The Webinar Will Begin Shortly

Questions/Comments: Share questions and comments in the chat window on the right side of your screen. Questions will be reviewed during the Q&A period.

Closed Captioning: http://bit.ly/NCATScc

Zoom Technical Support: +1.888.799.9666 ext. 2 or https://support.zoom.us/hc/en-us

Feedback: Let us know what you thought of this presentation https://forms.gle/mk2PJNLMJbM1DSya7

NCATS Improving Health Through Smarter Science

National Center for Advancing Translational Sciences

NCATS Small Business Funding: Early-Stage Support to Commercialize Your Translational Science Innovation

January 23, 2020

Office of Strategic Alliances
National Center for Advancing Translational Sciences,
National Institutes of Health

Georgia Bio

Join the conversation
@ncats_nih_gov | #NCATSsbir

National Center for Advancing Translational Sciences

Featured Speakers:

Lili Portilla, M.P.A.
Director of Strategic Alliances, Office of Strategic Alliances
National Center for Advancing Translational Sciences
National Institute of Health

Maria Thacker-Goethe, M.P.H.
CEO, Georgia Bio
Executive Director, Georgia BioEd Institute

Monique LaRocque, M.P.H.
Senior Vice President
Ogilvy Health | FKH

Thank You to Our Collaborator:

Webinar Objectives

- Expand awareness about the NCATS, its SBIR and STTR programs and other small business resources to help foster innovation and technology development
- Provide tips to help small businesses and research organizations in IDeA states successfully access and apply for NCATS resources
- Increase the number of high-quality SBIR and STTR applications, especially among geographically diverse states and women- and minority-owned businesses

Agenda

Introductions and Objectives

NCATS SBIR & STTR Programs

- Overview
- NCATS Research Focus
- The NCATS SBIR and STTR Program: Tips for Success
- Case Studies
- Other Small Business Resources

Moderated Q&A

- Please use the chat or Q&A function to submit questions at any time during the presentation

What Does the National Center for Advancing Translational Sciences (NCATS) Do?

NCATS

- 1 of 27 Institutes and Centers at the National Institutes of Health (NIH).
- Conducts and supports research on the science and operation of translation to allow more treatments to get to more patients more quickly.
- Focuses on what is common across diseases and the translational process.

Translational Sciences

- 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 Scientific Initiatives

- **Clinical Translational Science**
  - Clinical and Translational Science Awards
  - Rare Disease Clinical Research Network
  - New Therapeutic Uses program
- **Preclinical Translational Science**
  - NCATS Chemical Genomics Center
  - Therapeutics for Rare and Neglected Diseases program
  - Bridging Interventional Development Gaps program
- **Re-engineering Translational Sciences**
  - Toxicology in the 21st Century
  - Microphysiological Systems (Tissue Chip) program
  - Office of Rare Diseases Research

Translational Science and Research Areas of Interest

SBIR and STTR programs support NCATS' **mission to transform the translational science process** so that **new treatments and cures** for disease can be **delivered to patients more efficiently.**

Topics of Interest

1. Preclinical Drug Discovery & Development
2. Biomedical, Clinical & Health Research Informatics
3. Clinical, Dissemination & Implementation Research

