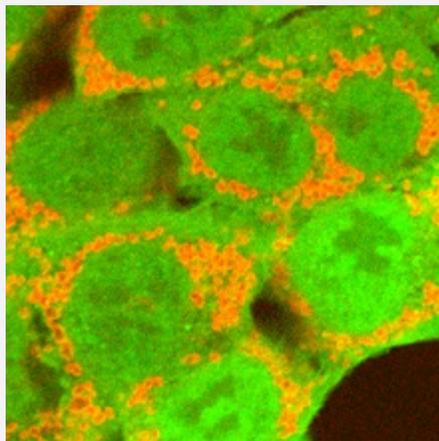


ncats.nih.gov/rnai



RNA Interference

Program Overview

Small interfering RNA (siRNA) molecules are pieces of RNA that block the activity of genes through a natural process called RNA interference (RNAi). RNAi has emerged as a powerful tool used in thousands of labs worldwide to understand gene function. By blocking or modulating a gene's function, RNAi can tell us about the role of any gene in maintaining health or causing disease, an invaluable step in identifying potential drug targets. In tests called genome-wide RNAi screens, scientists use automation to introduce siRNAs into human cells to block the activity of each gene, one at a time. This process can produce a complete list of all genes involved in a particular biological function or disease process. Scientists also can use these techniques to understand what roles genes play in drug effectiveness.

RNAi's potential usefulness has been limited by the lack of expertise to perform genome-wide RNAi screens, the lack of methodologies that can properly interpret these experiments and the absence of comprehensive RNAi data in public databases for researchers to reference. To address these problems, NCATS operates a state-of-the-art RNAi screening facility, and NCATS staff assists NIH intramural investigators with all stages of project planning and execution.

The initiative provides public access to RNAi data generated from these experiments through the National Library of Medicine's PubChem database. In addition, siRNA sequence information is available from private-sector biotechnology partners. For instance, researchers can access Life Technologies' Silencer® Select siRNA library, which includes 65,000 siRNA sequences that target more than 20,000 human genes.

The goal is to speed scientific discoveries in all disease areas.

Performing High-Throughput RNAi Screens

The RNAi high-throughput screening facility, administered by NCATS' Division of Pre-Clinical Innovation staff, offers a robotic platform with integrated, automated devices for conducting all aspects of screening assays (tests), including manipulating chemicals and cells, reading the results and imaging the cells. Offline (non-robotic) devices can perform smaller-scale work from assay optimization through medium-scale screening. Investigators have the option of using several different siRNA libraries and other small molecules involved in RNAi. For data analysis, the facility offers powerful computational tools.

In addition to enabling collaborations on specific projects, RNAi facility staff work to develop methods that advance the science of RNAi screening and informatics and pursue new technologies for exploring gene function. Currently, NCATS scientists are working on developing a platform that offers complementary high-throughput genomic technologies to advance the field of functional genomics.

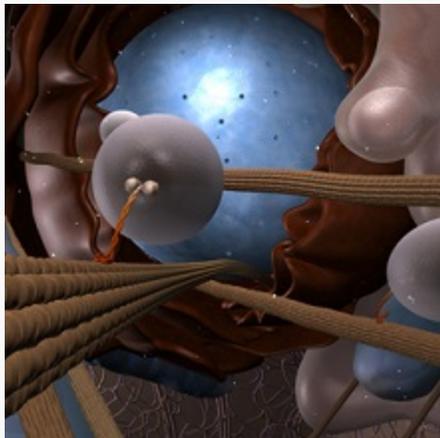


U.S. Department of Health
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National Institutes of Health

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For More Information

RNAi Proposal Contact

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RNAi Projects

ncats.nih.gov/rnai/projects

Data Access

pubchem.ncbi.nlm.nih.gov

Data Submission Help

info@ncbi.nlm.nih.gov

Work with Us

NIH investigators are eligible to collaborate with NCATS to access its RNAi screening facility resources. Open application cycles occur once each year and last about two months. Those interested in learning more should contact [Anna Rossoshek](#).

Researchers and biotechnology companies may contribute RNAi data to PubChem, including siRNA sequence information and biological activity data. Researchers who need assistance with submitting siRNA sequences or RNAi experimental data to PubChem may contact the [National Library of Medicine](#).

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.