The NIH Small Business Program and Data and Information Transparency Support *Turning Discoveries into Health*

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Deputy Director of the Office of Extramural Research (OER)
National Institutes of Health

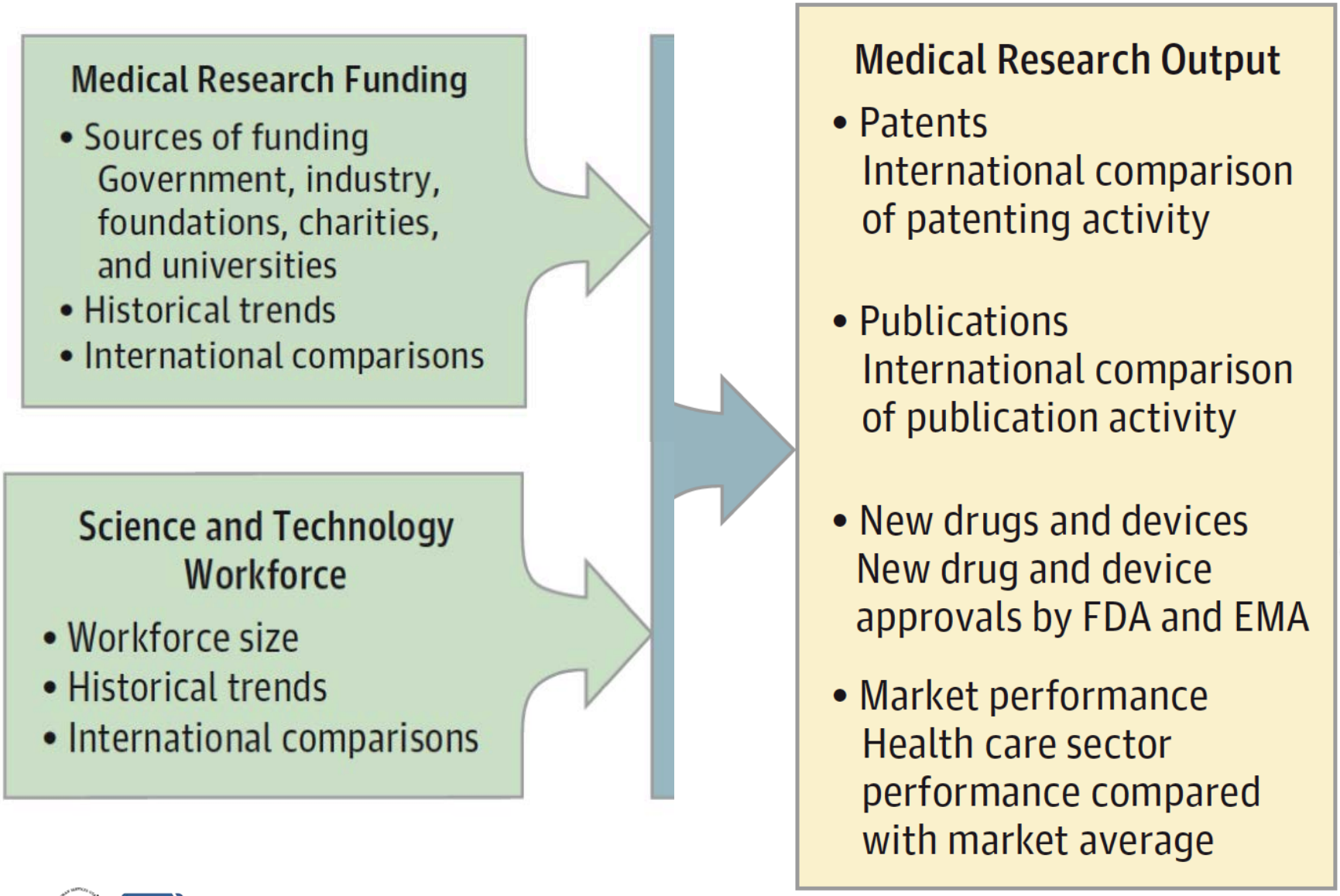
Federal Demonstration Partnership
May 10, 2018
Washington, DC
Disclosures: None