2020 Deadlines

January 6

April 6

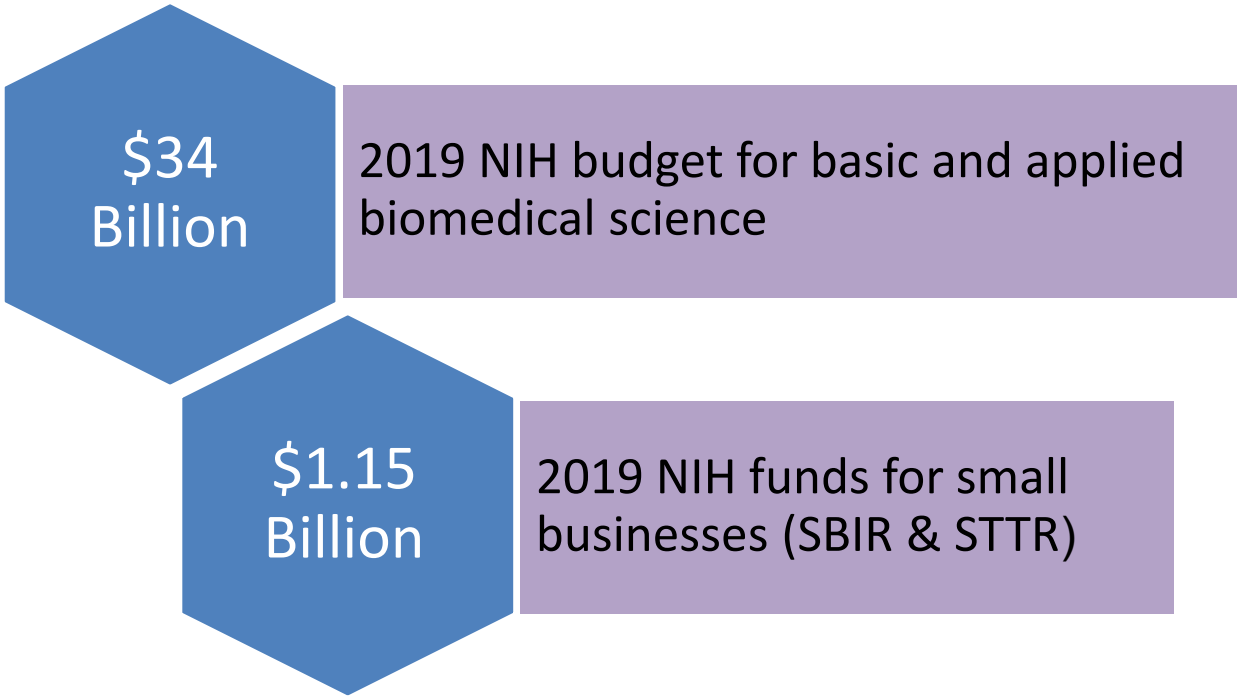
September 8

Funding Overview

The Small Business Innovation Research (SBIR) and Small Business Technology Transfer (STTR) programs are some of the largest sources of early-stage capital for innovative small companies in the United States. These programs allow U.S.-owned and operated small businesses to engage in federal research and development (R&D) that has a strong potential for commercialization.

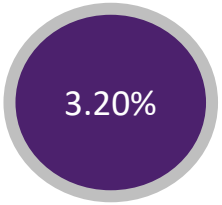
Omnibus Solicitation	• Investigator-initiated grant funding • Standard Deadlines: April 5, September 5, January 5
Grant Solicitations in Targeted Areas	• Grant to advance a particular technology/research area • Due dates may vary
Contract Solicitation	• Contract opportunity to advance areas of high research interest • Typically due in October or November

SBIR and STTR: One of the Largest Sources of Early-Stage Financing



Congressionally Mandated Programs

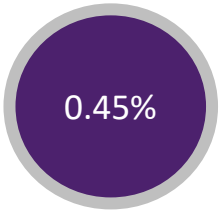
SET ASIDE



(FY19)

SMALL BUSINESS INNOVATION RESEARCH (SBIR) PROGRAM

Set-aside program for small business concerns to engage in federal R&D – with potential for commercialization



(FY19)

SMALL BUSINESS TECHNOLOGY TRANSFER (STTR) PROGRAM

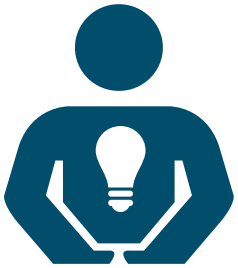
Set-aside program to facilitate cooperative R&D between small business concerns and U.S. research institutions – with potential for commercialization

The Benefits

NCATS Small Business Programs (SBIR/STTR)

Stable and predictable. Not a loan. Funds don't have to be repaid.

Non-dilutive. IP rights are retained by the small business.



Technical assistance to advance and commercialize technologies for public good.

Projects undergo NIH's rigorous scientific peer review process, which awardees leverage to attract other funding and collaborations.

NIH SBIR/STTR Is a Three-Phase Program

Who Is Eligible for SBIR Funding?

Criteria for applying to SBIR:

- ✓ U.S. businesses with **500 or fewer employees**
- ✓ **PI Primary employment with small business** at the time of the award and duration of the project
- ✓ **More than 50% U.S.-owned** by individuals and independently operated

OR

- ✓ More than 50% owned and controlled by other business concern(s) that is/are > 50% owned and controlled by one or more individuals

OR

- ✓ More than 50% owned by multiple venture capital operating companies, hedge funds, private equity firms or any combination of these

Who Is Eligible for STTR Funding?

Criteria for applying to STTR:

- ✓ **An established cooperative research and development effort delineated as**:
  - Minimum 40% by small business concern; minimum 30% by U.S. college or university, non-profit research organization or Federally-Funded R&D Center (FFRDC)

- ✓ **Formalized intellectual property agreement**
  - Should provide the necessary IP rights in order to carry out follow-on R&D and commercialization

- ✓ **Primary employment of the principle investigator with either the small business or research institution**

SBIR and STTR Critical Differences

	SBIR	STTR
Partnering Requirement	Permits partnering	Requires a non-profit research institution partner (e.g., university)
Work Requirement	Guidelines: May outsource 33% (Phase I) 50% (Phase II)	Minimum Work Requirements: 40% small business 30% research institution partner
Principal Investigator	Primary employment (>50%) must be with the small business	PI may be employed by either the research institution partner or small business

Award is always made to the small business

Investigator-Initiated Grants

Omnibus "Parent" SBIR/STTR Grant Solicitation

SBIR: PA-19-272 **STTR:** PA-19-270

We encourage applications for topics within the respective Center or Institute's mission

Read the "Program Descriptions and Research Topics" Section in the Solicitation for more details

Standard Deadlines:

April 5, September 5, January 5

Commercializing Understudied Proteins from the Illuminating the Druggable Genome Project (IDG)

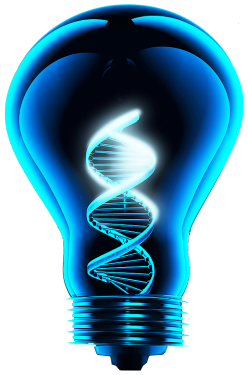
Purpose: to solicit applications from small business concerns to initiate early research ultimately leading to the commercialization of understudied proteins identified in the "Illuminating the Druggable Genome" project.

