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National Center for Advancing Translational Sciences (NCATS) <http://ncats.nih.gov/>
NIH Common Fund <http://commonfund.nih.gov>
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NIH TO FUND COLLABORATIONS WITH INDUSTRY TO IDENTIFY NEW USES FOR EXISTING COMPOUNDS

Crowdsourcing initiative will enable exploration of potential treatments in eight disease areas

The National Institutes of Health has awarded $12.7 million to match nine academic research groups with a selection of pharmaceutical industry compounds to explore new treatments for patients in eight disease areas, including Alzheimer's disease, Duchenne muscular dystrophy and schizophrenia. The collaborative pilot initiative, called Discovering New Therapeutic Uses for Existing Molecules <http://www.ncats.nih.gov/research/reengineering/rescue-repurpose/therapeutic-uses/therapeutic-uses.html>, is led by the National Center for Advancing Translational Sciences (NCATS) and funded by the NIH Common Fund.

The process of developing a new therapeutic is long and difficult. The average length of time from target discovery to approval of a new drug is more than 13 years, and the failure rate exceeds 95 percent. This failure rate means, however, that many existing partially developed compounds could be advanced to clinical trials more quickly than starting from scratch.

"With thousands of diseases remaining untreatable, there is a sense of urgency to accelerate the pace at which discoveries are transformed into therapies for patients," said Health and Human Services Secretary Kathleen Sebelius. "This program helps forge partnerships between the pharmaceutical industry and the biomedical research community to work together to tackle problems that are beyond the scope of any one organization or sector."

"Innovative, collaborative approaches that improve the therapeutic pipeline are crucial for success," said NIH Director Francis S. Collins, M.D., Ph.D. "This unique collaboration between academia and industry holds the promise of trimming years from the long and expensive process of drug development."

AbbVie (formerly Abbott); AstraZeneca; Bristol-Myers Squibb Company; Eli Lilly and Company; GlaxoSmithKline; Janssen Research & Development, LLC; Pfizer; and Sanofi are participating in the pilot phase of the program.

NCATS launched this initiative in 2012 to help re-engineer the research pipeline using an innovative strategy to identify new uses for compounds that have undergone significant research and development by industry, including safety testing in humans. The center crowdsourced the industry compounds to academic researchers nationwide to gain ideas for new therapeutic uses with the ultimate goal of developing new treatments for patients.

The program also tested newly created template agreements <http://www.ncats.nih.gov/research/reengineering/rescue-repurpose/therapeutic-uses/agreements.html>, which enabled negotiations to be completed in fewer than 11 weeks, versus a typical timeline of a year or more.

“Public-private collaborations are crucial for successful translation; no one organization can succeed alone,” said NCATS Director Christopher P. Austin, M.D. “This initiative has created a marketplace to connect academic researchers with potential new drugs, as well as template agreements that streamline the process by limiting the amount of negotiation required before a project can begin.”

Each award recipient will test a selected compound for its effectiveness against a previously unexplored disease or condition. The eight disease areas represented are alcohol dependence, 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), nicotine dependence, peripheral artery disease, schizophrenia and two rare diseases: Duchenne muscular dystrophy and the progressive lung disease lymphangioleiomyomatosis. For more details about each project, please visit <http://www.ncats.nih.gov/therapeutics-projects.html>.
The projects, award recipient principal investigators and industry partners are:

The efficacy and safety of a selective estrogen receptor beta agonist (LY500307)
Academic Partner: Alan Breier, M.D., Indiana University, Indianapolis
Industry Partner: Eli Lilly and Company

Fyn inhibition by AZD0530 for Alzheimer’s disease
Academic Partners: Stephen M. Strittmatter, M.D., Ph.D.; Haakon Berge Nygaard, M.D., Ph.D.; and Christopher H. Van Dyck, M.D., Yale University, New Haven, Conn.
Industry Partner: AstraZeneca

Medication development of a novel therapeutic for smoking cessation
Academic Partners: Darlene H. Brunzell, Ph.D., Virginia Commonwealth University, Richmond and Kenneth Alan Perkins, Ph.D., University of Pittsburgh
Industry Partner: Janssen Research & Development, LLC

