Catalyzing Innovation: NIH National Center for Advancing Translational Sciences

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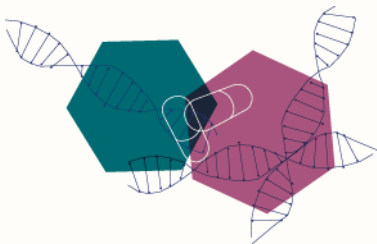
GUIRR Meeting, October 9, 2012



Creation of the National Center for Advancing Translational Sciences (NCATS)

- Established on December 23, 2011
- Part of Consolidated Appropriations Act 2012 (PL 112-74)

NCATS: Mission

To catalyze the generation of innovative methods and technologies that will enhance the development, testing, and implementation of diagnostics and therapeutics across a wide range of human diseases and conditions.

NCATS: Programs & Initiatives

Clinical and Translational Science Activities

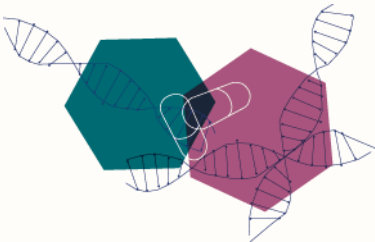
- Clinical and Translational Science Awards

Rare Diseases Research and Therapeutics

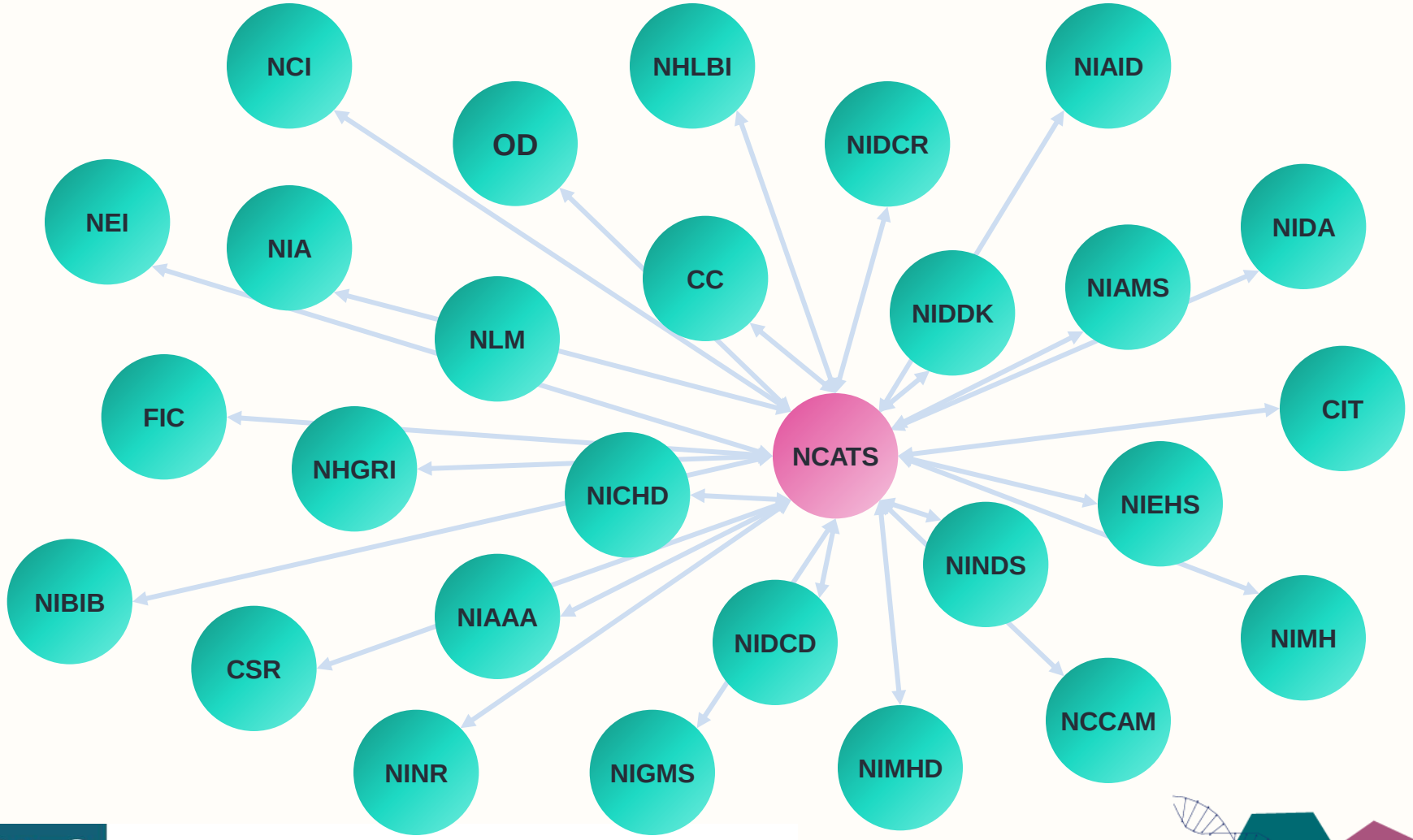
- Therapeutics for Rare and Neglected Diseases
- Office of Rare Diseases Research
- Bridging Interventional Development Gaps

