NIH Therapeutics for Rare and Neglected Diseases Program

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What is the Therapeutics for Rare and Neglected Diseases (TRND) program?

The National Institutes of Health (NIH) Therapeutics for Rare and Neglected Diseases (TRND) program is a congressionally mandated effort to encourage and speed the development of new drugs for rare and neglected diseases. The federal budget for fiscal 2009 dedicated $24 million to establish this initiative.

TRND will bridge the wide gap in time and resources that often exists between basic research and human testing of new drugs. The effort is grounded in, but aims to improve upon, existing processes for drug development in the pharmaceutical industry.

How is TRND structured?

The NIH Office of Rare Diseases Research (ORDR) will handle oversight and governance of TRND. Researchers will perform TRND's laboratory work in a new facility administered by the intramural program of the National Human Genome Research Institute (NHGRI).

TRND will build upon the successes of the similarly structured NIH Chemical Genomics Center (NCGC). NCGC facilitates drug development from the basic research lab to the pre-clinical stage, which is when researchers begin to lay the groundwork for possible human testing of candidate drugs.

Picking up where NCGC's work leaves off, TRND will concentrate its efforts on the pre-clinical stage of drug development. TRND's aim will be to move candidate drugs forward in the drug development pipeline until they meet Food and Drug Administration (FDA) requirements for an Investigational New Drug (IND) application. Once TRND generates enough data to support an IND application for a candidate drug, the drug would then be handed off to an experienced organization outside of NIH, such as a pharmaceutical company, for human testing and other aspects of clinical development. There may be situations in which TRND starts or remains involved in human studies.

Like NCGC, TRND will pull together researchers with expertise in a broad and diverse range of scientific disciplines and disease areas. Specifically, TRND will encourage investigators from both inside and outside of NIH to submit projects for work within its

intramural facility. This will create ongoing collaborations that will benefit researchers and, most importantly, patients with rare and neglected diseases.

What are TRND's goals?

TRND will strive to develop candidate drugs for rare and neglected diseases that meet FDA requirements for an IND application, which is necessary for human testing. TRND expects to license most of its IND-worthy candidate drugs to biopharmaceutical companies for clinical development.

In addition, TRND will seek to advance the entire field of drug development by encouraging scientific and technological innovations aimed at improving success rates in the crucial pre-clinical stage of development.

What are TRND's activities?

TRND will focus on the pre-clinical stage of drug development that is vital to meeting the FDA's requirements for the human testing of new drugs. TRND's activities will include:

- Optimizing the chemical structure of promising drug compounds to maximize their activity and minimize their toxicities.
- Testing optimized compounds in the safety and efficacy models required by the FDA.
- Testing drugs currently approved by the FDA in cells and animals to see if they might have previously unidentified potential for treating rare or neglected diseases.

What diseases will TRND initially target?

TRND plans to conduct pilot projects in fiscal 2009 in both rare and neglected diseases. These projects will include testing the feasibility of optimizing novel compounds and the possibility of re-purposing drugs already approved for other diseases.

NIH has not yet chosen the exact diseases and candidate compounds to be focused on by the TRND pilot projects. However, some will be selected from compounds identified by basic research projects that have been successfully completed at NCGC. Criteria will include quality of the candidate drug compounds, the clarity of the path to clinical testing, and the commitment and enthusiasm of collaborating researchers.

As it ramps up, TRND will add more projects in fiscal 2010. Eventually, NIH leadership envisions expanding TRND's work to encompass many more rare and neglected diseases. For more information on rare diseases, go to www.genome.gov/27531963. For more information on neglected diseases, go to www.genome.gov/27531964.

Where will TRND be located and who will manage it?

The location of TRND's laboratories has not yet been determined. TRND will have state-of-the-art equipment for medicinal chemistry, biological assays, bioinformatics, pharmacology and toxicology testing.

The scientists who will manage and coordinate TRND activities will be experienced, high-level experts from pharmaceutical and biotechnology organizations. These scientists will not perform their own independent research, but will work together with researchers from outside of NIH to translate their basic research findings into candidate drugs for patients with rare and neglected diseases.

TRND will initially model its infrastructure and staffing on best practices in the pharmaceutical and biotechnology industries, pulling together experience from many different companies to develop its own innovative paradigm. The program will also capitalize upon the many human, intellectual and technological resources available at NIH that are not easily accessed by industry.

Where can I learn more about TRND?

To learn more about TRND, go to the ORDR web site at http://rarediseases.info.nih.gov/TRND.