The Webinar Will Begin Shortly

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10 YEARS of NCATS
COLLABORATE. INNOVATE. ACCELERATE.

A Decade of Innovation: How NCATS is Impacting Translational Science through Small Business Growth

December 8, 2021

ncats.nih.gov/smallbusiness

NIH National Center for Advancing Translational Sciences

Featured Speakers

Chris Gibson, Ph.D.

Co-Founder & CEO
Recursion Pharmaceuticals

Chang Hee Kim, Ph.D.

CEO
GoDx, Inc.

Lena Wu, Ph.D.

Co-Founder and Former CEO and President
Intabio, Inc.

Lili Portilla, M.P.A.

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

MODERATOR

Agenda

Welcome, Introductions and Overview

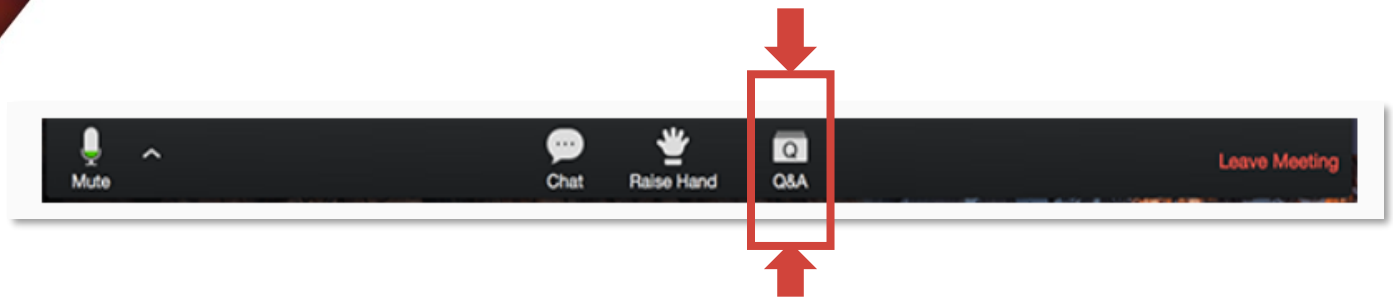
Meet the Panelists

- Chris Gibson, Ph.D.
- Chang Hee Kim, Ph.D.
- Lena Wu, Ph.D.

Discussion & Fire-side Chat

Moderated Q&A

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



NIH SBIR/STTR Is a Three-Phase Program

Phase I Feasibility Study

Budget Guide: $256,580K for SBIR and STTR *($325K Waiver)*
Project Period: 6 months (SBIR); 1 year (STTR)

Phase II Full Research/R&D

$1.7M for SBIR and STTR, over two years *($2M)*

Fast Track combines Phase I and Phase 2
Direct to Phase 2 – allows to skip Phase 1

Phase IIB Competing Renewal/R&D

Clinical R&D; Complex Instrumentation/to FDA
Funding Varies (~$1M per year) for up to 3 years

Phase III Commercialization

NIH, generally, not the "customer"
Consider partnering and exit strategy

NCATS
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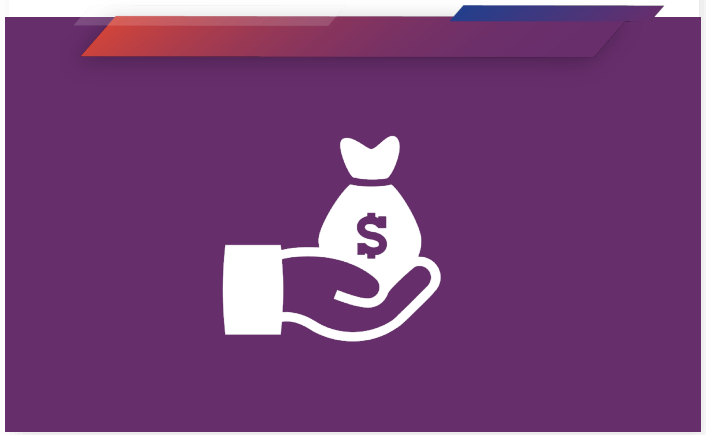
The Benefits

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.

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

2021-2022 DEADLINES:

January 5
April 5
September 6

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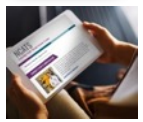

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Questions?

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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

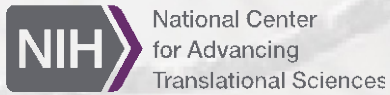
Research Priorities

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

THANK YOU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