and electronic health records into pre-clinical research)
- Expanding research efforts at NCATS into new therapeutic modalities
- Focusing on patient-driven research and patient/community engagement
- Forming innovative partnerships with a wide variety of stakeholders
- Identifying skillsets and competencies needed for training the next generation of translational scientists
- Using modern communication and dissemination tools to expand awareness of translational science to a wide variety of stakeholders

NCATS received 54 responses to the RFI from individuals, organizations and institutions representing academia, government, industry, patient advocates, and health care providers. A total of 119 unique participants from around the country and across multiple sectors signed into the webinars to learn more about the NCATS strategic planning process and how best to contribute comments about the Center's strategic priorities via the RFI. The analysis of RFI responses involved carefully reviewing each response, along with any attached supplementary materials, and identifying specific recommendations and actionable items provided. The responses addressed a broad array of priority issues across both the scientific and operational domains of translation, and directly informed the development of the objectives and example approaches within each strategic goal.

NCATS Strategic Principles

NCATS' strategic principles represent its philosophy. To accomplish the overarching goal of bringing more treatments to more patients more quickly, NCATS programs are guided by the following principles:

- **Catalytic:** NCATS is a catalyst that enables others to perform more efficient and effective translation.
- **Generalizable Principles:** NCATS uncovers fundamental principles shared among diseases and translational processes; widespread implementation of such generalizable principles will accelerate translation.
- **Innovative:** NCATS programs lead to profound improvements in translational understanding and effectiveness, producing innovation that establishes fundamentally new ways of doing translation that are multiplicative in their effects.
- **Collaborative:** Translational research endeavors require the expertise of multiple people and groups, particularly as the research is carried across through different phases of the translational science spectrum. NCATS approaches translation as a "team sport."
- **Patient-focused:** At all phases of translational science, NCATS is committed to patients and their communities and looks for opportunities to include the patient perspective. The ultimate goal of translation is tangible improvement in health, so the perspectives of and partnerships with patients are crucial.
- **Measurable:** NCATS continuously improves translational effectiveness, so programs must be designed and implemented with explicit indicators of success for translational progress.

Structure of the Plan

The Strategic Plan is organized into four overarching themes of translational science, collaboration and partnerships, education and training, and stewardship. Each theme is captured within a strategic goal, and collectively, they provide an overview of what NCATS plans to accomplish to achieve its mission. The objectives listed under each Strategic Goal offer greater context to the high-level themes and will be used to guide NCATS in developing, evaluating and refining its research and operational agenda over time. Example approaches are potential ways for NCATS to achieve these objectives. In brief, the strategic goals reflect NCATS' overarching mission, the objectives provide context for each goal, and the example approaches offer more specific tactics for undertaking the objectives.

Strategic Goal 1: Conduct and support innovative research that uncovers fundamental scientific and operational principles of translational science to catalyze the development and dissemination of novel medical interventions.

NCATS is fundamentally transforming how translation is conducted on a system-wide level through research in translational science that uncovers, characterizes and implements the scientific and operational principles underlying each step of the translational process. By developing new approaches, technologies, resources, and methods that transcend a particular discipline or single disease, NCATS empowers the entire biomedical research community to conduct translation more efficiently. NCATS will identify and develop additional scientific and operational innovations to overcome system-wide bottlenecks in translational research and deliver health benefits more quickly.

Objective 1-1: Translate biological discovery into therapeutic potential through advances in pre-clinical translation and the development of publicly available, cutting-edge tools and technologies.

NCATS conducts and supports collaborative research across the pre-clinical phase of the translational science spectrum to develop new methods, tools and technologies to catalyze pre-clinical research and make it more efficient. In doing so, the Center effectively "de-risks" novel and promising therapeutic and diagnostic discoveries and enables the translation of these innovations to a state where they can attract outside investment for further clinical development. In this way, NCATS serves as an adaptor between academia, nonprofit research institutions, and private sector companies, each with complementary resources and skills to bring new medical interventions to patients faster.