NIH - Turning Discoveries into Health




To seek fundamental knowledge about the nature and behavior of living systems and the ***application of that knowledge to enhance health, lengthen life, and reduce illness and disability****.*

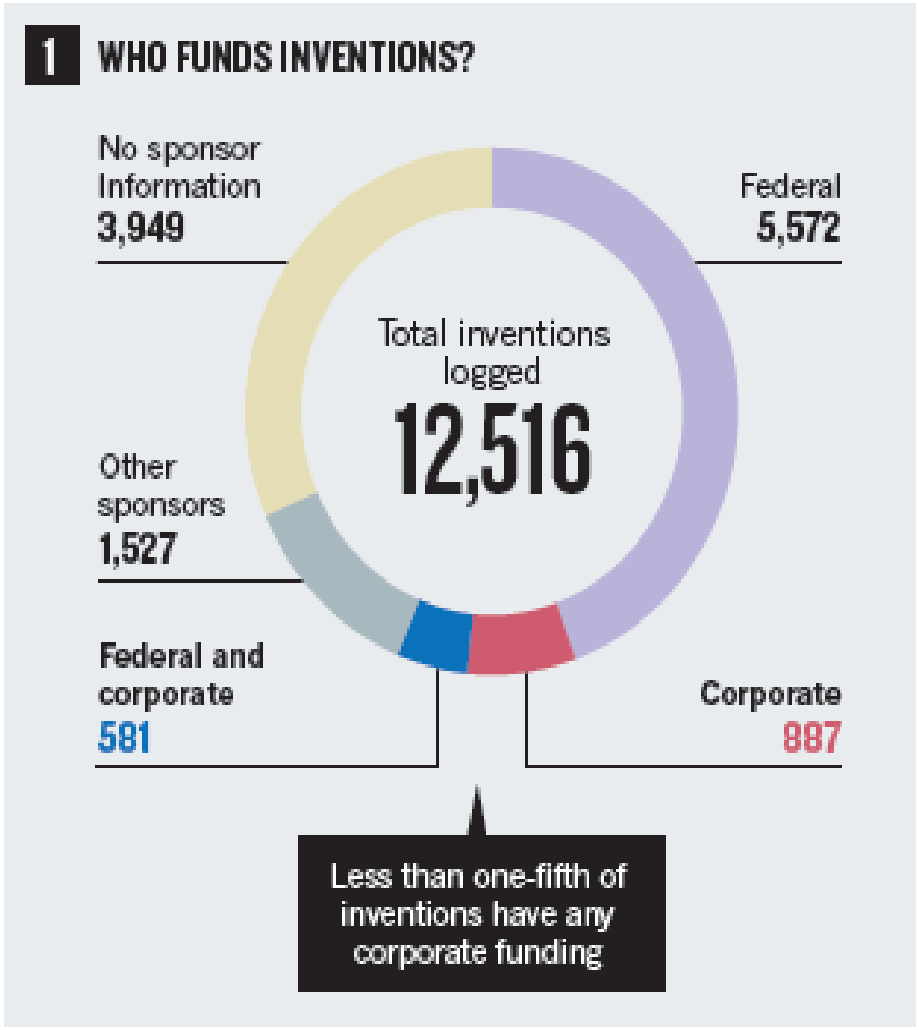
What We Care About – Ultimate Research Outcome

