



Discovering New Therapeutic Uses for Existing Molecules

Program Overview

Treatments currently are available for fewer than 10 percent of the more than 6,500 human diseases. The painful reality is that the average length of time from target discovery to approval of a new drug is currently about 14 years, with a failure rate exceeding 95 percent and the cost per successful drug \$2 billion or more.

The high failure rate of therapeutic development means there are many existing, partially developed therapeutic candidates — “agents” — that might be repurposed for use in a new disease indication. The National Center for Advancing Translational Sciences (NCATS) at the National Institutes of Health (NIH) has developed a program that brings together the best agents from pharmaceutical companies with the best new disease ideas from academic researchers. This strategy has the potential to produce new treatments much more quickly than starting from scratch by accelerating the pace at which basic research discoveries are transformed into new therapies for patients.

To explore this new approach, the [Discovering New Therapeutic Uses for Existing Molecules](#) (New Therapeutic Uses) program was launched as a pilot initiative in 2012. New Therapeutic Uses essentially serves as a “matchmaker” to provide academic investigators an unprecedented opportunity to access pharmaceutical industry agents and explore new ways to treat disease.

To encourage and speed development of these public-private partnerships, NCATS uses [template agreements](#). These agreements help streamline legal and administrative processes required for research collaborations across multiple organizations by reducing time, cost and effort and enabling greater participation than traditional partnerships. 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.

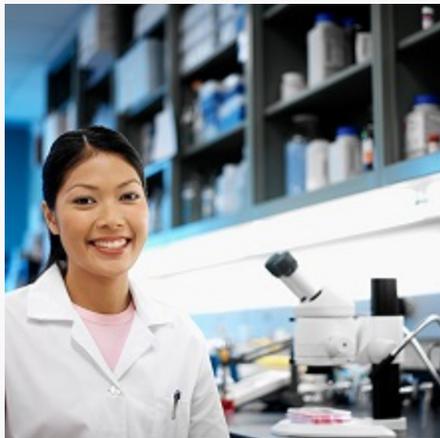
How to Get Involved

In early 2014, NCATS collaborated with AstraZeneca, Janssen Research & Development, L.L.C., Pfizer Inc., and Sanofi to make 26 [agents](#) available to researchers to crowdsource ideas for new uses. In May, NCATS issued a set of [funding opportunity announcements](#) (FOAs) 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. Through these FOAs, NIH will support projects that can go directly into studies that provide data on the relationship of dosing and response for the intended use (Phase IIa) and also some projects that may need additional pre-clinical and/or feasibility studies conducted within the target populations (Phase Ib).

For the first time, agents suitable for exploration of pediatric indications are included in the program. NIH will provide an extra year of support (as compared with those for adult indications) to complete additional studies that evaluate safety, dosage and side effects in healthy volunteers (Phase Ia) and juvenile toxicity studies required before pediatric trials can begin. Summary information about all available agents is listed at www.ncats.nih.gov/therapeutics-directory.html.



ncats.nih.gov/therapeutics.html



Participating companies provide pre-clinical and clinical supplies of drugs and placebos to funded investigators. They also provide suitable documentation so funded investigators can file an Investigational New Drug application with the Food and Drug Administration (FDA).

To be eligible for funding, U.S. investigators must submit a short pre-application (X02) by July 15, 2014, to undergo NIH review. The investigators submitting those applications identified as highly meritorious and relevant to NCATS program priorities will receive contact information for the appropriate pharmaceutical company based on the agent or mechanism of action identified in their pre-application.

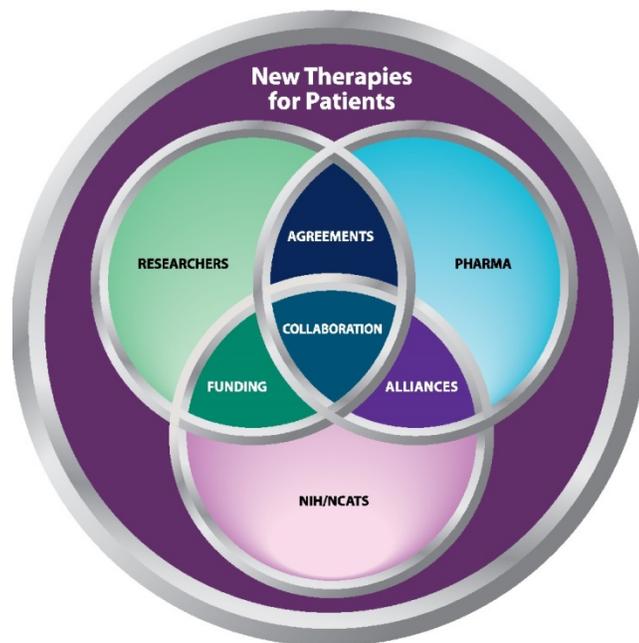
Applicants then will sign confidential disclosure and collaborative research agreements with the industry partner to obtain detailed information about the specific agent of interest in order to develop a full application (UH2/UH3 or UH3) with input from the pharmaceutical partner.

Full applications, due Jan. 16, 2015, will undergo NIH peer review, and funding selections will follow. Cooperative agreements will be awarded in summer 2015. In addition to NCATS, multiple NIH Institutes and Centers and the FDA Office of Orphan Products Development are contributing funding for these FOAs.

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, lymphangioliomyomatosis (a progressive lung disease), nicotine dependence, peripheral artery disease and schizophrenia. Within three months of receiving funds, three compounds already were being tested in humans for new uses, including potential treatments for schizophrenia (two agents) and Alzheimer's disease.

"The progress we have made on our collaborative projects is remarkable given the complexities of initiating clinical trials," said AstraZeneca Vice President of Emerging Innovations, Scientific Partnering and Alliances Don Frail. "The knowledge brought by the investigators, together with the tools, resources and expertise made available by our company, allowed the projects to move forward very quickly, and quality data have already emerged."



For More Information

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2013 Projects

ncats.nih.gov/therapeutics-projects.html

Funding Information

ncats.nih.gov/therapeutics-funding.html

Frequently Asked Questions

ncats.nih.gov/therapeutics-faq.html