Importantly, NCATS selects and carries out collaborative research projects to not only jump-start new therapeutic or diagnostic development, but also to help uncover general principles of translational science, or to test a new technology or approach. In addition, NCATS creates innovative methods to overcome translational bottlenecks in areas including therapeutic/diagnostic target identification, assay development, medicinal chemistry, drug screening, efficacy and toxicology testing, and implementation of new medical interventions.

NCATS broadly disseminates the innovative tools and technologies it develops to enable all participants in the biomedical science ecosystem to conduct pre-clinical research more efficiently and effectively. By improving our understanding of the principles underlying successful translational science, NCATS makes translational research more likely to deliver upon the ultimate goal of patient benefit.

Example approaches:

- Accelerate therapeutic target identification and validation through advanced gene editing technologies and screening with newly developed small molecule probes.
- Develop innovative biochemical and cellular assay platforms and chemical library screening approaches in the context of disease biology to overcome translational barriers in therapeutic development for underserved diseases.

- Use NCATS expertise in medicinal chemistry and informatics to develop and demonstrate innovations in hit-to-lead optimization of therapeutic candidates.
- Collaborate with academic, nonprofit and industry partners in drug synthesis, formulation, pharmacokinetics and toxicology to support initial regulatory review of interventions in development.
- Develop and integrate new models of human biology to better understand disease physiology and predict candidate drug safety, toxicity and efficacy.
- Develop and disseminate more efficient and effective approaches to predict how chemicals may affect human health, and organize the data collected from such approaches into predictive computational models for toxicity assessment.
- Provide collaborative access to NCATS' state-of-the-art resources and expertise in automation, compound management, analytical chemistry and informatics, among others.
- Make research data, tools and technologies produced by NCATS broadly accessible and available to the entire translational community.

Objective 1-2: Build well-connected, collaborative national capacity to conduct high-quality clinical research more effectively.

NCATS is innovating in the clinical phase of translation through the creation and development of novel processes, sharing of best practices, collaboration, and harmonization of research approaches at the local, regional and national levels. Beginning with a strong foundation of clinical researchers and research institutions, NCATS works to overcome common bottlenecks in clinical and translational research. These bottlenecks include engagement and recruitment of participants in clinical studies, streamlined review of the conduct of clinical studies, methods for measuring clinical outcomes, effective clinical trial design and statistics, and patient adherence to therapeutic regimens. Collaborative clinical research institutions with harmonized network capacity to efficiently and effectively respond to translational science needs will enable a more efficient national clinical research ecosystem with a diverse range of partners ready to quickly launch and complete studies to better understand human diseases and test medical interventions to treat them. NCATS is committed to actively disseminating clinical research innovations to all stakeholders for their use in making their own clinical translation more efficient and effective.

Example approaches:

- Develop innovations that significantly reduce delays in clinical study start-up, such as streamlined institutional review board review processes for multi-site trials.
- Create new community outreach approaches to speed participant recruitment and reach underrepresented communities.
- Implement processes that streamline and harmonize the planning, conduct, comparability, and reporting of clinical research.
- Collaborate to develop and disseminate cutting-edge, efficient, and generally applicable methods for disease characterization and diagnosis, including the use of genetic and environmental health information for use in precision medicine.
- Develop innovative methods and approaches that lead to appropriate clinical outcome measures that can be applied across diseases and for regulatory applications.
- Catalyze the innovation, testing and harmonization of information technologies to enable efficient clinical hypothesis generation and testing, facilitate participant recruitment for research, and enable straightforward cross-comparison of data sets.
- Develop and standardize content for clinical translational workforce education and training, including new emphases on team science, experiential learning, and entrepreneurship.
- Catalyze new approaches for improving adherence to treatments and interventions and determining the impact of those interventions in relevant communities and populations.

Objective 1-3: Identify biological commonalities among diseases and apply the knowledge of shared mechanisms and pathways to the testing and treatment of multiple human diseases and conditions.

The explosion of data on genes, cells and biological pathways over the last several decades has highlighted the remarkable connectedness of human biology. These relationships have profound implications for translational science, as studying commonalities across diseases presents opportunities for understanding not one but many diseases and can potentially accelerate translational research for several areas of biomedicine. New scientific insights gained from advances in genomics and the widespread use of molecular probes are leading to the discovery of shared genetic and biochemical pathways across diseases, which enable rapid application of the insights gained from the study of one disease to the treatment of others. NCATS therefore takes an integrative approach in all its programs.