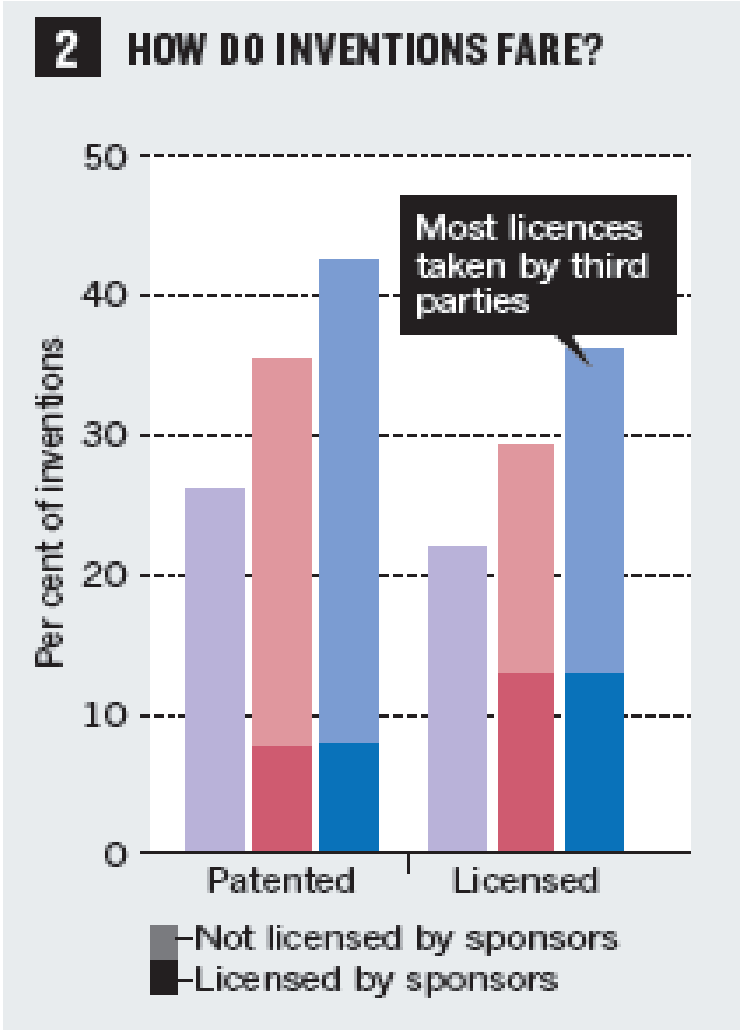


Moses H et al. JAMA 2015;313:174-189

Who pays for inventions?

