

U.S. NEWS MAY 21, 2009, 9:47 A.M. ET

NIH Launches Drug-Development Program for Rare Diseases

By [JENNIFER CORBETT DOOREN](#)

WASHINGTON -- The National Institutes of Health is wading into the drug-development process by launching a program aimed at finding and getting new drugs ready for testing in humans with rare diseases.

The development program would also target so-called neglected diseases, while rare in the U.S., are often linked to parasites that sicken millions of people who live in tropical parts of the world.

"We've never tried to directly develop medicines for rare and neglected diseases," said NIH Acting Director Raynard Kington, who announced the program Wednesday. NIH has traditionally engaged in basic science and has only had limited programs for developing drugs.

The program comes with new money from Congress and is aimed at finding new treatments for some of the 6,800 rare diseases that collectively affect about 25 million Americans. Of those diseases only 200 have treatments, according to NIH.

Each disease might only affect a few hundred or few thousand people, making it unprofitable for pharmaceutical companies to work on such treatments. It typically takes about \$10 million, and two to four years, to move a compound through the process of discovery and making it into a form that can be tested in the laboratory and later animals before asking the Food and Drug Administration for permission to test a drug in humans. Up to 90% of possible drug candidates fail in the pre-clinical stage.

"We will be de-risking these projects sufficiently to make them adoptable by private industry," said Christopher Austin, the director of NIH's Chemical Genomics Center, which will be involved in research. He and other NIH officials said the goal is to work with private industry, not compete against it.

The project, known as Therapeutics for Rare and Neglected Diseases Program, will be overseen by the NIH Office of Rare Diseases Research.

Stephen Groft, the director of the rare diseases office, said the program is starting with \$24 million in funding this year with expectation of receiving the same amount each year until 2013, which effectively means the program would only be able to focus on a couple of compounds per year.

"Preclinical work is hard and our resources will be limited," he said. However, he said other funds from NIH centers and existing programs can be used to augment the broader drug-development program.

Groft said NIH would work with researchers and patient advocacy groups to identify possible molecules that could be developed into a potential treatment. The goal of the program is to turn over potential new medicines to private companies for further development. Information about chemicals that failed in tests will be published.

One target under consideration for the NIH drug-development program is a potential new treatment for schistosomiasis, a parasitic disease that affects about 200 million people world-wide and can cause internal bleeding and organ damage. In 2008 a team of researchers from NIH and Illinois State University wrote about chemicals that appear to inhibit an enzyme that's needed by the parasites that spread the disease to survive. So far the chemicals, known as oxadiazoles, have been tested in the laboratory and in mice but funding ran out for further testing.

Write to Jennifer Corbett Dooren at jennifer.corbett-dooren@dowjones.com

Copyright 2009 Dow Jones & Company, Inc. All Rights Reserved



This copy is for your personal, non-commercial use only. Distribution and use of this material are governed by our [Subscriber Agreement](#) and by copyright law. For non-personal use or to order multiple copies, please contact Dow Jones Reprints at 1-800-843-0008 or visit www.djreprints.com