- SBIR: PA-19-034

  Next deadline: April 6, 2020

- STTR: PA-19-033

  Next deadline: April 6, 2020



IDG

ILLUMINATING the DRUGGABLE GENOME

Other Funding Opportunities

- **Administrative Supplements to Promote Diversity in Research & Development in Small Businesses**

  PA-18-837 and NOT-OD-19-016

  Expires on September 5, 2021

- **NIH HEAL Initiative℠**

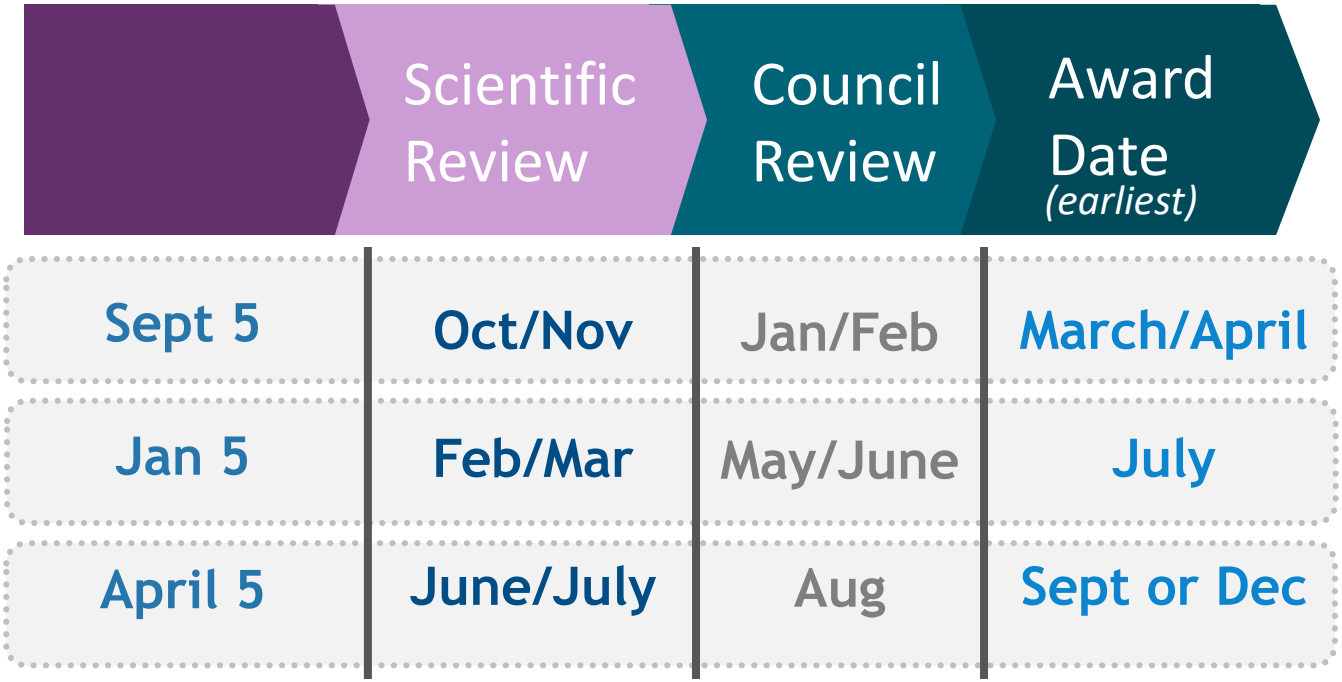
  America's Startups and Small Businesses Build Technologies to Stop the Opioid Crisis

  RFA-DA-19-019 (SBIR) and RFA-DA-19-020 (STTR)

  Next deadline: April 9, 2020

- **Commercialization Readiness Pilot (CRP) Program Technical Assistance**
  - SBIR/STTR: PAR-19-334 (SB1, R44 - Clinical Trial Not Allowed)
  - Next deadline: April 6, 2020

Deadlines and Application Process Timeline



SBIR & STTR

Developing a Successful Application

Scored Review Criteria

Significance	Does the product address an important **problem**, and have commercial potential? Is there a market pull for the proposed product?
Approach	Are **design and methods** well-developed and appropriate? Are problem areas addressed? Are potential pitfalls and alternative approaches provided?
Innovation	How novel is the **technology/product** and the **approaches** proposed to test its feasibility?
Investigator	Are the investigators, collaborators and consultants appropriately trained and **capable** of completing all project tasks?
Environment	Does the **scientific environment** contribute to the probability of success? **Facilities**? **Independence**?
Commercialization	Is the company's **business strategy** one that has a high potential for success?

Electronic Grant Application Submission

- SBIR/STTR applications must be submitted electronically.

Registrations are required; START EARLY

- DUNS Number (Company)
- System for Award Management (SAM)
- Grants.gov (Company)
- eRA Commons (Company and all PD/PIs)
- SBA Company Registry at SBIR.gov

http://era.nih.gov/applicants/index.cfm

Special Designations

- Encouraging participation in innovation and entrepreneurship by socially and economically disadvantaged small businesses (SDB) and women-owned small businesses (WOSB).
- What is a Socially and Economically Disadvantaged Small Business (SDB)?
  - The firm must be 51% or more owned and control by one or more disadvantaged person or persons.
  - The disadvantaged person or persons must be socially disadvantaged and economically disadvantaged.
  - The firm must be small, according to SBA's size standards.
  - Small businesses must self-certify by registering in the System for Award Management.
- What is a Women-Owned Small Business (WOSB)?
  - A firm must be at least 51% owned and controlled by one or more women, and primarily managed by one or more women (who must be U.S. citizens).
  - The firm must be "small" in its primary industry in accordance with SBA's size standards for that industry.
  - SBCs self-certify on the SF 424 (R&R) Form.