Re-engineering Translational Sciences

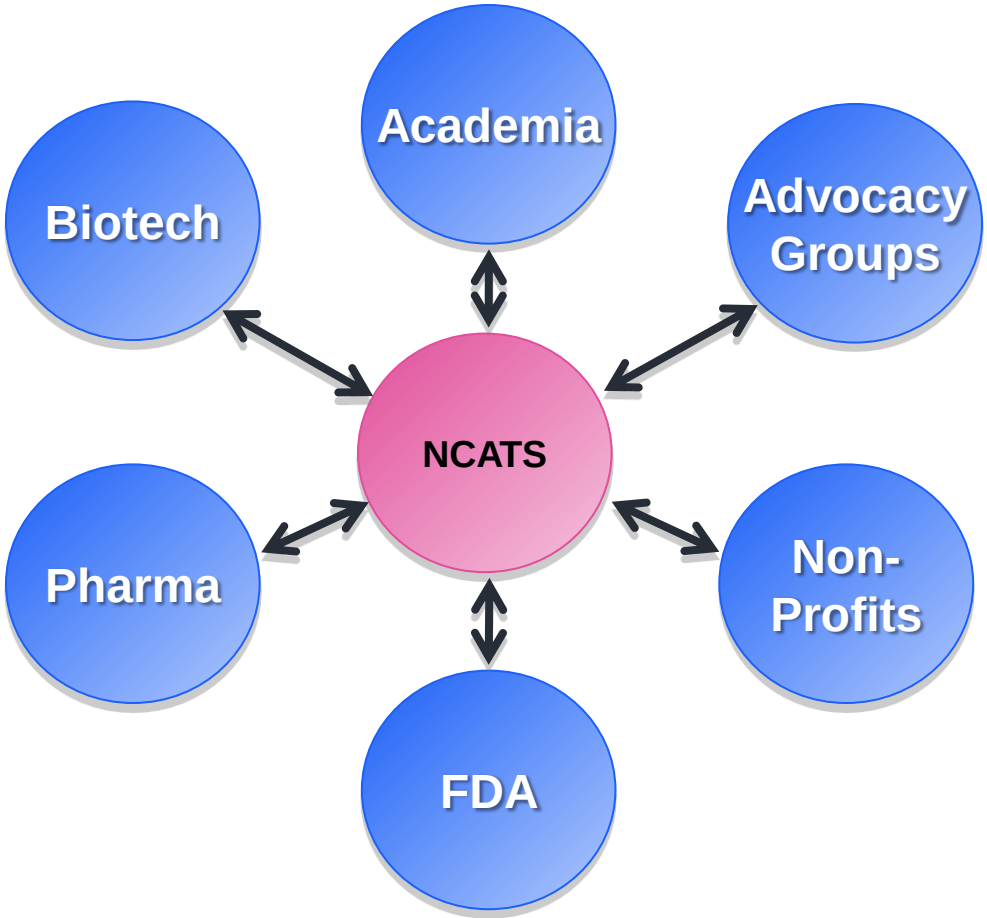
- NIH Chemical Genomics Center
- Toxicology in the 21st Century



