NCATS Improving Health Through Smarter Science

NCATS Division of Pre-Clinical Innovation

Developing, demonstrating, and disseminating new paradigms in early drug discovery

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- The Problem: There has been a tremendous jump in our understanding of human biology and disease, yet turning those basic discoveries to actual treatments remains costly, complex, and time-consuming.
  - Function of many genes and their products still remain unknown.
  - Current disease models have poor predictivity in humans.
  - Potential toxic/side effects of candidate therapies are hard to assess.

- How DPI is improving the process:
  - Predictive toxicology
  - Predictive efficacy
  - Derisking undruggable targets/untreatable diseases to help partners cross the "Valley of Death"
  - Novel public-private partnerships, increased patient engagement

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How can you get involved?

- **The Challenge**
  - Expand the number of molecular targets for therapeutic development.
  - Improve predictivity of toxicity and efficacy testing methods.
  - Improve the efficiency of preclinical development through patient engagement.

- **The Opportunity**
  - Please visit our posters to discuss opportunities for patient engagement.
    - RNAi: unlocking disease mechanisms, one gene at a time.
    - Assay Development and Screening Technologies: preclinical drug discovery in rare diseases.
    - Chemistry Technologies: discovery of novel drug combinations.
    - Stem Cell Translation Laboratory: a "collaboratory" to bring stem cell therapies to patients.

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