A novel compound for alcoholism treatment: A translational strategy
Academic Partner: Fatemeh Akhlaghi, Pharm.D., Ph.D., University of Rhode Island, Kingston

NIH Intramural Partner: Lorenzo Leggio, M.D., Ph.D., M.Sc., National Institute on Alcohol Abuse and Alcoholism; National Institute on Drug Abuse
Industry Partner: Pfizer

Partnering to treat an orphan disease: Duchenne muscular dystrophy
Academic Partners: Kathryn R. Wagner, M.D., Ph.D., Kennedy Krieger Institute, Baltimore and Stanley C. Froehner, Ph.D., University of Washington, Seattle
Industry Partner: Sanofi

Reuse of ZD4054 for patients with symptomatic peripheral artery disease (PAD)
Academic Partner: Brian H. Annex, M.D., University of Virginia, Charlottesville
Industry Partner: AstraZeneca

Therapeutic strategy for lymphangioleiomyomatosis (LAM)
Academic Partner: N. Tony Eissa, M.D., Baylor College of Medicine, Houston
Industry Partner: AstraZeneca

Therapeutic strategy to slow progression of calcific aortic valve stenosis
Academic Partners: Jordan D. Miller, Ph.D.; Maurice Enriquez-Sarano, M.D.; and Hartzell V. Schaff, M.D., Mayo Clinic, Rochester, Minn.
Industry Partner: Sanofi

Translational neuroscience optimization of GlyT1 inhibitor for cognitive impairments associated with schizophrenia
Academic Partner: John H. Krystal, M.D., Yale University, New Haven, Conn.
Industry Partner: Pfizer

These cooperative agreements will fund projects for researchers to conduct pre-clinical validation and additional safety studies as needed. If specific milestones are met, clinical feasibility studies or proof-of-concept clinical trials will be initiated to test whether the selected compounds may be effective as treatments for other diseases. The projects will be supported for up to three years.

NCATS leads this effort with additional scientific expertise provided by the National Cancer Institute; National Heart, Lung, and Blood Institute; National Institute on Aging; National Institute on Alcohol Abuse and Alcoholism; National Institute on Drug Abuse; National Institute of Mental Health; and the National Institute of Neurological Disorders and Stroke.

For more information about the Discovering New Therapeutic Uses for Existing Molecules pilot initiative, visit <http://ncats.nih.gov/therapeutics.html>. To submit comments regarding the pilot phase of the program, see the RFI at <http://grants.nih.gov/grants/guide/notice-files/NOT-RM-13-021.html>.

The National Center for Advancing Translational Sciences (NCATS) is a distinctly different

entity in the research ecosystem. Rather than targeting a particular disease and fundamental science, NCATS focuses on what is common across diseases and the translational process. The Center emphasizes innovation and deliverables, relying on the power of data and new technologies to develop, demonstrate and disseminate improvements in translational science. For more information, visit <http://www.ncats.nih.gov>.

The NIH Common Fund encourages collaboration and supports a series of exceptionally high-impact, trans-NIH programs. Common Fund programs are designed to pursue major opportunities and gaps in biomedical research that no single NIH Institute could tackle alone, but that the agency as a whole can address to make the biggest impact possible on the progress of medical research. Additional information about the NIH Common Fund can be found at http://commonfund.nih.gov.

About the National Institutes of Health (NIH): NIH, the nation's medical research agency, includes 27 Institutes and Centers and is a component of the U.S. Department of Health and Human Services. NIH is the primary federal agency conducting and supporting basic, clinical, and translational medical research, and is investigating the causes, treatments, and cures for both common and rare diseases. For more information about NIH and its programs, visit www.nih.gov.

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ADDITIONAL QUOTES ABOUT DISCOVERING NEW THERAPEUTIC USES FOR EXISTING MOLECULES

ACADEMIA

Stephen M. Strittmatter, M.D., Ph.D., Vincent Coates Professor of Neurology and Professor of Neurobiology; Director, Cellular Neuroscience, Neurodegeneration and Repair; Director, Adler Memory Clinic in Neurology, Yale School of Medicine: “The NCATS team was crucial in bringing together our group at Yale, together with AstraZeneca, to test the efficacy of this drug in Alzheimer’s disease. Now, we’re beginning a trial, together with AstraZeneca, in order to find out whether this drug can slow down the loss of memory and preserve memory function in the millions of Americans suffering with this disease.”