Catalyzing Collaborations Within NIH

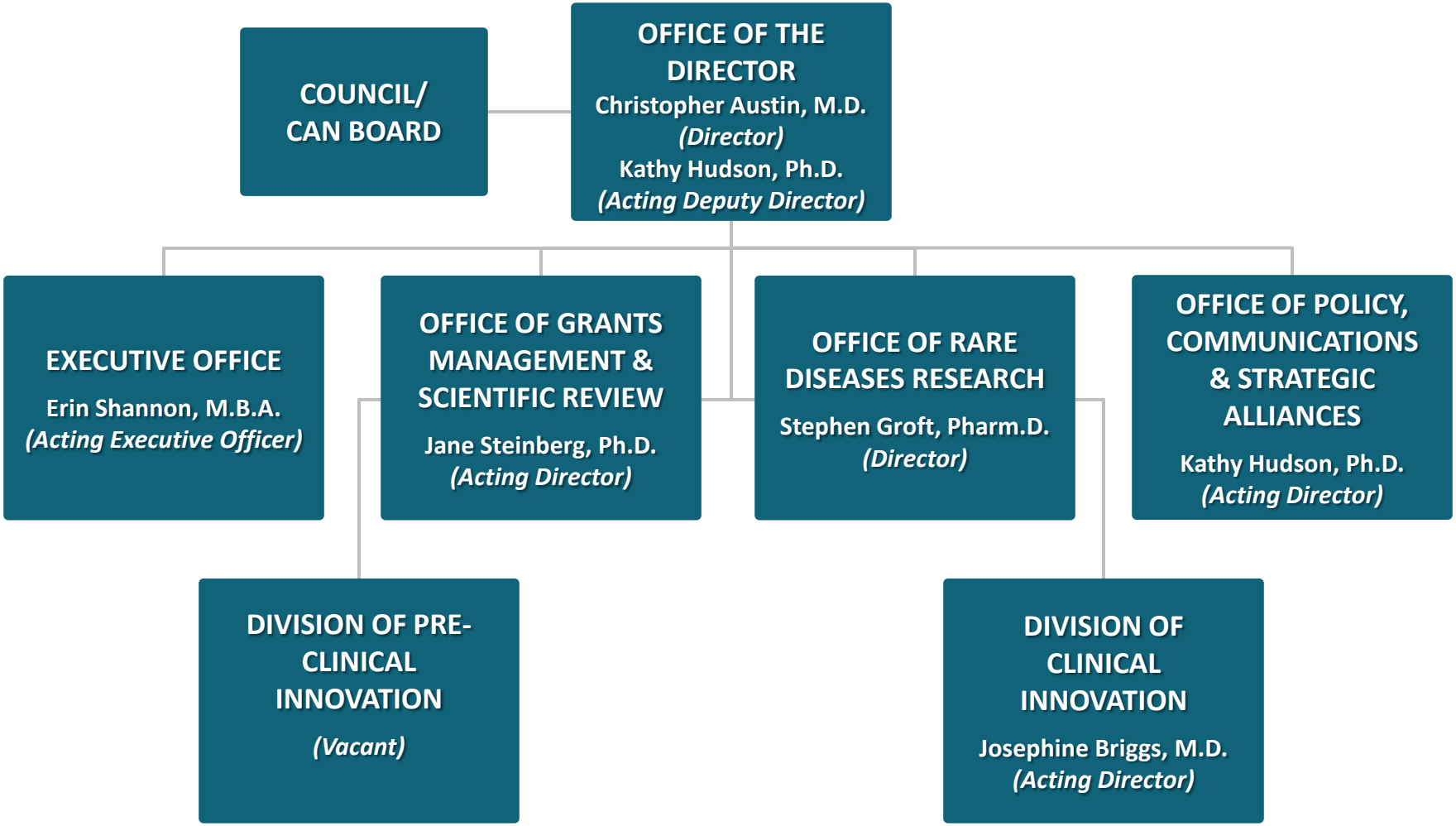


Catalyzing Collaborations Outside NIH

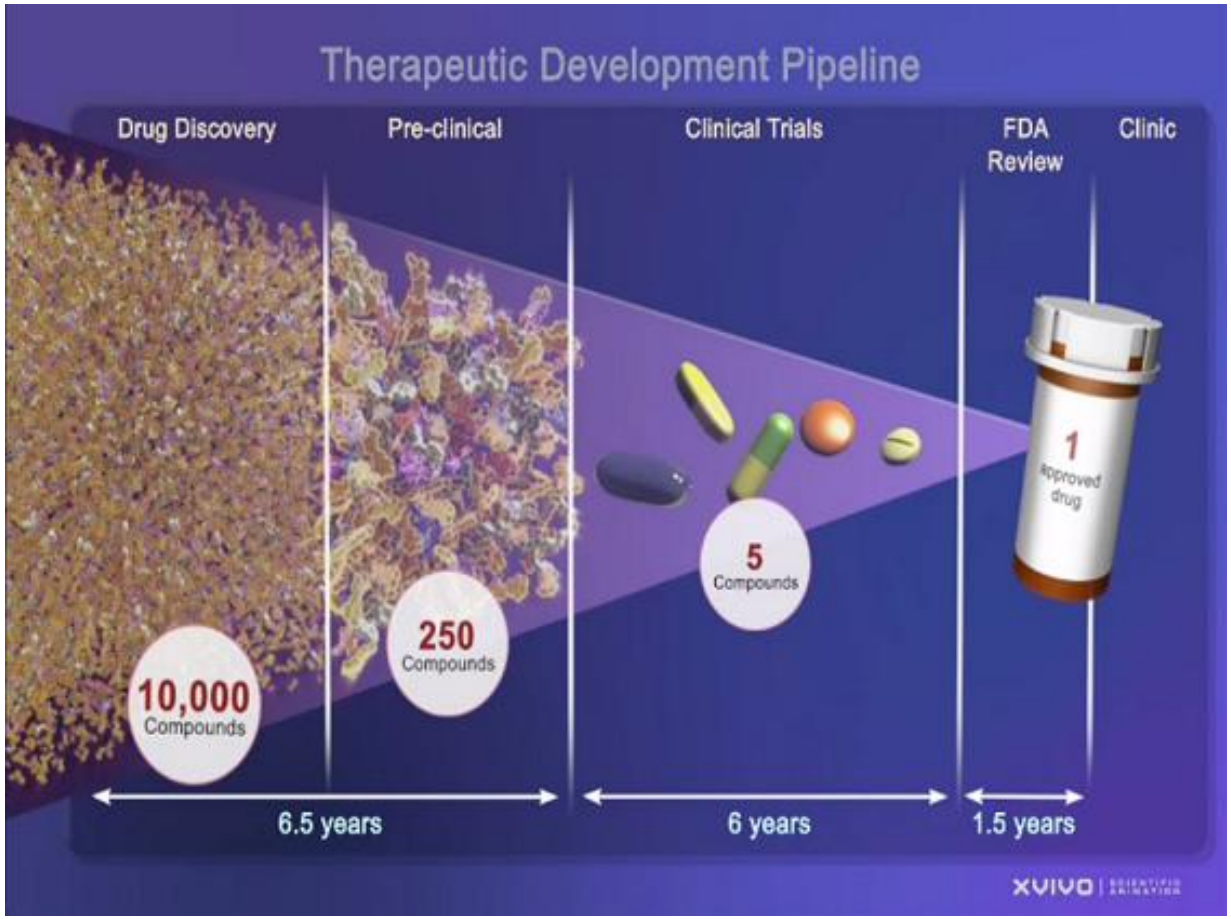


- Complements — does not compete with — the work of others
- Revolutionizes the process of translation by promoting innovative research
- Galvanizes and supports new partnerships
- Supports and augments regulatory science and its application
- Expands the precompetitive space

NCATS Organization

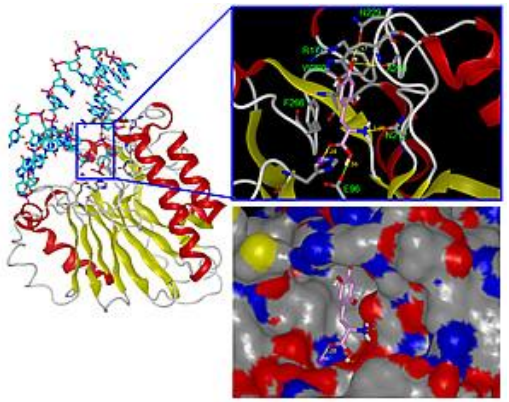


Development of New Therapeutics Is Slow, Expensive and Failure-Prone



Division of Pre-Clinical Innovation (DPI)

