



## Discovering New Therapeutic Uses for Existing Molecules

### Program Overview

Eighty percent of drugs that enter human clinical testing are never approved for use. One common reason is that clinical studies fail to show effectiveness in treating the disease or condition — the “indications” — the drug was designed to treat.

This high failure rate means there are many existing, partially developed therapeutic candidates (assets) that might be repurposed for use in a different disease indication. The National Center for Advancing Translational Sciences (NCATS) [Discovering New Therapeutic Uses for Existing Molecules](#) (New Therapeutic Uses) program at the National Institutes of Health (NIH) essentially serves as a “matchmaker” to provide academic investigators an unprecedented opportunity to access pharmaceutical industry agents and explore new ways to treat disease. [Launched in 2012 as a pilot initiative](#), the New Therapeutic Uses approach could produce new treatments for patients more quickly than starting from scratch.

To facilitate the required public-private partnerships, NCATS uses [template agreements](#) to help streamline legal and administrative processes needed for research collaborations across multiple organizations. The agreements already have reduced time, cost and effort and enabled greater participation than traditional partnerships. For example, during the pilot program, the templates helped shorten the time required to establish collaborations to about three months instead of the more typical nine months to one year.

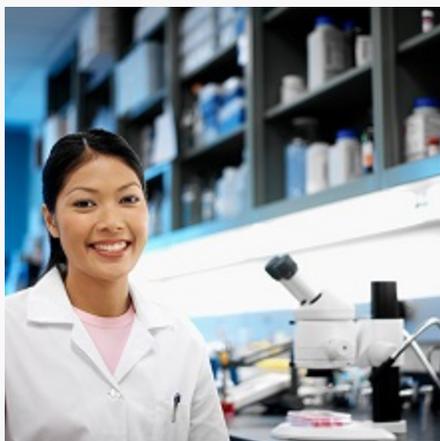
### A New Phase of the Program

In early 2014, NCATS collaborated with AstraZeneca, Janssen Research & Development, L.L.C., Pfizer Inc., and Sanofi to make 26 [assets](#) available to researchers to crowdsource ideas for new uses. In May 2014, NCATS issued a set of [funding opportunity announcements](#) asking U.S.-based researchers for short proposals describing how they would investigate specific hypotheses using an agent available through this program to explore a specific disease area.

In July 2015, NCATS [awarded nearly \\$3 million](#) to fund cooperative agreements with four academic research groups to conduct pre-clinical validation, clinical feasibility studies, or proof-of-concept clinical trials to test whether the selected agents may be effective against a previously unexplored disease target.

The [projects](#) cover four disease areas: type 2 diabetes, acute myeloid leukemia (an aggressive blood cancer), glioblastoma (one of the most aggressive brain tumors in adults) and Chagas disease (a neglected tropical disease causing heart, digestive and neurological problems). As with the projects from the pilot program, the partner companies provide the drug and matched placebo as well as data and other resources at no cost to NCATS or the academic institutions. The companies also provide suitable documentation so funded investigators can file an Investigational New Drug application with the Food and Drug Administration. Other NIH Institutes and Centers provide scientific expertise on the projects. Summary information about all available assets is listed at <https://ncats.nih.gov/ntu/assets/current>.

[ncats.nih.gov/ntu](http://ncats.nih.gov/ntu)

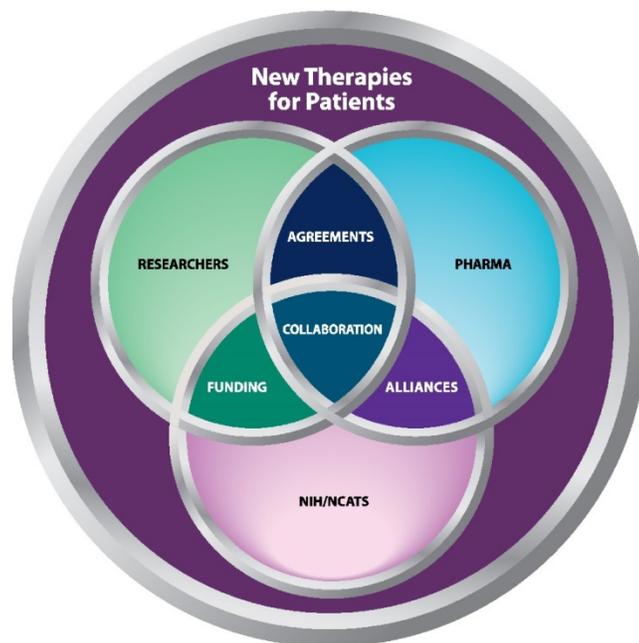


## Building on a Successful Pilot

This latest round of the New Therapeutic Uses program is aimed at building on the successes of the pilot initiative. In June 2013, as part of the pilot, NIH [awarded \\$12.7 million](#) to nine academic research groups for [projects](#) to explore new treatments for patients in eight disease areas: alcoholism, Alzheimer's disease, calcific aortic valve stenosis (a condition in which the heart valve hardens and makes it difficult to pump blood out of the heart), Duchenne muscular dystrophy, lymphangi leiomyomatosis (a progressive lung disease), nicotine dependence, peripheral artery disease and schizophrenia. Within three months of receiving funds, researchers already were testing three compounds in humans for new uses, including potential treatments for schizophrenia (two agents) and Alzheimer's disease.

The Alzheimer's disease project team at Yale University found that a [compound originally developed as a cancer therapy could be used to treat Alzheimer's disease](#).

The Yale scientists gave the compound to mice with Alzheimer's disease. After four weeks, the mice showed reversal of Alzheimer's symptoms such as spatial learning impairments and memory loss. The drug already had been tested for safety in humans and passed key steps in the development process. By repurposing an existing drug, investigators began testing the drug in humans within three months, whereas it would typically take a decade from the discovery of a promising compound to its readiness for clinical trials.



## For More Information

### Contact

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### Projects

[ncats.nih.gov/ntu/projects](http://ncats.nih.gov/ntu/projects)

### Funding Information

[ncats.nih.gov/ntu/funding](http://ncats.nih.gov/ntu/funding)

### Frequently Asked Questions

[ncats.nih.gov/ntu/about/faq](http://ncats.nih.gov/ntu/about/faq)