Example approaches:

- Develop a publicly available knowledge base of interrelated biological pathways and characteristics across diseases, enabling researchers to identify common therapeutic targets.
- Cluster diseases with common mechanisms and molecular targets to enable multiplex therapeutic development of new therapeutic candidates with the potential for broader utility beyond a single indication.
- Apply knowledge of common molecular origins across diseases to repurpose drugs as new treatments for unmet medical needs.

Objective 1-4: Drive the development of new medical interventions, including for the many rare diseases that currently lack safe and effective treatments.

NCATS has a strategic focus on rare diseases, which are difficult to assess because of the large number of disorders, the complexity of each disease, small patient populations, and the limited availability of data. Rare diseases are devastating and costly for patients, their families, communities and society. The burden of rare diseases is attributable to both disease severity and the toll of the often lengthy and difficult process of diagnosis. NCATS takes an integrated approach to the understanding, diagnosis and treatment of rare diseases, investing resources and expertise across the translational science spectrum to move potential treatments forward for development and clinical testing. By also examining the scientific and operational outcomes of rare disease research for their potential applications to related rare and even common diseases, NCATS seeks to replace the one-disease-at-a-time approach to clinical and translational science with more efficient efforts. Central to the NCATS approach is the involvement of patients, families and caregivers throughout the translational process. Taken together, these approaches exemplify NCATS' unwavering commitment to accelerating translation in order to bring more effective treatments to all patients.

Example approaches:

- Apply a disease commonality approach to accelerate rare diseases understanding and treatment.
- Develop, demonstrate, and disseminate innovative and flexible clinical research methods that address the challenges of small patient populations.
- Promote common, scalable and participant-centered patient registry platforms that advance both individual rare diseases research and the understanding of shared characteristics among multiple diseases.
- Develop innovative and generalizable approaches for the conduct of natural history studies of rare diseases that enable the efficient identification of biomarkers, clinical outcomes and other measures required for the development and approval of medical interventions.

Objective 1-5: Develop interoperable and integrative biomedical informatics resources to facilitate translational innovation in disease prevention, diagnosis and treatment.

Translational science is fundamentally integrative, building on scientific and operational discoveries and processes as a basis for making translation more efficient and effective. Identifying the general principles for effective translation will be aided by the collation and comparison of data across diseases, therapeutic targets, interventions and projects. Such comparisons require not only proactive design and conduct of individual studies to make them compatible, but also biomedical informatics resources that are interoperable for facile data comparison, integration and analysis. Translational science with regards to informatics encompasses a broad range of disciplines including bioinformatics, cheminformatics, computational biology and chemistry, *in vivo* modeling, statistics, and medical informatics. Development of algorithms, software and technology platforms across these disciplines and their dissemination to the broader community is a key NCATS focus.

Example approaches:

- Create and foster a data-sharing environment that accelerates discovery and enables creation of new knowledge through meaningful integration of diverse biomedical and clinical data sets.
- Support the development of collaborative information technology resources to enable communication across clinical research sites and connect the health data sets they collect, including among others electronic health records, clinical trial outcomes and mobile health data.
- Serve as a clearinghouse and "neutral broker" of information that supports translation by emphasizing standardization and interoperability of current and future datasets.
- Develop and implement policies and practices for broad access to and sharing of informatics resources.

Objective 1-6: Ensure that NCATS-supported research spans the entire translational science spectrum, and address new areas of need and opportunity.

The pace of scientific discovery is accelerating and providing new opportunities for improving our understanding of and applications for translational science. Advances in our understanding of human physiology and behavior continuously fill each phase of the translational spectrum with exciting new therapies to develop, new operational strategies to test, and new technologies to engineer. NCATS will therefore regularly evaluate its research portfolio and identify new strategic opportunities for translational research and translational science.