Inventions logged by the University of California system 1990 - 2005



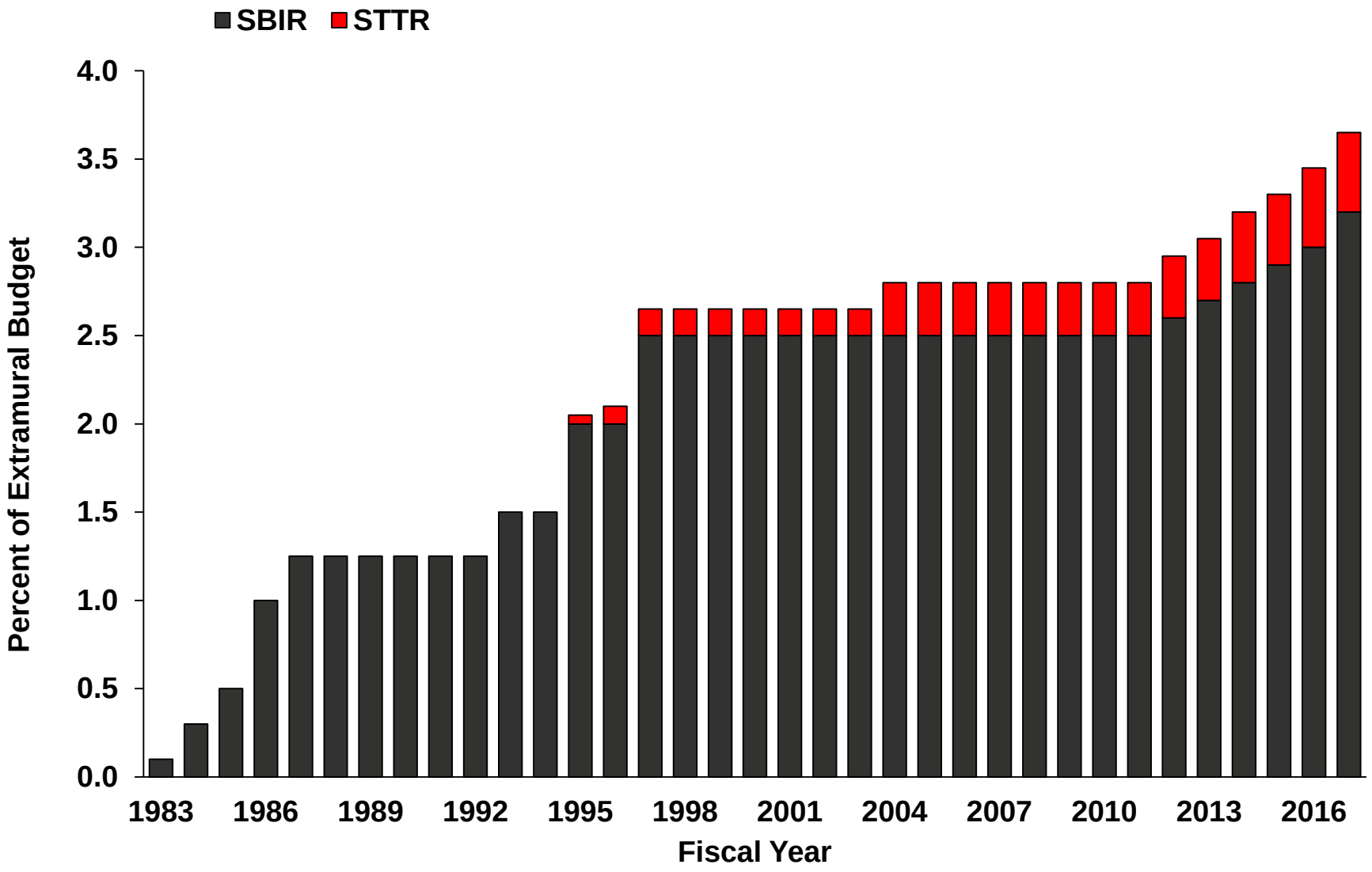


Wright, B Nature 2014;297

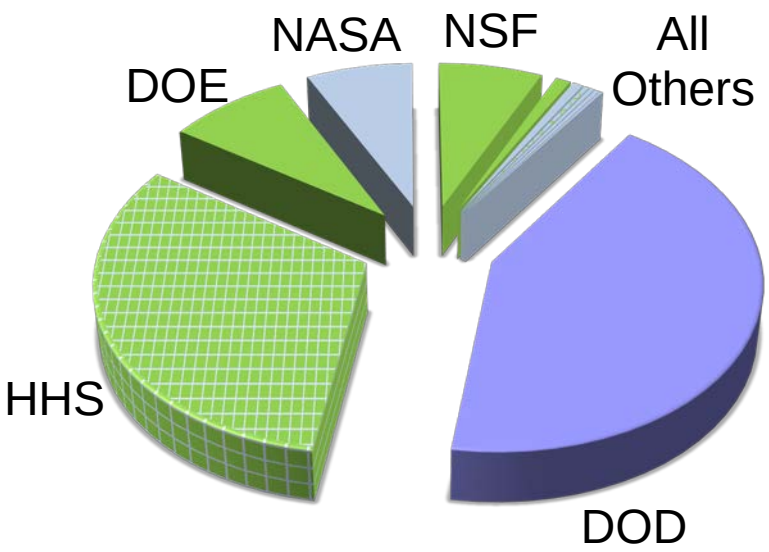
SBIR and STTR: America's Seed Fund

- Congressionally mandated set aside
- Stimulate technological innovation
- Meet federal R&D needs
- Foster participation of minorities and disadvantaged persons in tech innovation
- Increase private-sector commercialization of innovations resulting from federal R&D
- Foster collaborative research between small businesses and research institutions
- Foster technology transfer out of academia