Top 8 Tips

1. **Review Funding Opportunity Announcements (FOAs)**, NCATS research topics of interest and eligibility carefully and make sure that your area of focus aligns with NCATS areas of interest

2. **Review sample applications**: https://www.niaid.nih.gov/grants-contracts/sample-applications

3. **Do your homework** on NIH funded applications – NIH RePORTER: http://projectreporter.nih.gov

4. **Talk to an NIH Program Officer** about your application: https://sbir.nih.gov/engage/ic-contacts

    - Contact an appropriate NIH Program Director in advance (**at least 1 month before due date!**), to discuss your specific aims and receive feedback

    - Before submitting an application, you can share abstracts to get feedback from program officers about your idea

5. **Register early** for SBIR or STTR electronic submission process

6. Use NIH ASSIST to **streamline the application process:** https://public.era.nih.gov/assist/public/login.do

7. Specify the Institute and study section for which you're applying

8. **SUBMIT EARLY** (days not hours and minutes)

Common Application Problems

- No Significance: Unimportant problem, unconvincing case for commercial potential or societal impact
- Inadequately defined test of feasibility
- Lack of innovation
- Diffuse, superficial or unfocused research plan
- Questionable reasoning in experimental approach
- Failure to consider potential pitfalls and alternatives
- Lack of experience with essential methodologies
- Unfamiliar with relevant published work
- Unrealistically large amount of work proposed

Important Facts to Remember

- Eligibility is determined at **time of award**
- PD/PI is **not** required to have a Ph.D./M.D.
- PD/PI is required to have expertise to oversee project scientifically and technically
- Applications **may be** submitted to **different agencies** for similar work to support different aims/objectives
- Awards may not be accepted from different agencies **for duplicative projects**

Communication After Review

Rejection is painful, but:

- Discuss Summary Statement with your NIH Program Director
- Be open to reviewer critiques; be constructive not defensive in your response
- Understand the review process and dynamics: https://www.csr.nih.gov/
- Consider reapplying, but spend A LOT of time reading and revising your aims page

SBIR & STTR

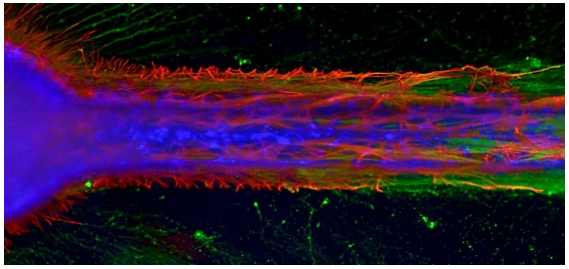
Case Studies

NCATS SBIR Success Story: AxoSim
Novel Nerve-on-a-chip technology

AxoSim

"With the dataset we generated [using the NCATS Phase 1 grant] from testing those four drugs, we were able to start discussing the platform with pharmaceutical companies. ...It showed the promise of this technology." – ***Lowery Curley Ph.D., Co-Founder and CEO***

AxoSim is a contract research organization dedicated to improving preclinical pharmaceutical development. Using advanced "nerve-on-a-chip," AxoSim facilitates the prediction of neurological safety and efficacy early in the drug development pipeline. By providing an alternative to animal testing, pharmaceutical companies will have access to high content data faster and earlier than currently possible.



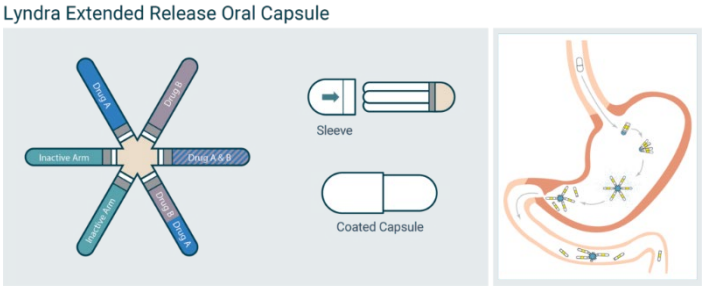
NCATS SBIR Success Story: Lyndra Therapeutics
Novel drug delivery technology

Lyndra™ THERAPEUTICS

Changing the pill, not the patient

"The NCATS SBIR grant helped us at a very critical stage. It was an important funding source, especially around our manufacturing ability." – ***Andrew Bellinger, MD, Former Lyndra CSO***

Medication non-adherence is a $289 billion problem in the U.S. alone. Instead of having to take medications daily or more frequently, Lyndra's oral, ultra-long-acting, sustained-release therapies, could allow patients to take medication weekly, or even monthly, thus improve medication adherence and health outcomes and help lower the cost of care. NCATS funded Lyndra's development of an Oral Ultra Long-Acting Ivermectin for Malaria Elimination



Small Businesses: Get Your Questions Answered

NCATS Small Business Programs (SBIR/STTR)

Questions about the SBIR and STTR funding application process or whether your project idea is a good fit? We're available to help!