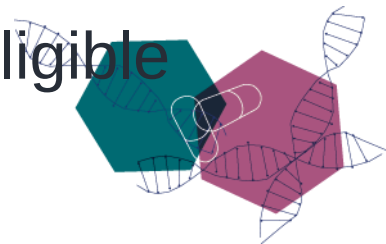
- Therapeutics for Rare and Neglected Diseases (TRND)
- Toxicology in the 21st Century (Tox21)
- Bridging Interventional Development Gaps (BrIDGs)
- Molecular Libraries Probe Production Center
- RNA interference (RNAi)



DPI currently has 300+ collaborations with investigators across the U.S. and around the world.

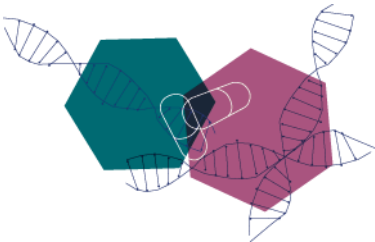
Bridging Interventional Development Gaps (BrIDGs) Program

- Model: Contract access collaboration between DPI and extramural labs (Formerly NIH-RAID Program)
- Projects
  - Enter with clinical candidate identified
  - Any disease eligible
  - Gap analysis followed by data generation using DPI contracts to generate data necessary for IND filing
  - Exit at or before IND
  - Milestone driven
  - Therapeutic modalities: any (small molecules, peptides, oligonucleotides, gene therapy, antibodies, recombinant proteins)
- Eligible Applicants
  - Academic (US and Ex-US), Non-Profit, SBIR eligible businesses



BrIDGs Highlights

- 180 applications submitted since 2005
  - 34 approved
- 19 completed projects (two in FY12)
  - 12/12 submitted INDs approved
  - 5 projects in Phase 1, three in Phase II
  - 5 agents licensed during or after BrIDGs involvement