Example approaches:

- Advance the translation of regenerative medicine by developing scientific and technological innovations to overcome the major limitations currently impeding the clinical application of induced pluripotent stem cells (iPSCs).
- Examine ways for NCATS to stimulate innovation and facilitate translation in the medical device and diagnostic research and development space.
- Expand research into the development and qualification of biomarkers for application in disease diagnosis and measurement of treatment response.
- Explore the therapeutic potential of newly developed technologies for human gene editing.
- Explore opportunities for NCATS' involvement in research on clinical implementation and social determinants of health.
- Expand research into novel therapeutic modalities (e.g., chemical libraries of novel scaffolds, structures, and properties) to enable modulation of undrugged genomic targets.
- Analyze scientific and operational failures across the translational spectrum in a systematic manner, and disseminate lessons learned to improve the process in the future.

Strategic Goal 2: Advance translational team science by fostering innovative partnerships and collaborations with a strategic array of stakeholders.

Translating a basic discovery to a demonstrated improvement in public health requires a translational research team of scientists, clinicians, research participants and other stakeholders having a wide range of expertise and perspectives of the scientific and operational roadblocks. Progressing through the phases of the translational science spectrum requires the creation of productive and mutually beneficial collaborations that depend not only on individual excellence, but on teamwork, coordination, cooperation and communication. Partnerships and collaborations across individuals and organizations are essential because the expertise, capabilities and viewpoints required for successful translation tend to reside in different groups with distinct missions. For example, developing a potential therapy to the point of regulatory approval can require expertise in molecular biology, medicinal chemistry, compound synthesis and formulation, pharmacology and toxicology, technology transfer, clinical science, regulatory science, integrating patient perspectives, and entrepreneurship, among others. However, incentive structures and professional and cultural barriers can make teamwork difficult to navigate. For this reason, NCATS places high value on innovation in team science and partnership development, and designs and tests novel partnership structures that cut across traditionally siloed scientific disciplines, organizations and sectors. Successful approaches are shared for all in the biomedical research community to use to improve their own translational efforts. Additionally, NCATS recognizes that translation is a global effort and therefore engages in partnerships and collaborations with international entities in multiple sectors.

Objective 2-1: Engage patients, community members and nonprofit organizations meaningfully in translational science, and develop and broadly disseminate best practices for patient-focused research.

Engaging patients, community members and nonprofit organizations in all aspects of translational research is important to advancing translation. As the eventual recipients of these research endeavors, patient participation as members of the research team provides insight and meaning to the creation, testing and clinical implementation of new medical interventions. Engagement of patients and their communities throughout the lifecycle of a translational research project ensures the outcomes are relevant to and directly address patient needs and will be more readily adopted by the community. NCATS views the science of patient and community engagement as a key area for exploration and innovation and will drive research efforts to develop, demonstrate and broadly disseminate best practices for patient-focused research.

Example approaches:

- Identify and disseminate fundamental principles of successful patient and community engagement.
- Develop educational and training resources for diverse audiences on the best practices for patient and community engagement.
- Identify the factors that facilitate patient-centered activities and develop tools to reduce resistance or remove barriers to patient engagement.
- Ensure that all research activities at NCATS appropriately include patient and community members in efforts that improve research design, implementation and dissemination.

Objective 2-2: Share resources and expertise across the federal government through collaborative research, particularly with other NIH Institutes and Centers.

NCATS serves as a partner, convener and expert on translational science and shares its expertise and resources with colleagues at other NIH Institutes and Centers (ICs) and across the federal government. NCATS enables our colleagues to move their translational efforts to a higher level by matching their domains of expertise with NCATS' translational science expertise and technologies to advance research

more efficiently and effectively. NCATS also attempts to bridge translational pitfalls by identifying and collaborating with other government agencies to enable the research to progress, even after NCATS' specific role in the effort is complete. Partnering with researchers across NIH who have knowledge of particular disease areas is essential to demonstrating the utility of translational technologies and strategies developed by NCATS. The same is true with other government agencies with complementary roles in the translational ecosystem. For example, partnerships with the Food and Drug Administration (FDA) are critical to ensuring innovations and interventions are developed in close consultation with the regulators who will ultimately evaluate them. NCATS is committed to working with all of its federal partners to collaboratively advance translational science.

