



Therapeutics for Rare and Neglected Diseases

Program Overview

There are more than 6,500 identified rare and neglected diseases, yet only about 250 treatments are available for these conditions. One reason is that the limited numbers of patients can make gathering information and designing drug studies difficult. As a result, scientists often know little about the symptoms and biology of these conditions. Another reason is that some private companies may find it difficult to justify the cost of developing drugs for such small rare disease markets. Even in the case of infectious diseases — such as malaria — that inflict health burdens on large numbers of people in the developing world, the private sector often neglects therapeutic development because of insufficient economic incentives.

The Therapeutics for Rare and Neglected Diseases (TRND) program, led by NCATS, is designed to combat these challenges. Like all NCATS programs, TRND supports the development of new technologies and more efficient paradigms for translation in the context of important unmet medical needs.

The TRND program is grounded in partnerships with academic, government, pharmaceutical and patient advocacy groups. NCATS researchers work with collaborators to advance potential treatments for rare and neglected diseases to first-in-human trials, an approach known as “de-risking.” This strategy can make potential new drugs more commercially viable and attractive to outside partners, which can invest in their further development and additional clinical trials.

Spanning the Pre-Clinical Spectrum

TRND support spans the pre-clinical spectrum, including medicinal chemistry optimization, drug metabolism and pharmacokinetics, toxicology, formulation, and other studies required to achieve Investigational New Drug (IND) application clearance with the Food and Drug Administration. In limited cases, the program may support first-in-human clinical studies.

Through TRND, NCATS helps close the gap between basic research and clinical testing of new drugs. The current portfolio of [projects](#) involves a wide array of diseases and partnerships with renowned academic hospitals, patient advocacy groups, pharmaceutical and biotechnology companies, and other government agencies. Ongoing research includes focuses on hereditary conditions such as Duchenne muscular dystrophy and Niemann-Pick disease type C, cancers including chronic lymphocytic leukemia, and parasitic diseases such as schistosomiasis. Through TRND’s accelerated development process, some past projects have gone from demonstration of lead compound activity to the dosing of the first patient in a clinical trial in as little as two years.



ncats.nih.gov/trnd.html



How to Apply and Collaborate

NCATS' TRND program provides collaborators with access to significant in-kind resources and expertise to develop new therapeutics for rare and neglected diseases. No monetary funds are awarded. Academic, nonprofit foundation, industry and other government agency representatives from within and outside of the United States are eligible for TRND program support. In general, pre-clinical expertise and regulatory resources are available to support the development of promising, well-validated therapeutic candidates from as early as lead compound optimization through submission of the IND application.

Applications to the program are accepted only during specified solicitation periods through the online [proposalCENTRAL](#) application system. Potential applicants undergo initial pre-application screening, and submitted proposals are evaluated rigorously by an external panel of drug development experts as well as by TRND staff. All applicants receive detailed feedback about specific strengths and weaknesses of their proposals. Once collaborators have been chosen, a project team forms and a project plan is developed, including a timeline, milestones, deliverables and "go/no-go" decision points.

About NCATS and Translational Science

NCATS is one of 27 Institutes and Centers at the National Institutes of Health. The Center was established to transform the translational process so that new treatments and cures for disease can be delivered to patients faster.

Translation is the process of turning observations in the laboratory, clinic and community into interventions that improve the health of individuals and the public — from diagnostics and therapeutics to medical procedures and behavioral changes.

Translational science is the field of investigation focused on understanding the scientific and operational principles underlying each step of the translational process. NCATS studies translation as a scientific and operational problem.

NCATS focuses not on specific diseases, but on what is common among them. The Center serves as an adaptor to enable other parts of the research system to work more effectively.

Through its cross-cutting programs in rare diseases, translational technologies, strategic alliances and other areas, NCATS is:

- Developing new approaches, technologies, resources and models;
- Demonstrating their usefulness; and
- Disseminating the data, analysis and methodologies to the community.

For More Information

Contact

301-435-0888
trnd@mail.nih.gov

About TRND

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TRND Projects

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Frequently Asked Questions

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