

NCATS Drug Repurposing

*Seeking New Treatments
Using Existing Drugs*

Turning a promising molecule into an approved drug often takes at least 10 years and costs an average of \$2.6 billion. At the National Center for Advancing Translational Sciences (NCATS), drug repurposing is a key strategy for bringing more treatments for all people more quickly.

Drug repurposing finds approved drugs and investigational compounds that might be able to treat other diseases and conditions. Approved drugs have already been tested in humans, so some repurposed treatments could enter clinical trials much sooner. They also could be approved for new uses by the U.S. Food and Drug Administration (FDA) faster.

How NCATS Supports Drug Repurposing

NCATS experts use the latest technology to speed the search for existing treatments that can fill unmet health needs. They use robots to rapidly test thousands of drug candidates at the same time. NCATS scientists use advanced computerized methods to quickly analyze data and better predict which repurposed drugs are most likely to work against other diseases.

Repurposing Research Finds Potential Solutions

NCATS' in-house teams collaborate with other researchers to pursue effective, safe treatments for a range of diseases.

- **Niemann-Pick disease type C1 (NPC1):** NCATS created a public-private team to [move a repurposed compound, 2-hydroxypropyl-beta-cyclodextrin \(HPBCD\), into clinical trials](#) for NPC1. This fatal neurodegenerative disease has no FDA-approved therapies.

- **COVID-19 and human cytomegalovirus:** NCATS partnered with a pharmaceutical company to develop the anti-parasite drug emetine as a [treatment for COVID-19](#). NCATS scientists also found emetine to be potentially effective to treat [human cytomegalovirus infection](#).
- **Small cell lung cancer:** NCATS worked with the National Cancer Institute to [find and test in a clinical trial](#) a repurposed drug combination, berzosertib and topotecan, that takes advantage of a weakness in small cell lung cancer.
- **Multidrug-resistant bacterial infections:** NCATS scientists worked with colleagues at the National Institute of Allergy and Infectious Diseases to perform a real-time high-throughput screen to [identify a synergistic combination drug therapy](#) for a patient in the NIH Clinical Center.
- **Beta thalassemia and sickle cell disease:** NCATS worked with partners to advance two repurposed drugs, [benserazide](#) and [decitabine](#), into trials to treat the two related blood disorders.
- **Autoimmune pulmonary alveolar proteinosis:** NCATS repurposed a biologic therapy, granulocyte macrophage colony stimulating factor (GM-CSF), used for blood and bone marrow conditions, into an [inhaled treatment for a rare lung disease](#). This treatment is being tested now in clinical trials by collaborators at Cincinnati Children's Hospital.