Kathryn R. Wagner, M.D., Ph.D., Director, Center for Genetic Muscle Disorders, Kennedy Krieger Institute: “Duchenne muscular dystrophy is a devastating disorder. It’s the most common muscular dystrophy and it’s one of the most common genetic disorders of mankind. This is a really unique program in which academic investigators have been given access to drugs in the pharmaceutical private sector.
The program offers the potential for novel treatments in a short period of time to make a real difference to the lives of those with disease.”

INDUSTRY

Freda Lewis-Hall, M.D., Chief Medical Officer, Pfizer: “In many cases, the molecules we already have might — with new knowledge, science and information — be found to be appropriate for the treatment of other diseases. This is a wonderful opportunity because we already know a lot about them, we understand what some of the effects are, we’ve seen them in other environments.”

Jan Lundberg, Ph.D., Executive Vice President, Science and Technology, and President, Lilly Research Laboratories: “We are delighted to be collaborating with the NIH and Indiana University to explore potential new treatments for people with schizophrenia, a terrible illness. Public private partnerships such as this bring great scientists together to help solve today’s tough biomedical challenges and can help to accelerate breakthroughs for patients who are waiting.”

PATIENT ADVOCATES

Margaret A. Anderson, Executive Director, FasterCures: “NCATS is unique in that it can offer a safe space for experimentation in terms of how you bring together different partners and sectors to forge collaborative models. There’s a real hunger and a thirst right now for these models; for figuring out the key that’s going to turn the lock.”

Todd Sherer, Ph.D., Chief Executive Officer, Michael J. Fox Foundation for Parkinson’s Research: “The translational research process has a lot of room for improvement. While there definitely are examples of successes that we’re having even today — new treatments that are coming on the market — it’s clear that this can happen in a more efficient way, with less expense. NCATS is focused on promoting collaborative science in order to optimize opportunities and recognizes that we need a balance between collaboration and competition.”

GOVERNMENT

Francis S. Collins, M.D., Ph.D., Director, National Institutes of Health: “Developing a drug takes time and money; about 95 percent of the drugs fail during development. Even those that have been extensively tested and proven safe in humans sometimes don’t make the cut; perhaps because they were not quite as effective as they were supposed to be or had undesirable side effects. But some of these compounds may have surprising therapeutic properties that have not yet been recognized — it would be a shame to not take another look at these drugs and test them for other conditions.”

Lorenzo Leggio, M.D., Ph.D., M.Sc., Chief, Clinical Psychoneuroendocrinology and Neuropsychopharmacology Section, Intramural Research Program of the National Institute on Alcohol Abuse and Alcoholism and the National Institute on Drug Abuse, National Institutes of Health: “Alcoholism is a leading cause of mortality and morbidity with limited

treatment options. Our translational research may contribute by testing whether targeting the ghrelin receptor may be a new treatment approach for alcoholism. The NCATS program will facilitate a unique cross-talk between academia and NIH with pharmaceutical industry, allowing the collaboration of an extraordinary team with different but complementary backgrounds."

The html version of this release contains a video of Dr. Kathryn Wagner, M.D., Ph.D., Kennedy Krieger Institute, Baltimore on Duchenne muscular dystrophy and how the New Therapeutic Uses program can make a difference in potentially accelerating a treatment for this devastating disorder:
<http://www.youtube.com/watch?v=C4XOI23v0SA&feature=youtu.be>

The web version also contains an audio clip of Dr. Jordan Miller, Ph.D., Mayo Clinic, Rochester, Min., on his team's research effort to test the safety of an investigational drug in heart valve disease patients in slowing progression of stenosis, an abnormal narrowing of vessels or other structures at <http://www.ncats.nih.gov/files/Miller.mp3> and <http://www.ncats.nih.gov/files/Miller.ogg>

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