Mandated SBIR/STTR Investments



SBIR/STTR Budgets by Agency FY16



$2.85 Billion

SBIR: $2.5 Billion
STTR: $361 Million

Grants
Contracts

SBIR·STTR
America's Seed Fund

* Provides grants and contracts

NIH National Institutes of Health
Office of Extramural Research

Agencies with SBIR and STTR Programs	Budget
Department of Defense (DOD)	$1.288B
Dept of Health and Human Services (HHS), inc National Institutes of Health (NIH)*	$891.0M
Department of Energy (DOE), including Advanced Research Projects Agency – Energy (ARPA-E)	$228.6M
National Science Foundation (NSF)	$187.7M
National Aeronautics and Space Administration (NASA)	$183.4M

Agencies with SBIR Programs	Budget
U.S. Department of Agriculture (USDA)	$28.8M
Department of Homeland Security (DHS)	$17.0M
Department of Commerce: National Oceanic and Atmospheric Administration (NOAA) and National Institute of Standards and Technology (NIST)*	$12.5M
Department of Transportation (DOT)	$11.6M
Department of Education (ED)	$7.5M
Environmental Protection Agency (EPA)	$4.9M

NIH Small Business Program (sbir.nih.gov)

Project funding
for small businesses

Supports feasibility, proof-of-concept, and research and development

Often awarded to:
start-up or **university spin-out companies**
(based on an academic lab's technology)

What are SBIR and STTR Programs?

The Small Business Innovation Research (SBIR) and Small Business Technology Transfer (STTR) programs, also known as America's Seed Fund, are one of the largest sources of early-stage capital for technology commercialization in the United States. These programs allow US-owned and operated small businesses to engage in federal research and development that has a strong potential for commercialization.

In Fiscal Year 2016, NIH's SBIR and STTR programs will invest over 870 million dollars into health and life science companies that are creating innovative technologies that align with NIH's mission to improve health and save lives. A key objective is to translate promising technologies to the private sector and enable life-saving innovations to reach consumer markets.

FY18 > $1 Billion

NIH SBIR/STTR
3-Phase Program

Discovery
Phase I

Feasibility

Development
Phase II

Full R/D

Competing Renewal Award
Phase IIB

$3M for up to 3 years

Commercialization
Phase III

Phase I → ***Phase II***

Fast-Track

Direct to Phase II

Congressional authority expired

Commercialization Readiness Pilot (CRP)