NCATS Programs Advance Drug Repurposing

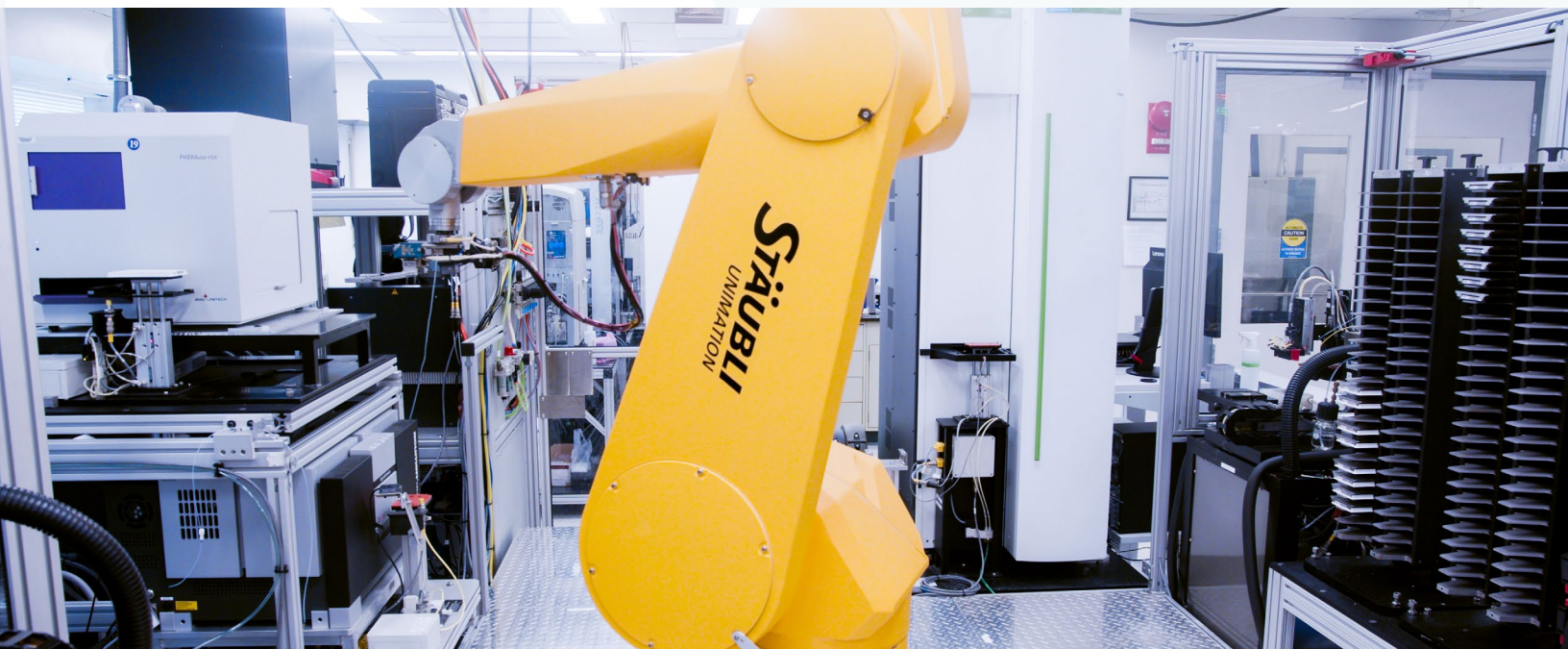
NCATS develops innovative tools and supports clinical trials to help researchers turn existing drugs into new treatments.

- **OpenData Portal for COVID-19:** This [research resource](#) offers real-time data about how different variants of SARS-CoV-2, the virus behind COVID-19, may respond to thousands of approved drugs.
- **ACTIV-6 trial for COVID-19:** This [clinical trial](#) is testing existing prescription and over-the-counter medications to treat symptoms of COVID-19. ACTIV-6 has delivered evidence-based results on the lack of effectiveness with repurposed treatments such as [ivermectin](#), [fluticasone and fluvoxamine](#).
- **Collaborative research template agreements:** NCATS' New Therapeutic Uses program developed [template agreements](#) to start public-private drug repurposing projects sooner. The templates cut project start times from a year or more down to three or four months.

- **The NCATS Pharmaceutical Collection:** The largest library of its kind, the regularly updated [NPC compound library](#) includes nearly 3,000 drugs that are approved for clinical use by U.S., European, Japanese, Canadian and Australian regulatory authorities. NCATS uses the NPC to identify potential new uses for already-approved drugs.
- **Biomedical Data Translator:** The [Translator program](#) integrates vast amounts of biomedical research and clinical health data from across many sources. Scientists can pose questions about drug repurposing opportunities, for example, and Translator will synthesize relevant data to deliver evidence-based answers.

Learn more about NCATS' drug repurposing work at ncats.nih.gov/preclinical/repurpose.

Banner image: Trish Dranchak instructs post-doctoral student James Song (from Anirban Banerjee's NICHD lab) on pipetting techniques for 1536-well plates in NCATS' Assay Development and Screening Technology Lab. (Daniel Soñé Photography)



A high-throughput screening robot in NCATS' laboratories.

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