Example approaches:

- Partner with NIH ICs to identify common roadblocks in translation that apply across diseases so that NCATS can focus its science in areas of greatest need.
- Partner with NIH ICs to support translational research to accelerate intervention development by combining translational science expertise with disease expertise.
- Create and share template agreements that facilitate the initiation of new collaborations among various stakeholders with government agencies.
- Collectively develop the scientific tools, standards and approaches that support efficient translation of research for regulatory assessment.
- Serve as a convener for issues of common interest across the federal government with regard to translational research and translational science.
- Provide interagency leadership to coordinate rare diseases research.
- Coordinate with the FDA and other federal agencies to advance areas of the translational landscape that are rapidly evolving, such as adaptive/novel clinical trial designs, and patient and community engagement strategies.

Objective 2-3: Form innovative collaborations with scientists at academic and other research institutions to translate early-stage discoveries into novel medical interventions.

Investigators at research institutions in the U.S. and around the world make discoveries in basic, clinical and public health research that can serve as the foundation for translational research to improve public health. Academic medical research institutions have extensive capabilities and capacity to carry out clinical, patient care, and public and community health research. NCATS teams with these investigators to collaboratively "de-risk" promising pre-clinical interventions by applying expertise and resources to efficiently generate necessary evidence and data for safety and efficacy assessment. When successful, these collaborative efforts provide evidence to attract external partners for further development and dissemination. Importantly, each collaborative project has a dual mission: first, to de-risk and advance a project to the next phase of translation; and second, to understand the scientific and operational underpinnings of successful approaches and apply them to future projects for more efficient translation.

Example approaches:

- Build novel types of collaborations with researchers to combine their deep knowledge of disease biology and chemistry with the expertise of NCATS researchers in pre-clinical development, project management, clinical research and regulatory science to advance the development of promising therapeutic candidates.
- Design innovative collaborative projects to advance clinical translational science in all facets from first-in-human intervention studies to community health.
- Form and participate in international partnerships with groups and organizations around the world as appropriate to leverage complementary capacities and accelerate progress.

Objective 2-4: Devise new collaborative structures with private-sector partners to advance translational technologies for patient benefit.

NCATS' mission includes devising new collaborative partnerships with private-sector entities in order to increase the efficiency and effectiveness of translation. The Center proactively seeks the necessary partners to ensure that the programs to which it commits resources have the greatest chance of success in reaching patients. Such projects in the later stages of therapeutic development generally require collaboration with a biopharmaceutical company with the expertise and financial and technical resources to achieve the ultimate goal of patient benefit. NCATS also supports small businesses and entrepreneurship programs as an integral part of advancing translational science and developing new translational technologies.

Example approaches:

- Partner with colleagues in the pharmaceutical, biotechnology and venture capital communities to identify and address translational science roadblocks in their areas of expertise.
- Identify common priorities and projects with industry partners that would leverage complementary efforts to achieve translational goals more efficiently, and avoid duplication.
- Promote collaborative efforts between biopharmaceutical companies and the biomedical research community to advance therapeutics development, particularly in the areas of rare diseases and drug repurposing.
- Assist small businesses in developing and commercializing translational technologies and products, such as innovative tools and platforms that would support the creation of interventions or disease diagnostics.
- Partner with the private sector to provide exchange programs and experiential learning opportunities for translational scientists.

Strategic Goal 3: Develop and foster innovative translational training and a highly skilled, creative and diverse translational science workforce.

A key to advancing the burgeoning field of translational science is through development of translational science education and training, and support for a diverse translational science workforce. Translation is inherently cross-disciplinary, and will benefit not only from robust training in one or more scientific research domains, but also from broad-based education on the scientific and operational principles that underlie sound translational science. This type of training will enable team members to be more effective in project planning and management, as they will be able to anticipate the needs and requirements at the next phases of the translational process. To foster an innovative translational science workforce, NCATS will catalyze the development, utilization and dissemination of training concepts and programs in translational science; foster ongoing efforts to support translational science as a discipline; and engage broad audiences about translational science so they may participate in the translational process or pursue a career in translational science.

