NCATS

Improving Health Through Smarter Science

Trans-NIH RNAi Facility

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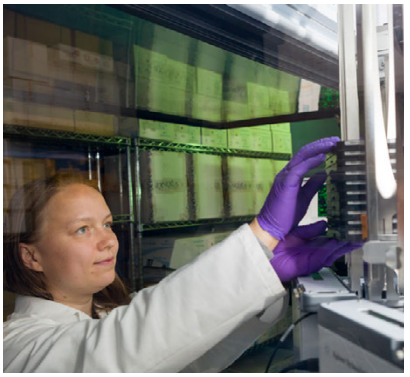
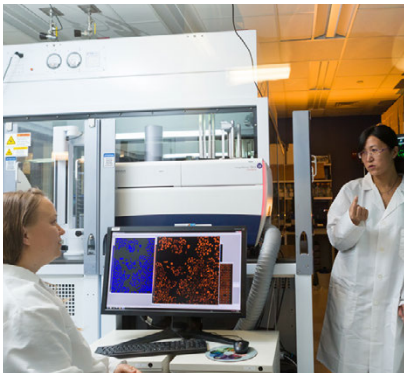
NCATS designed its Trans-NIH RNAi Facility (TNRF) to help NIH investigators access the latest functional genomics technology to advance drug discovery and scientific knowledge about health and disease.

TNRF goals are collaborations that enable:

- NIH investigators to perform genome-wide and targeted ribonucleic acid interference (RNAi) and clustered regularly interspaced short palindromic repeats (CRISPR) screening projects (assay development, screening and validation) to:
  - Understand fundamental biological mechanisms
  - Accelerate target discovery for therapeutic development
- Development and demonstration of methods that advance the science of functional genomics screening and informatics
- Education and outreach to increase awareness of TNRF tools and methods
- New and complementary technologies for exploring gene function





About the Technology

Small interfering RNA (siRNA) and short hairpin RNA (shRNA) molecules are pieces of RNA that block the activity of genes through RNAi. This process has emerged as a powerful tool used in thousands of laboratories worldwide to understand gene function. Because each RNA molecule can block a different gene, RNAi can tell scientists about the role of any gene in maintaining health or causing disease.

In tests called genome-wide and targeted RNAi screens, scientists use robots to introduce siRNAs and shRNAs into human cells to block the activity of genes. Scientists can use these techniques to understand how genes affect drugs' effectiveness and how they affect disease processes.

Another technology available through TNRF is CRISPR/Cas9, which stands for clustered regularly interspaced short palindromic repeats/CRISPR-associated protein 9. This technique enables researchers to make precise edits to a genome, removing, adding, activating or repressing genes based on desired outcomes. This could involve correcting a problem caused by a faulty gene by replacing it with a properly functioning version.

U.S. Department of Health and Human Services
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NIH…Turning Discovery Into Health

National Center for Advancing Translational Sciences

About the Facility

TNRF includes a robotic platform with integrated, automated devices for conducting all aspects of screening assays, 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, as well as targeted CRISPR and small molecule libraries. For data analysis, the facility offers powerful computational tools.

Emerging tools for interrogating gene function (e.g., CRISPR) represent new and complementary screening approaches to RNAi. The TNRF team offers diverse scientific and technical expertise and is heavily invested in exploring these emerging technologies, in terms of both their utility and their pitfalls, similarly to what has been achieved with RNAi, to develop and offer a menu of screening platforms. Complementary use of siRNA, CRISPR and shRNA will ensure that all three technologies remain critical to the success of future functional genomics projects. Learn more about the goals of TNRF.

NCATS TNRF staff work closely with collaborating NIH investigators throughout the planning and execution of each research project. TNRF experts offer advice and assistance on assay development, screening, data analysis, and follow-up, and they typically serve as co-authors for published results. In addition to collaborations, TNRF staff work to develop methods that advance the science of RNAi screening and informatics and pursue new technologies for exploring gene function.

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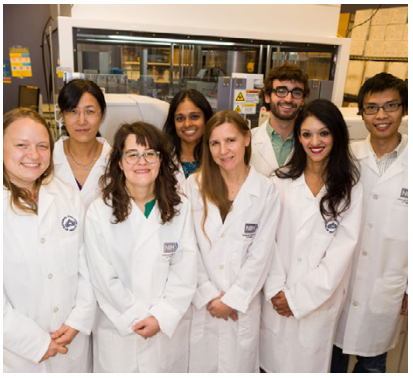

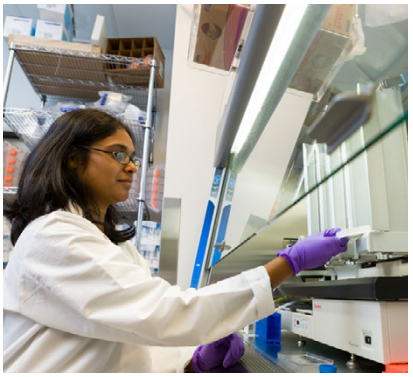

For More Information

About TNRF
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TNRF Projects
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Work with TNRF
ncats.nih.gov/rnai/work

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