Congressional authority expired

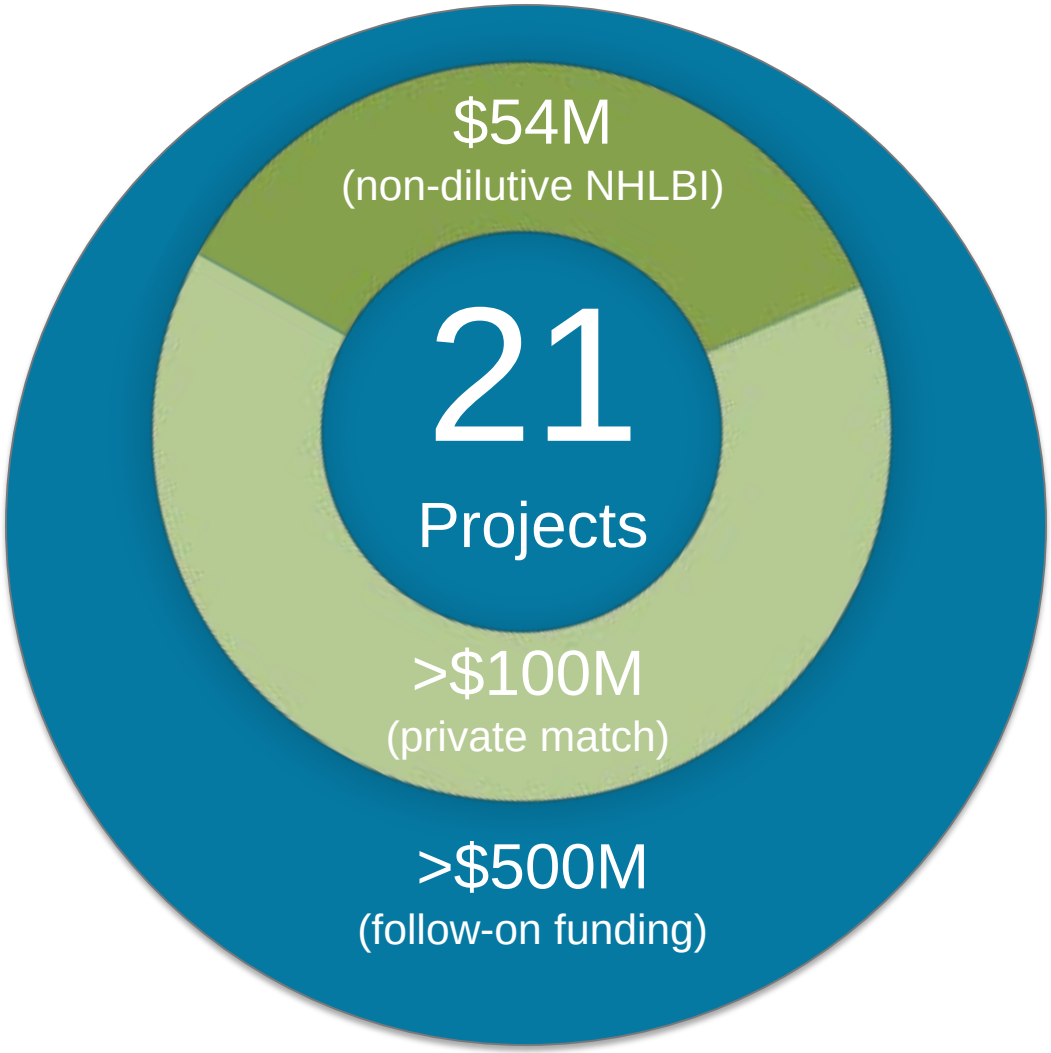
$3M for up to 3 years

Matthew Portnoy, PhD
NIH SBIR/STTR Program Coordinator
Phone: 301-435-2688
Email: mportnoy@mail.nih.gov

SBIR.NIH.GOV

National Institutes of Health
Office of Extramural Research

NHLBI SBIR Phase 2B: Strategic Partners



Outcomes include:

- Equity investments
- Co-development partnerships
- Licensing
- Regulatory filings
- Clinical trials

Enabling Commercialization: NIH Entrepreneurial Education Programs

Niche Assessment Program Foresight S&T

(Phase I awardees)

https://sbir.nih.gov/nap

Commercialization Accelerator Program Larta, Inc.

(Phase II awardees)

https://sbir.nih.gov/cap

I-Corps™ at NIH

Coulter College Commercializing Innovation (C3i)

- Focused on medical device development
- Provides mentoring and expert consulting services

More Enabling: Entrepreneurs in Residence

Ethel Rubin, PhD

Entrepreneur-in-Residence
ex-Medtronic, CSO 2 startups
VC consultant, 25+ products
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410-591-4736

Stephen Wolpe, PhD

Entrepreneur-in-Residence
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Rockefeller Univ/Genetics Institute/Wellstat
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stevewolpe@nih.gov
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Luis Gutierrez, MBA

Entrepreneur-in-Residence
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Ex-EVP/CCO, Aptiv Solutions
Ex-Group President, Covance
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Steve Flaim, PhD, FACC, FAHA

Investor-in-Residence
Director, Angel Capital Assoc'n
Director, Tech Coast Angels SD
S&TAdvisor, UCSD von Liebig Center
flaimsf@nhlbi.nih.gov
858-204-9185

Pitch Coaching and Partnership Facilitation

BIO International Convention

THE MEDTECH CONFERENCE
Powered by AdvaMed

RESI REDEFINING EARLY STAGE INVESTMENTS

LIFESCIENCES™ SUMMIT

ACA
ANGEL CAPITAL ASSOCIATION

NIH National Institutes of Health
Office of Extramural Research

More Enabling

NHLBI Small Biz Hangouts

NIH National Heart, Lung, and Blood Institute

YouTube http://bit.ly/SmallBizHangouts

Regulatory

- Navigating the FDA Website
- "First Contact" with FDA
- Finding the Right Regulatory Consultant
- Medical Device Regulation Overview