Objective 3-1: Identify and broadly disseminate the distinct knowledge, skillsets and core competencies needed by translational scientists.

The future of translational science requires the convergence of a broad array of disciplines — including among others biology, chemistry, computer science, engineering, medicine and public health — into a united effort to uncover the scientific and operational principles of efficient and effective translation. However, the characteristics and attributes required to be a successful translational scientist go beyond the competencies of existing individual disciplines and include an understanding of the translational spectrum as a whole. For example, an expert in biochemistry looking to translate a compound into a potential treatment will benefit from familiarity with areas such as toxicology and pharmacokinetics, biomarker development and validation, regulatory requirements for pre-clinical and clinical phase testing, current standard of clinical care, and patient preferences for treatment, to name a few. Such awareness enables practitioners of translational science to intentionally design and conduct their research in a way that anticipates the expertise and data needed for subsequent phases of translation. Furthermore, since translational science is focused on identifying the general scientific and operational principles of efficient and effective translation, training must continuously improve as the discipline develops. To accomplish these goals, NCATS will collaborate with its partners in science training and workforce development to define and disseminate the requisite knowledge, core competencies and skillsets of translational scientists.

Example approaches:

- Collaborate with partners in academia, government, industry and elsewhere to identify the knowledge, skills and approaches that will be needed by the next generation of the translational science workforce.
- Highlight the many and varied career paths available to translational scientists and the competencies required by each.
- Incorporate an international perspective in the development of core competencies in recognition of the global span of translational science.
- Emphasize trans-disciplinary training.

Objective 3-2: Develop and support translational science training and career development programs to cultivate a multi-faceted and diverse translational science workforce.

Increasing recognition and interest in translational science has created an urgent need for training and career development programs that provide the particular skills, knowledge, characteristics and perspectives critical for success in this burgeoning field. A greater emphasis must be placed on trans-disciplinary workforce development that includes non-traditional knowledge areas such as project management, entrepreneurship, communication and team science. Equally important as professional

diversity in the translational workforce is personal diversity, which brings different perspectives, creativity and individual enterprise to address complex translational problems. NCATS supports the development of translational science education and training needed for the continued growth of the translational science discipline.

Example approaches:

- Develop translational science education and training programs to prepare trainees for translational careers in sectors including academia, industry, nonprofit and government.
- Identify areas of opportunity and value for translational science education and training with respect to the evolving biomedical research landscape such as in industry, public policy, research management, advocacy and communications.
- Grow a workforce not defined by a single discipline, but one that works across and at the boundaries of other more "traditional" life sciences with a focus on successful team qualities and attributes.
- Leverage trans-NIH commitments to enhance workforce diversity, expand the pool of translational science-trained professionals, and ensure the translational science workforce is broadly representative across racial, ethnic, sex, gender, age, socioeconomic, geographic and disability status.
- Support entrepreneurial training programs and opportunities for translational scientists to gain experience in business development.

Objective 3-3: Support the widespread acceptance of translational science as a formal discipline to help provide a clear and sustainable career path for translational scientists.

NCATS is committed to fostering the recognition and growth of translational science as a scientific discipline with unique attributes, research goals, knowledge requirements, operational approaches and deliverables. This will provide a stable foundation for the accumulation of translational science knowledge and a research community that will contribute to the collective advancement of translation.

Example approaches:

- Collaborate with universities, professional societies and other NCATS stakeholders to communicate the importance and value of recognizing translational science as a distinct discipline.
- Support opportunities for an open discussion about a way to assess career development and advancement in translational science.
- Partner with research universities and other interested parties to support the design and development of translational science degree programs.

Objective 3-4: Expand understanding of translational science through the deployment of cutting-edge communication and dissemination tools and technologies.

There is a pressing need to increase the awareness and understanding of translational science and its contributions to biomedical research and public health. NCATS will communicate the importance of this new discipline using a full suite of modern communication and engagement technologies to motivate individuals at all levels to become informed and engaged in translational science.

Example approaches:

- Expand awareness of the unique role of translation in delivering on the promise of science and medicine for patients.
- Emphasize the key concept of translational science as a growing field that is defining the scientific and operational principles of translation to improve its efficiency and effectiveness.

