

NCATS

Improving Health Through Smarter Science

Therapeutics for Rare and Neglected Diseases (TRND) Program

There are several thousand identified rare and neglected diseases, of which only a few hundred have any available treatments. Some obstacles to developing treatments include:

- Difficulties in diagnosis,
- Widely dispersed patients and scientific experts and
- A perception of high economic risk in developing drugs that would serve either a relatively small population with a rare disease or a larger population suffering from a common disease that primarily affects developing countries.

NCATS is tackling these challenges by working to identify common elements among rare and neglected diseases to get more treatments to more patients more quickly.

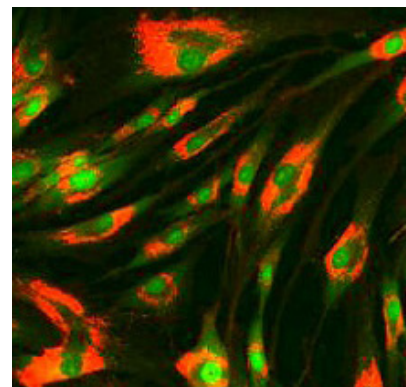
The TRND program is grounded in partnerships with academic, government, pharmaceutical and patient advocacy groups. NCATS researchers work with collaborators to advance potential treatments for rare and neglected diseases to first-in-human trials, an approach known as “de-risking.” This strategy can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

Spanning the Pre-Clinical Spectrum

TRND research 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 conduct first-in-human clinical studies.

Through TRND, NCATS helps close the gap between basic research and clinical testing of new small molecule and biologic drugs as well as cell-based and gene therapies. The portfolio of **projects** involves a wide array of diseases and partnerships with renowned academic hospitals, patient advocacy groups, pharmaceutical and biotechnology companies, and other NIH Institutes and Centers and government agencies. Research focuses on diverse hereditary conditions, such as Duchenne muscular dystrophy, Pompe disease and β -thalassemia, and infectious diseases such as schistosomiasis and malaria. 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.

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How to Collaborate

TRND program scientists seek to collaborate with academic, nonprofit, industry and other government researchers from within and outside the United States. By collaborating with TRND scientists, partners have access to significant pre-clinical expertise to develop new therapeutics for rare and neglected diseases. No monetary support is awarded. In general, TRND supports the development of promising, well-validated small molecule and biological therapeutic candidates from as early as lead compound optimization through submission of the IND application.

Collaborations are initiated through a proposal submission and evaluation process. Interested investigators undergo initial pre-proposal screening, and proposals are evaluated rigorously by TRND staff, with additional input from scientists in other NIH Institutes and Centers and from external drug development experts. Once collaborators have been chosen, a project team is established, 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.

Rather than targeting a particular disease or fundamental science, NCATS focuses on what is common across diseases and the translational process. 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.

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For More Information

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Frequently Asked Questions

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