

## Background

The National Center for Advancing Translational Sciences (NCATS) was established in December 2011 to advance the development, testing and implementation of diagnostics and therapeutics across a wide range of human diseases and conditions. NCATS strives to support research to reduce, remove or bypass costly and time-consuming bottlenecks in the therapeutic development pipeline.

By improving the process by which these tools and medicines are developed, NCATS aims to make translational science more efficient, less expensive and less risky. In this way, NCATS complements — and does not compete with — the work of the private sector and the other NIH Institutes and Centers.



NCATS unifies complementary programs in these cross-cutting areas:

### ► Clinical and Translational Science Activities

- **Clinical and Translational Science Awards (CTSA)**, a consortium of medical research institutions working to improve the way clinical and translational research is conducted nationwide

### ► Rare Diseases Research and Therapeutics

- **Office of Rare Diseases Research**, which coordinates and supports research on rare diseases
- **Bridging Interventional Development Gaps**, which provides access to critical pre-clinical resources needed for the development of new therapeutic agents
- **Therapeutics for Rare and Neglected Diseases**, a program to encourage and speed the development of new drugs for rare and neglected diseases

### ► Re-engineering Translational Sciences

- **NIH Chemical Genomics Center**, which provides researchers with access to the large-scale screening and chemistry capacity necessary to identify compounds that can be used as chemical probes to validate new therapeutic targets
- **Discovering New Therapeutic Uses for Existing Molecules**, part of the Rescuing and Repurposing Drugs initiative, matches researchers with a selection of molecular compounds from industry to test ideas for new therapeutic uses
- **Tissue Chips for Drug Screening**, which aims to develop 3-D human tissue chips that accurately model the structure and function of human organs to better predict drug safety
- **Toxicology in the 21st Century (Tox21)**, which is screening a collection of 10,000 compounds to identify potentially toxic disruptions in biological pathways and develop ways to predict toxicity more accurately

In addition, NCATS' **Cures Acceleration Network** enables overarching and flexible support for a variety of initiatives and is designed to address scientific and technical challenges that impede transitional research.

## Innovative Initiatives and Programs

### Clinical and Translational Science Awards

*Providing Critical Infrastructure Support to Strengthen NIH-Funded Clinical and Translational Research*

Research institutions supported through the CTSA program aim to accelerate scientific discovery along the entire biomedical research continuum — from basic science to patient studies to clinical practice — using an integrated approach. Through institutional grants, CTSA-supported organizations provide core resources, essential mentoring and training, and opportunities to develop innovative approaches and technologies designed to enhance and re-engineer existing translational research capabilities. By supporting integrated academic homes for clinical and translational science, this program fosters change to improve the quality, safety, efficiency and speed of clinical and translational research. (<http://ncats.nih.gov/ctsa.html>)

### Tissue Chips for Drug Screening

*Mimicking Human Physiology to Predict Drug Safety and Toxicity*

One of the most common reasons for the failure of new medications is toxicity that was not detected in pre-clinical studies. NCATS is working with the Defense Advanced Research Projects Agency and the Food and Drug Administration (FDA) to develop a better model to predict drug safety and efficacy: chips composed of diverse human cells and tissues that accurately model the structure and function of human organs. If successful, drug safety and efficacy assessments could be conducted more accurately and possibly earlier in the translational pipeline, which would help scientists concentrate on the most promising new drugs. (<http://ncats.nih.gov/tissue-chip.html>)



Wyss Institute Photo

### Rescuing and Repurposing Drugs

*Matching Drug Compounds with NIH-Funded Scientists' Ideas for New Uses*

The most efficient path to new therapeutics is to find novel uses for established drugs that either already are approved (drug repurposing) or unapproved (drug rescuing). Through the Therapeutics Discovery program, NCATS is working with industry to provide academic investigators and small businesses with the funding and information they need to investigate new uses for compounds from industry-provided drug collections. NCATS also is developing a comprehensive database of approved and investigational drugs and working with the FDA to advance opportunities in this promising area. (<http://ncats.nih.gov/therapeutics.html>)



### Toxicology in the 21st Century

*Screening Environmental Chemicals and Drugs for Toxicity*

People are exposed to many different chemicals during their lifetime, from consumer products to food additives to pharmaceutical drugs. The Tox21 program is a collaborative initiative among NIH, the FDA and the Environmental Protection Agency (EPA) to test 10,000 chemical compounds to evaluate their potential to disrupt processes in the human body that may lead to adverse health effects. The compounds undergo testing in the high-throughput robotic screening system at the NCATS Division of Pre-Clinical Innovation. Creating new methods for assessing chemical toxicity has the potential to improve how scientists evaluate environmental chemicals and develop new medicines. The program is administered by all partners and funded by the EPA and the National Toxicology Program, part of NIH's National Institute of Environmental Health Sciences. (<http://ncats.nih.gov/tox21.html>)

### For More Information

Visit the NCATS website at [ncats.nih.gov](http://ncats.nih.gov), contact [info@ncats.nih.gov](mailto:info@ncats.nih.gov) or call 301-435-0888.