- Expand awareness efforts with key stakeholders about NCATS as a catalyst for this young field, and as a collaborative partner in all translational research.
- Tell the NCATS story and highlight the Center's translational advances using a variety of traditional and new media approaches and outlets.
- Collaborate with allied organizations to develop electronic resources that communicate the value and impact of translational science.

Strategic Goal 4: Enhance good stewardship of public funds by promoting and employing efficient and effective management practices.

NCATS is a steward of public resources, and, as such, has the responsibility to deploy those resources in the most effective manner. This requires not only supporting innovative research, but also fostering continuous improvement in its operations to improve scientific stewardship. NCATS will use efficient management practices and will work with its employees, awardees and partners throughout the government and beyond to leverage available resources toward the development and dissemination of medical interventions.

Objective 4-1: Continually assess and optimize internal business practices.

Just as NCATS seeks to increase the efficiency and efficacy of translation, the Center places a high priority on continually assessing and optimizing its own internal operations. NCATS will continue to foster an open and collaborative working environment with flexible approaches to best address and manage scientific, operational, and administrative change. This includes optimizing internal organization and infrastructure, including technology systems, and encouraging innovative approaches to internal operating procedures.

Example approaches:

- Identify and solve common operational issues in the translational space to optimize use of public resources.
- Continually assess and adapt NCATS' organizational structure to optimally support evolving Center programs and their operations.
- Develop and implement information technology infrastructure platforms to facilitate workflow and enhance internal operating procedures.
- Use all available authorities, funding structures, and business models, and establish new ones to maximize efficiency and effectiveness in accomplishing organizational goals.

Objective 4-2: Ensure all scientific programs and operational activities are conducted in a rigorous, robust and data-driven manner.

As a public science agency, NCATS is committed to using transparent, scientific approaches in support of the most meritorious science that furthers the Center's mission. To fulfill this responsibility, NCATS emphasizes accountability using defined indicators of success. NCATS is committed to objective assessment of all efforts through the use of clear metrics and deliverables; promoting data collection and analysis; ensuring the consistent application of measurement criteria; and exploring new research funding mechanisms and opportunities that optimize the impact of team science. This will advance NCATS' mission in an innovative, collaborative and data-driven manner.

Example approaches:

- Ensure results-based accountability of translational science programs using defined indicators of success.
- Measure, actively manage, and report on the impact of NCATS-funded projects and programs.
- Use robust data collection and analysis methods to guide data-driven decision making.

Objective 4-3: Foster complementary connections and teamwork across NCATS programs and divisions.

Collaborative efforts across NCATS' programs leverage complementary expertise and tools, reduce redundancy, and ensure the best science is being conducted in the most efficient manner. Strategic alignment of NCATS' scientific initiatives will continue to be reinforced by the creation of unique project

teams that address scientific, administrative and management priorities in support of the Center's objective of capitalizing on all available opportunities to accelerate translational science.

Example approaches:

- Identify opportunities for complementarity between NCATS intramural and extramural programs to take advantage of their distinct strengths and increase efficiency.
- Explore use of novel funding, program management and awards management approaches to support the best translational science.
- Facilitate collaborative efforts among NCATS extramural programs to leverage resources and harmonize best practices.

Objective 4-4: Disseminate research results and best practices broadly, and promote a culture of openness, sharing and transparency.

Dissemination is a key principle of NCATS' mission as a catalyst of translational science. NCATS will maximize the impact of the Center's translational science efforts by proactively creating and sharing resources, methodologies, operational models, tools and data, as well as resources designed to facilitate sharing and collaboration. NCATS also promotes a culture of sharing and transparency among its grantees and staff, which fosters the trust and creativity on which team-based translational innovation is built, thus maximizing productivity and enhancing stewardship.

Example approaches:

- Share databases, repositories and templates for establishing collaborations, guidelines, best practices and other material resources to empower the larger scientific community and facilitate crowdsourcing of ideas and solutions.
- Encourage NCATS-supported researchers to develop dissemination plans that engage community, patient, and other relevant stakeholders in their projects.
- Develop new approaches for sharing translational research results and resources for public use and benefit.

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