- Check out our online FAQs
- Contact us to set up an appointment to discuss your project idea: NCATS-SBIRSTTR@mail.nih.gov
- View current funding opportunities and spread the word: ncats.nih.gov/smallbusiness

Additional Small Business Resources

I-Corps™ at NIH (PA-19-029)

Program for SBIR Phase I grantees to help:

- Define the value proposition (e.g., clinical utility) **early** before spending millions – saves time AND money
- Assess IP and regulatory risk before design and build
- Better understand core customers and the **specific** steps required for downstream commercialization
  - Teams are required to conduct 100 interviews
- Gather information essential to customer partnerships/ collaborations/ purchases before doing the science
- Identify financing vehicles before they are needed (helping to avoid the "Valley of Death")
- Next Deadline: January 21, 2020 for the May-June 2020 session

NIH Technical Assistance Programs

(open to all eligible NIH SBIR/STTR Awardees)

Niche Assessment Program
Foresight Science & Technology

(Phase I awardees)

- Identifies other uses of technology
- Determines competitive advantages
- Develops market entry strategy

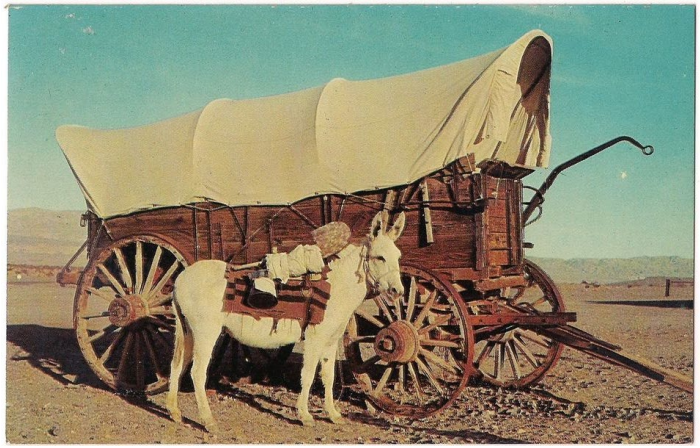
Commercialization Accelerator Program
Larta, Inc.

(Phase II awardees)

- "Menu" of technical assistance/training programs in:
  - Strategic/business planning
  - FDA requirements
  - Technology valuation
  - Manufacturing issues
  - Patent and licensing issues
- Helps build strategic alliances
- Facilitates investor partnerships
- Individualized mentoring/consulting

Crossing the "*Valley of Death*" with the NCATS Therapeutic Development Team

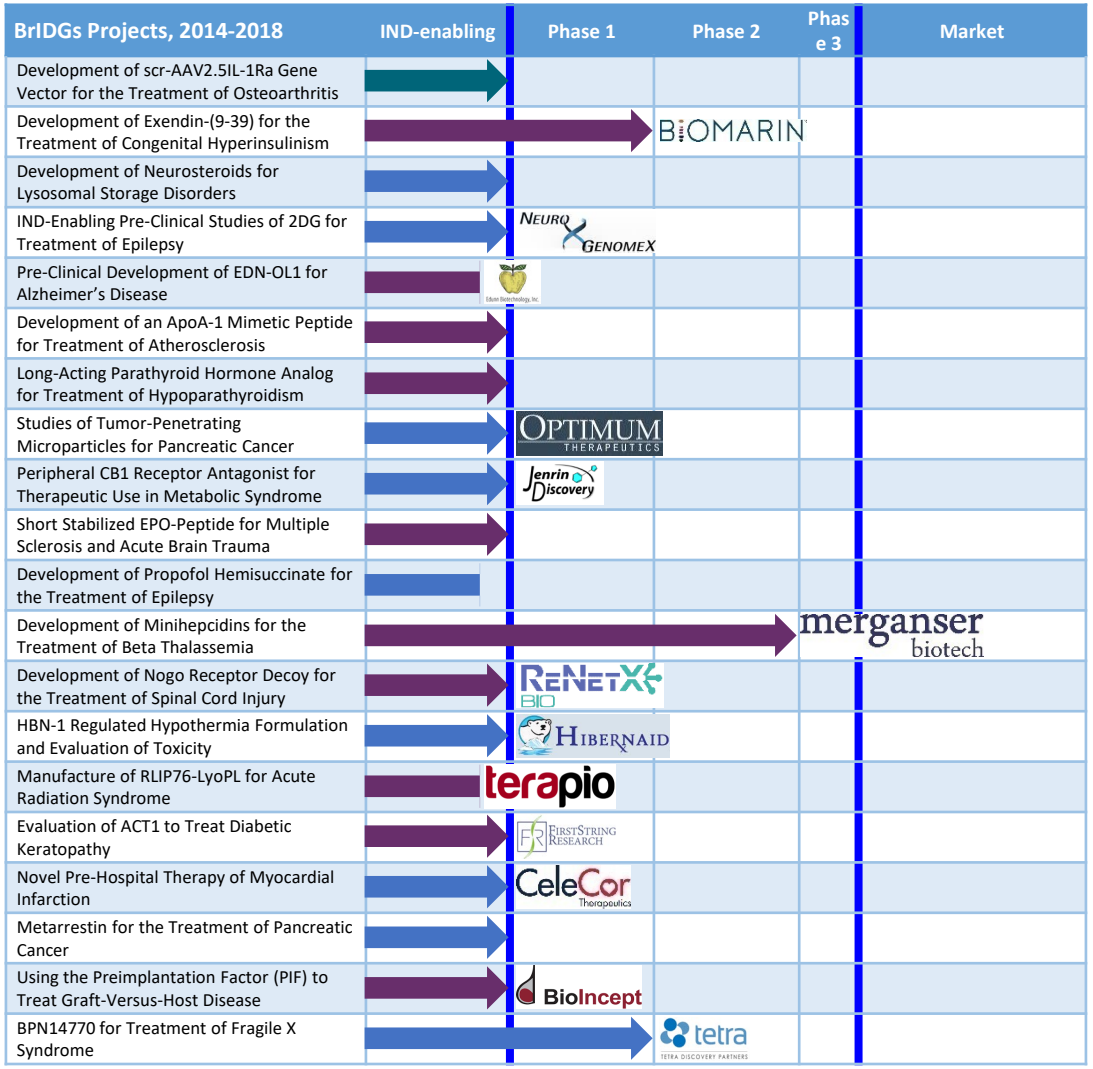
- Medicinal chemistry lead optimization
- Evaluation of functional activity, potency, pharmacokinetics (PK), pharmacodynamics (PD), and efficacy
- Biomarker development
- Definition and optimization of dose and schedule for *in vivo* activity
- Development and implementation of pharmacological assays
- Chemical and biologics process research and development
- Manufacturing of bulk substance (GMP and non-GMP)
- Development of suitable formulations
- Development of analytical methods
- Production and stability studies of dosage forms
- Range-finding initial toxicity
- Investigational New Drug (IND)-directed toxicology, with correlative pharmacology and histopathology
- Planning of clinical trials (Phase 1 and/or Phase 2)
- Regulatory and IND filing support
- Natural history and patient-finding studies



