NCATS
Improving Health Through Smarter Science

Transforming Translational Science

The National Center for Advancing Translational Sciences (NCATS) is one of 27 Institutes and Centers at the National Institutes of Health (NIH). NCATS conducts and supports research on the science and operation of translation to allow more treatments to get to more patients more quickly. The Center focuses on what is common across diseases and the translational process.

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.

Bridging the Gap

Several thousand diseases affect humans, of which only about 500 have any treatment. A novel drug can take 10 to 15 years and more than $2 billion to develop, and failure occurs about 95 percent of the time. Even when a new drug or other intervention is developed and shown to be effective in clinical trials, many years may pass before the drug is available to all patients who could benefit from it.

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

- Lack of understanding about the science of translation, leading to unpredictability and frequent failure of possible interventions
- A shortage of qualified investigators
- Organizational structures and incentives that do not encourage the teamwork essential to successful translation
- Inflexible, inefficient clinical trial designs and low participation in studies
- Inexperience with navigating potential therapeutics through regulatory pathways

NCATS is bridging these gaps by **developing** new approaches, technologies, resources and models; **demonstrating** their usefulness; and **disseminating** the resulting data, analyses and methodologies to the broader scientific community.

Translation Is a Team Sport

The translation of a basic discovery to a demonstrated improvement in public health requires cross-disciplinary teams of scientists, clinicians and other stakeholders with wide-ranging expertise and perspectives.

NCATS studies translation on a system-wide level as a scientific and operational problem, developing and supporting innovative collaborations across traditionally separate scientific disciplines and organizations, including government agencies, academia, industry and patient organizations. Serving as a catalyst for translational science, the Center convenes teams with diverse expertise — in disease biology, toxicity, informatics, biomarkers, clinical trials, public health and other areas — to reduce, remove or bypass significant bottlenecks across the entire continuum of translation.
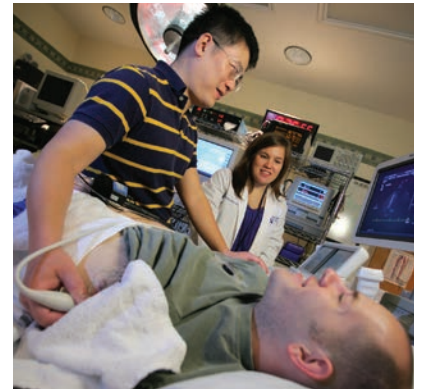
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NIH…Turning Discovery Into Health

NIH National Center for Advancing Translational Sciences
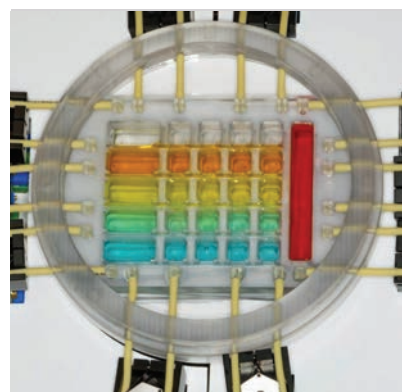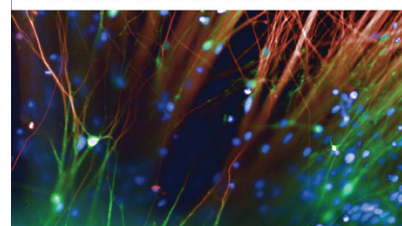
Spanning the Full Spectrum of Translation

NCATS' programs, initiatives and other resources span the entire spectrum of translational science. Examples include but are not limited to the following:

- **Assay Guidance Manual (AGM):** The AGM and related initiatives provide and support best-practices resources and training courses for successful development and implementation of robust, early-stage drug discovery assays.

- **Biomedical Data Translator:** Through the Translator program, scientists are integrating multiple biomedical data sources to reveal new insights about diseases and treatments and to identify novel opportunities for research.

- **Bridging Interventional Development Gaps (BrIDGs):** BrIDGs supports targeted contract access to drug development resources that allow academic, not-for-profit and small business collaborators to generate the data they need to submit an Investigational New Drug application to the Food and Drug Administration (FDA).

- **Clinical and Translational Science Awards (CTSA) Program:** The CTSA Program supports an innovative national network of medical research centers that work together to improve the translational research process. Goals are to train the translational science workforce, engage patients and communities in every phase of translation, promote integration of special and underserved populations across the human lifespan, improve the conduct of multisite clinical trials and advance the use of cutting-edge informatics.

- **Discovering New Therapeutic Uses for Existing Molecules:** New Therapeutic Uses supports collaborations between pharmaceutical companies and academic researchers to rapidly test new ideas for existing investigational compounds.

- **Genetic and Rare Diseases (GARD) Information Center:** The GARD website and staff provide comprehensive information on rare and genetic diseases for patients, their families, health care providers and the public. NCATS administers GARD in collaboration with the National Human Genome Research Institute.

- **Rare Diseases Clinical Research Network (RDCRN):** The RDCRN program is designed to advance medical research on rare diseases by providing support for clinical studies and facilitating collaboration, study enrollment and data sharing. Through the RDCRN consortia, physician scientists and their multidisciplinary teams work together with patient advocacy groups to study more than 200 rare diseases at sites across the nation.

- **Therapeutics for Rare and Neglected Diseases (TRND):** TRND supports research collaborations among NIH and academic scientists, patient organizations and biopharmaceutical companies to encourage and speed the discovery of new drugs for rare and neglected diseases.

- **Tissue Chip for Drug Screening:** The Tissue Chip program supports the development of 3-D human tissue on microchip platforms that model the structure and function of human organs, such as the lung, liver and heart, with the aim of developing an integrated system that can mimic complex functions of the human body. NCATS conducts the program in collaboration with the Defense Advanced Research Projects Agency and the FDA.

- **Toxicology in the 21st Century (Tox21):** Tox21 is designed to improve toxicity testing methods to enable faster, more efficient evaluation of chemicals' effects on human health. The program is a collaborative effort among NIH — including NCATS and the National Toxicology Program at the National Institute of Environmental Health Sciences — the Environmental Protection Agency and the FDA.