Commercialization

- Writing your Phase II Commercialization Plan
- Identifying and Connecting With Your Customer
- How Pharma Evaluates New Therapeutic Opportunities

Intellectual Property

- Intellectual Property Basics for the New Innovator
- Making Your Mark: The Use and Care of Trademarks
- Patent Litigation: Basics, Defense, and Offense – Parts 1 & 2

SBIR/STTR Reauthorization update

- Congress passed a 5 year SBIR/STTR Reauthorization as part of S.2943, the 2017 National Defense Authorization Act (NDAA)
- SBIR/STTR programs reauthorized for 5 year FY18 through FY22
- Simple, one line reauthorization
- No pilot programs extended, all expire 9/30/17
  - SBIR Direct Phase II
  - Commercialization Readiness Pilot Program (SB1)
  - 3% Agency SBIR Admin funds
  - NIH Phase 0 Proof of Concept Partnership Pilot
- Bills circulating in Congress as we speak on these and other areas. – Stay tuned.

SBIR and STTR reauthorization as part of the National Defense Authorization Act for Fiscal Year 2012 (HR1540)

SEC. 5127. PHASE 0 PROOF OF CONCEPT PARTNERSHIP PILOT PROGRAM (Pages 1094 - 1096)

"(jj) PHASE 0 PROOF OF CONCEPT PARTNERSHIP PILOT PROGRAM.—

"(1) IN GENERAL -- The Director of the National Institutes of Health may use $5,000,000 of the funds allocated under subsection (n)(1) for a Proof of Concept Partnership pilot program to **accelerate the creation of small businesses and the commercialization of research innovations from qualifying institutions**.

Phase 0 POC

"(3) PROOF OF CONCEPT PARTNERSHIPS.—

"(A) IN GENERAL.—A Proof of Concept Partnership shall be set up by a qualifying institution to award grants to individual researchers. These grants should **provide researchers** with the **initial investment** and the **resources to support the proof of concept work** and **commercialization mentoring** needed to translate promising research projects and technologies into a viable company. This work may include technical validations, market research, clarifying intellectual property rights position and strategy, and investigating commercial or business opportunities.

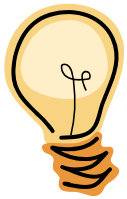
"(B) AWARD GUIDELINES

https://grants.nih.gov/grants/guide/rfa-files/RFA-HL-13-008.html

https://grants.nih.gov/grants/guide/rfa-files/RFA-OD-14-005.html

NIH Centers for Accelerated Innovations
Research Evaluation and Commercialization Hubs

Translate basic science discoveries into commercially viable products that improve health. REACH is fully and NCAI is partially funded through the Phase 0 Proof-of-Concept Centers provision of the 2011 STTR Reauthorization (Sec. 5127).



Innovation

NCAI
REACH

New Company

Licensing

Promising Technology	Private-Public Partnership	Patient and Societal Benefit

Turning discoveries into health

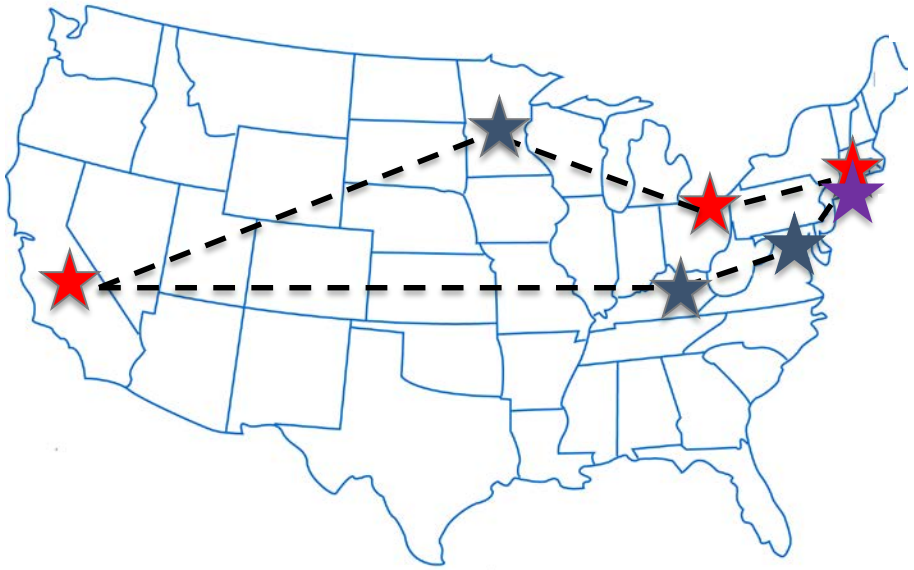
Proof-of-Concept Centers Support Milestone-Driven Development for Academic Innovators