NCATS Additional Resources: Bridging Interventional Development Gaps (BrIDGs)

- Model: Collaboration between Division of Preclinical Innovation (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 resources and expertise to generate data necessary for IND filing
  - Exit at or before IND
  - Milestone driven
  - Therapeutic modalities: small molecules, peptides, oligonucleotides, gene therapy, antibodies, recombinant proteins
- Eligible Applicants
  - Academic (U.S. and Ex-U.S.), Non-Profit, SBIR-eligible businesses

BrIDGs Projects



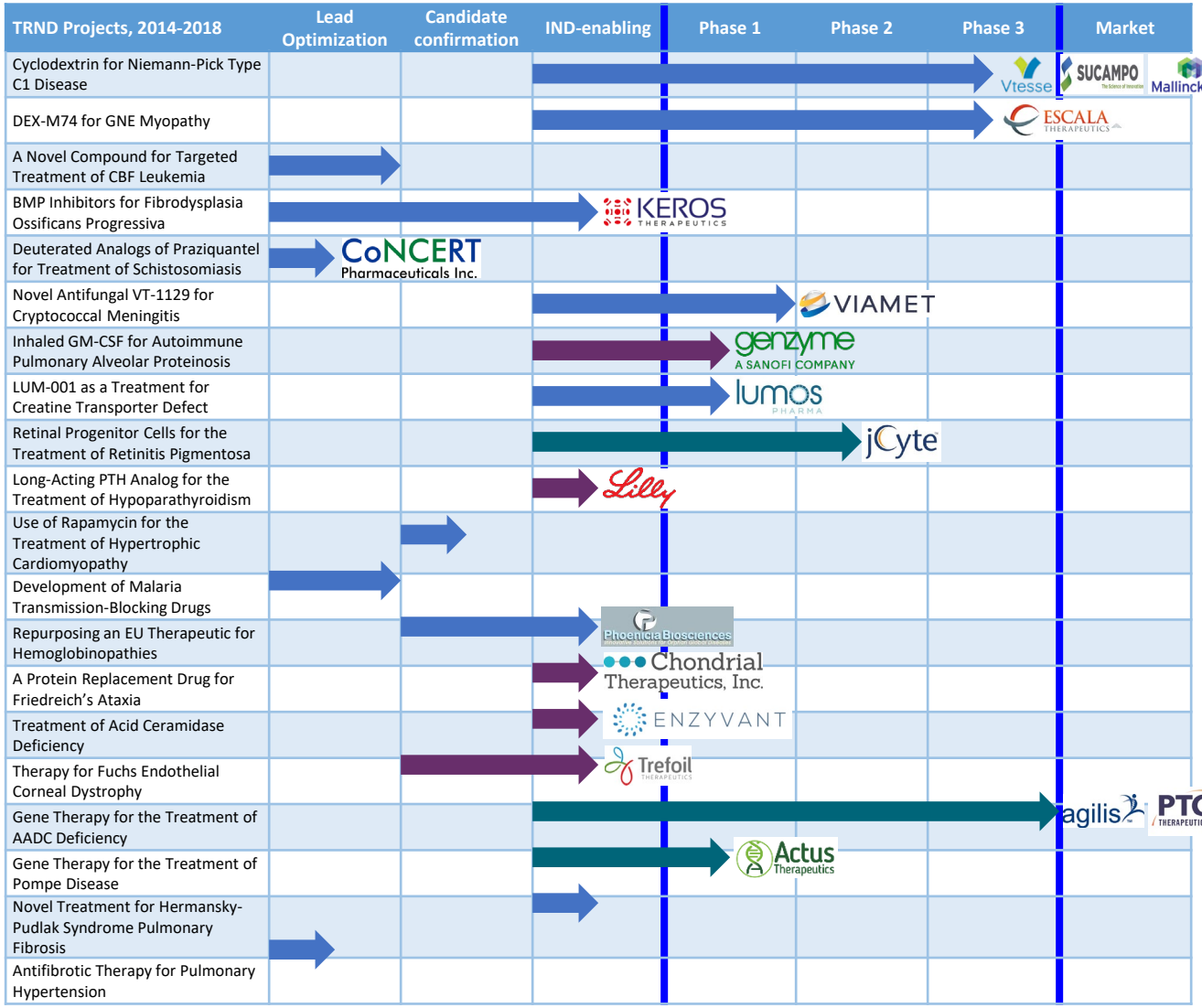
Small Molecule
Biologic
Gene and Cell Therapy