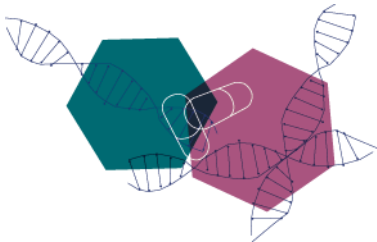


Therapeutics for Rare and Neglected Diseases (TRND) Program

- Model: Comprehensive drug development collaboration between DPI and extramural labs with disease-area / target expertise
- Projects
  - May enter at various stages of preclinical development
  - Disease must meet FDA orphan or WHO neglected tropical disease criteria
  - Taken to stage needed to attract external organization to adopt to complete clinical development/registration, max 2a
  - Milestone driven
  - Therapeutic modalities: small molecules, proteins
  - Serve to develop new generally applicable platform technologies and paradigms
- Eligible Applicants
  - Academic, Non-Profit, Government Lab, Biotech / Pharma
  - Ex-U.S. applicants accepted

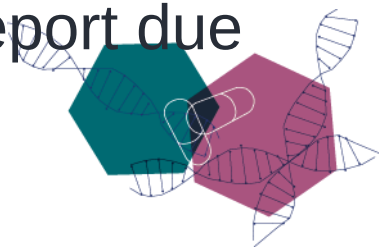
TRND Highlights

- 14 projects through pilot phase & 2 public solicitations since 2009
  - Mix of small molecules and biologics
  - Two innovative platform technologies
- 3 investigational drugs taken into humans
  - CLL: IND filed with US FDA 7/12/11, approved 8/5/11
    - Phase I trial commenced 9/11
  - SCD: IND filed 10/14/11, approved 11/10/11
    - Phase I trial commenced 12/11
  - HIBM: Complete response filed 7/27/12, approved 8/24/12
    - Phase 1 trial in patients commenced 9/13/12
- Initiated first natural history study
  - HIBM: NIH Clinical Center, 1st patient enrolled September 2011
- Every project is a unique Public-Private partnership
  - Many include foundation and patient advocacy input



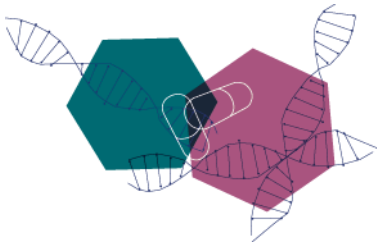
Overall CTSA Program History

- Approximately 2/3 of funds derived from GCRC program with balance from Common Fund;
- Twelve sites funded in 2006, with 5 to 10 new sites each year through 2011
- Maximum site budgets determined by legacy funding from GCRC's and related training programs
- Site budgets range from $4.0 to $23 M
- NCATS 2012 CTSA budget is $461 M
- Approximately 65% of NIH extramural funding goes to institutions that have a CTSA
- IOM study mandated by Congress, evaluation report due June 2013



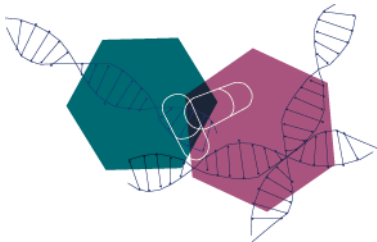
The CTSA 2.0 Vision

- An consortium-based infrastructure that facilitates a more effective implementation of clinical studies, particularly multisite studies
- An enriched pipeline of novel therapeutics and diagnostics, moving from basic laboratories (particularly NIH-supported laboratories) into clinical testing
- Innovation in translational research methods, including novel strategies to promote community and patient engagement in the research process
- National leadership engaged in protecting human subjects to improve oversight and minimize burden and delays in clinical trials
- National leadership in developing informatics standards to promote intra-operability of data resources and research tools



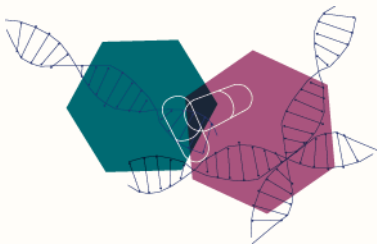
Characteristics of New NCATS Initiatives

- Address significant bottlenecks in the process of translation
- Highly collaborative across NIH, other government agencies, and with the private sector
- Quick to respond to needs of biomedical researchers



