National Center for Advancing Translational Sciences

Therapeutic Development Branch

The goal of the Therapeutic Development Branch is to help close the gap between basic research and preclinical testing of new small-molecule and biologic drugs, as well as cell-based and gene therapies. Work spans the entire preclinical development pipeline, including medicinal chemistry, toxicology, formulation, and other studies required to support an Investigational New Drug (IND) application to the U.S. Food and Drug Administration (FDA). The Therapeutic Development Branch is organized into separate but collaborative research teams to expedite this process.

Discovery Biology Team

The Discovery Biology team conducts studies that:

- Provide collaborative access to technology and expertise in the scientific disciplines of early discovery biology.
- Configure drug repurposing screens to accelerate drug development for rare and neglected diseases with a focus on lysosomal storage and infectious diseases.
- Use patient-derived induced pluripotent stem cells to develop new cell-based disease models.
- Advance projects from early preclinical drug development to IND applications and clinical trials.

Contact

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Visit https://ncats.nih.gov/trnd to learn more.

Medicinal Chemistry Team

The Medicinal Chemistry team delivers preclinical development candidates for evaluation in good laboratory practice safety studies prior to clinical trials. The team works in a multidisciplinary team environment to:

- Support preclinical development by conducting lead generation and lead optimization project collaborations both internal and external to NCATS and NIH.
- Develop novel medicinal technologies for accelerating the drug discovery process.

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Visit https://ncats.nih.gov/trnd to learn more.

Formulation Team

The goal of the Formulation team is to develop various dosage forms — such as capsules and injectables — for new therapeutics to be used in clinical studies. Developed formulations are manufactured under good manufacturing practice by contract manufacturing organizations (CMOs), and the specifications and drug product (DP) are used for filing IND applications to the FDA and to conduct clinical trials. Specifically, the team:

- Develops the most appropriate formulation for human administration.
- Develops, transfers and validates stability-indicating assay methods for the release of DP and subsequent stability testing.
- Fosters the DP manufacturing activity of the CMO to meet FDA specifications by maintaining the quality of the dosage form.
- Supports IND filings by providing collaborators with the documents and clinical study materials needed to initiate a Phase 1 clinical study.

Visit https://ncats.nih.gov/trnd to learn more.

Contact

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Safety Evaluation and Toxicology Team

The Safety Evaluation and Toxicology team conducts studies to:

- Improve the in vitro and in vivo safety evaluation of various therapeutics (e.g., small molecules, biologics, gene therapies).
- Understand the mechanisms of toxicities using innovative technologies to facilitate drug discovery.
- Make the drug development process more time and cost effective.

Visit https://ncats.nih.gov/trnd to learn more.

Contact

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