National Center for Advancing Translational Sciences

NCATS Additional Resources: 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 Phase 2a
  - Milestone driven
  - Therapeutic modalities: small molecules, proteins, peptides, oligonucleotides, gene therapy, antibodies, recombinant proteins
  - Aims to de-risk technology and develop new generally applicable platform technologies and paradigms
- Eligible Applicants
  - Academic, Nonprofit, Government Lab, Biotech/Pharma
  - Ex-U.S. applicants accepted

TRND Projects

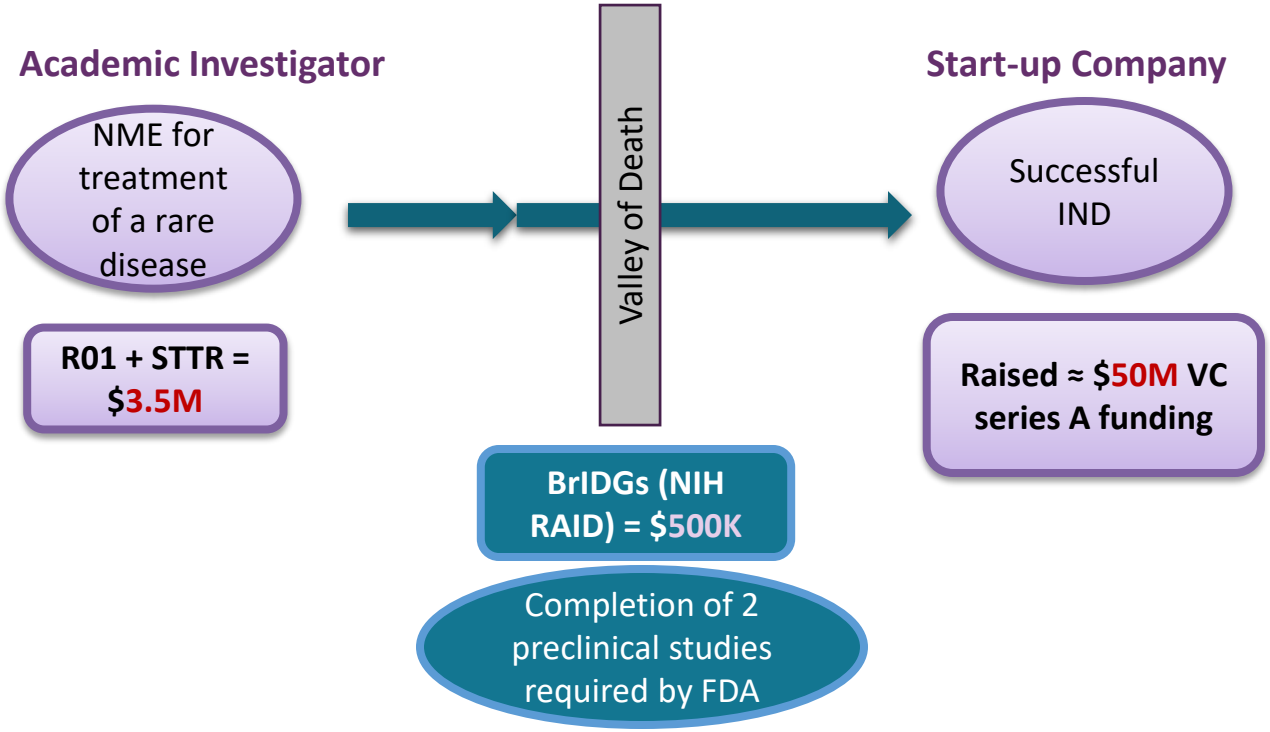


Small Molecule
Biologic
Gene and Cell Therapy

National Center for Advancing Translational Sciences

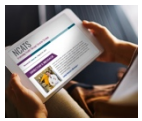
TRND/BrIDGs Project De-Risking Model

Minimum Time and Funding; Maximum Impact



Connect with NCATS
ncats.nih.gov/connect

- **Website:** ncats.nih.gov
- **Facebook:** facebook.com/ncats.nih.gov
- **LinkedIn:** www.linkedin.com/company/national-center-for-advancing-translational-science-ncats/
- **Twitter:** twitter.com/ncats_nih_gov
- **YouTube:** youtube.com/user/ncatsmedia



- **E-Newsletter:** ncats.nih.gov/enews
- **Listserv:** bit.ly/1sdOI5w

National Center for Advancing Translational Sciences

Questions?

ncats.nih.gov/smallbusiness

NCATS-SBIRSTTR@mail.nih.gov

Research Priorities: Preclinical Drug Discovery and Development

Innovative platforms for identification and prioritization of targets for therapeutic intervention with clear clinical impact, such as those that are: implicated for disease, have genetic variations that have been identified in functional regions of receptor targets and/or have high potential for biased signaling that would promote the beneficial effects of receptor signaling and reduce the unwanted effects
Tools and technologies to enable high-throughput screening of compound activity on currently "non-druggable" targets
Assays for high-throughput screening of rare diseases-related targets
Co-crystallization high-throughput screening techniques
Fluorescence probes to replace antibodies for determination of cellular protein translocation
Phenotypic assay development, including stem cell technology platforms for human "disease-in-a-dish" applications and the evaluation of toxicity
Interventions that target molecular pathways or mechanisms common to multiple diseases
Platforms for non-antibody biologics, cell-based therapies and gene therapy discovery
Small molecule and biologics analytical characterization
Accelerated bioengineering approaches to the development and clinical application of biomedical materials, devices, therapeutics and/or diagnostics