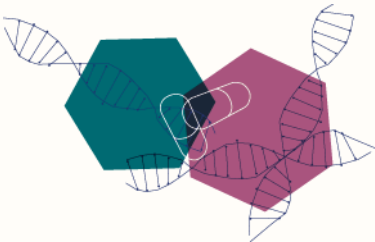
Therapeutics Discovery Pilot: Drug Rescue and Repurposing

- Collaborative pilot program matching researchers with compounds in an effort to find new uses for patients
- Creation of template agreements to streamline negotiations between researchers and pharmaceutical companies
- 58 compounds from eight companies:
  - Abbott
  - AstraZeneca
  - Bristol-Myers Squibb Company
  - Eli Lilly and Company
  - GlaxoSmithKline
  - Janssen Pharmaceutical Research & Development, L.L.C.
  - Pfizer Inc.
  - Sanofi



Therapeutics Discovery Pilot: Timeline

- **June 2012**: Funding announcement issued
- **August 2012**: X02 pre-applications received
- **December 2012**: Full applications due
- **May 2013**: Applications to council
- **May/June 2013**: Awards to be issued
- Program will be evaluated for success:
  - Does the use of template agreements speed negotiation time?
  - Does the pilot advance disease understanding?
  - Does the pilot result in promising new therapeutics?



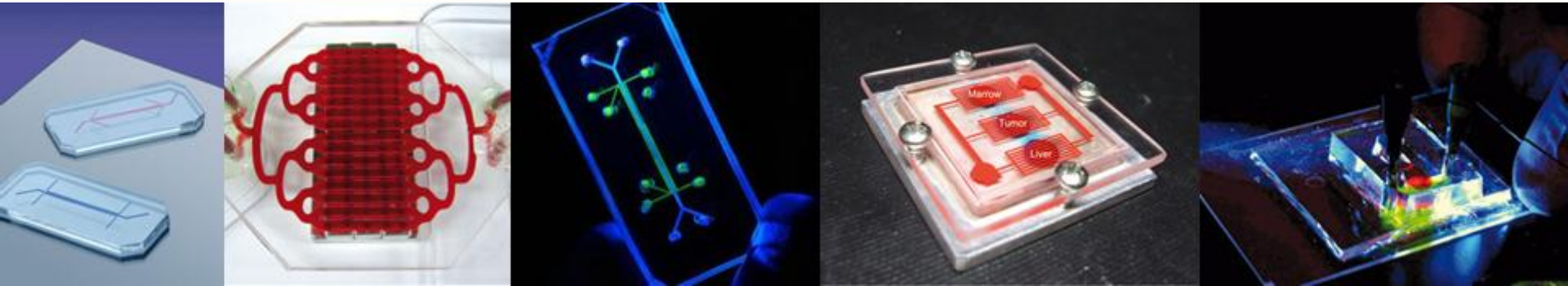
Tissue Chip for Drug Screening: Microsystems Initiative

- Aims to develop tissue chips that mimic human physiology to screen for safe, effective drugs using best ideas in engineering, biology, toxicology
- NIH Investment (NCATS + Common Fund) = $70M/5 years
- DARPA Investment = $75M/5 years
- FDA Investment = Regulatory and toxicology expertise
- NCATS and DARPA independently manage and fund separate, but highly coordinated programs

FY12 Appropriated and Reimbursable Funds = $13.9M

Tissue Chip for Drug Screening: Timeline

- **November 2011**: Funding announcements issued
  - *RFA RM-11-022*: Integrated Microphysiological Systems for Drug Efficacy and Toxicity Testing in Human Health and Disease (UH2/UH3)
  - *RFA RM-12-001*: Stem/Progenitor Cell-Derived Human Micro-organs and -tissues (U18)
- **July 2012**: 17 awards issued plus two additional awards funded by other NIH Institutes/Centers



Learn More About NCATS

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