Research Priorities: Preclinical Drug Discovery and Development

Development of novel technologies for enzyme replacement therapies (e.g., new cell culture/expression system) to solve a major bottleneck in rare diseases research
Innovative methods to determine alternative uses for existing therapeutic interventions for high priority areas, such as rare diseases and pain
Tools and technologies that increase the predictivity or efficiency of medicinal chemistry, biologic or other intervention optimization
Technologies to deliver nucleic acid therapeutics to tissues other than the liver
Methodologies and technologies to increase efficiencies of manufacturing therapeutics
Development of novel high-throughput technologies that focus on making translational research more efficient
GMP production of exosome/extracellular vesicles
Generation of producer lines for large-scale production of exosomes/extracellular vesicles
Extracellular RNA-based biomarkers and therapeutics of human diseases
Approaches to targeting the human microbiome for therapeutic or diagnostic purposes

Research Priorities: Preclinical Drug Discovery and Development

Scale up, manufacturing and characterization of IPS cells
3D printing technologies
Technologies to substantially improve the efficiency and reduce the cost of clinical-grade gene therapy vector manufacturing
Development of in vitro human tissue models (organs, 3D printing)
Technologies to allow therapeutic proteins other than lysosomal enzymes to be secreted and taken up by other cells via cross-correction
Novel strategies to prevent deleterious immune responses to gene therapy, genome editing and/or enzyme replacement therapy
Establishing more robust phenotypic screens that may help prioritize candidate compounds for further testing
Innovative technology for non-small molecule delivery
High-throughput epigenetics screening/characterization tools and technologies
Microphysiological systems (MPS)/Tissue Chips, including MPS/Tissue Chips that incorporate known functional variants, e.g., ACMG 59 or CPIC A alleles, for study comparison using the same derived genetic background across a set of tissue chips with the functional variant

Research Priorities: Biomedical, Clinical & Health Research Informatics

Searchable access to information about research resources, facilities, methods, cells, genetic tests, molecules, biologic reagents, animals, assays and/or technologies with evidence about their use in research studies
Cloud-based tools and methods for meaningful sharing, re-use and integration of research data
Novel platforms, technologies and tools for: (1) enabling clinical and translational research, particularly those with mechanisms for inclusion of patient-reported data and (2) integration of patient data collected from multiple devices and multiple/diverse clinical studies
Development of personalized phenotypic profiling (as well as personalized intervention) based on patient-centered integration of data from multiple data sources, including social media
Development of predictive models for translational science
Digital applications and tools (including telemedicine platforms) that facilitate/enhance translational research and medicine in rural populations
Generic disease registry template platforms that can be reused for multiple diseases
Mobile device validation tools to ensure data from different brands or versions have compatible results
Tools to assess algorithms developed with artificial intelligence and/or machine learning
Tools that allow for persistent identifier and attribution for data contributors that give credit to the data producers while ensuring that shared data has not been altered
Patient mobile tool platforms that facilitate tool developers to build “apps” that integrate into their medical records
Tools and environments that enable an easy interrogation of publicly available data

Research Priorities: Clinical, Dissemination and Implementation Research

Tools and technologies that increase the efficiency of human subjects research, that facilitate the rapid diagnosis and/or clinical trial recruitment and subject tracking, institutional review board evaluation and/or regulatory processes
Increased efficiency of clinical research conduct, including but not limited to regulatory decision support, patient eligibility analysis and recruitment and retention tracking
Tools, technologies and other strategies to evaluate and improve the process of informed consent
Educational tools for clinical and translational science
Computational or web-based health research methods, including: • Platforms for generally applicable and scalable multi-disease registries and natural history studies • Clinical trial designs and analyses (e.g., for pragmatic clinical trials)
Approaches, tools, platforms and environments to integrate data in novel ways for development of new biomarkers that can be tested in translational research paradigms for which there are barriers or bottlenecks
Strategies to enhance the quality of and accelerate the conduct of dissemination and implementation research
Tools and technologies that increase the efficiency of human subjects research, that facilitate the rapid diagnosis and/or clinical trial recruitment and subject tracking, institutional review board evaluation and/or regulatory processes

Research Priorities: Clinical, Dissemination and Implementation Research

Increased efficiency of clinical research conduct, including but not limited to regulatory decision support, patient eligibility analysis and recruitment and retention tracking
Sustainable solutions for effective tools and environments in translational research
Development and validation of patient reported outcomes, clinician-reported outcomes and biomarkers for rare diseases that are not already supported by a disease-specific NIH Institute or Center
Tools, technologies and other strategies that address medication adherence in clinical settings
Tools, technologies and other strategies that address and improve community engagement
Tools and technologies that address the rapid diagnosis and/or clinical management of rare diseases
Patient empowerment tools/apps that allow users to compare their treatment and outcomes to normative populations existing treatment guidelines
Telemedicine or digital health applications that focus on research in rural populations
Tools and technologies that help characterize human disease states and assist in assessing the impact of interventions