NIH Centers for Accelerated Innovations (NCAI)

UC CAI
University of California
Center for Accelerated Innovation

NCAI-CC
Cleveland Clinic

Boston Biomedical Innovation Center



National Institutes of Health (NIH)

Research Evaluation and Commercialization Hubs (REACH)

SBU
BNL
CSHL
FIMR
Long Island Bioscience Hub

ExCITE
an NIH REACH Hub

MN-REACH
University of Minnesota
Coaching to Success

FDA
CMS
NSF
USPTO
KAISER PERMANENTE

http://ncai-reach.nhlbi.nih.gov

How do the NCAI and REACH Work?

NCAI and REACH

Developing a scalable model and best practices for accelerating academic discoveries toward therapies and cures to address unmet medical needs.

The Centers Provide Comprehensive Product Development Support

Up to **$400K in project funding**

Project management and coaching by industry-experienced mentors

Personalized feedback

FDA, CMS, USPTO
Kaiser Permanente
Life science industry experts

Training and Resources

Business development
Regulatory planning
Financing and partnerships

I-Corps at the NCAI and REACH

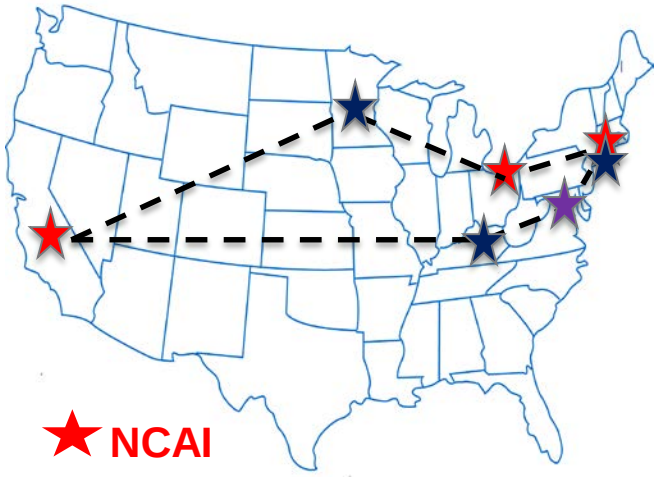
- NIH and NSF supported the three NCAI centers to develop best practices for tailoring the I-Corps curriculum for biomedical technologies.

- The centers leveraged this funding and continue to expand. For example, the Ohio center has extended its program to five partner institutions.

- The REACH hubs are all at institutions with NSF I-Corps Site awards and use the curriculum to strengthen the business elements of their technology development project proposals.

*Antman et al, Nature Reviews Drug Discovery***16**, 663–664 (2017)

Promising Output Indictors



★ NCAI

★ REACH

★ Program Partners

1047 Letters of intent and pre-applications

553 Full applications

220 Funded projects

Guidance from program partners ★

SBIR/STTR Award

8

New companies	Technology licenses and options	Follow-on Funding
		
31	**25**	**$485M**

https://ncai.nhlbi.nih.gov/ncai/

NIH National Institutes of Health
Office of Extramural Research

NIH Centers for Accelerated Innovations Program: principles, practices, successes and challenges

Commercializing innovations in academic environments is notoriously challenging. Here, we describe the progress of the NIH Centers for Accelerated Innovations program — initiated in 2013 to address these challenges — which we believe could help set a new standard for the early-stage commercialization of biomedical innovations in academic environments.

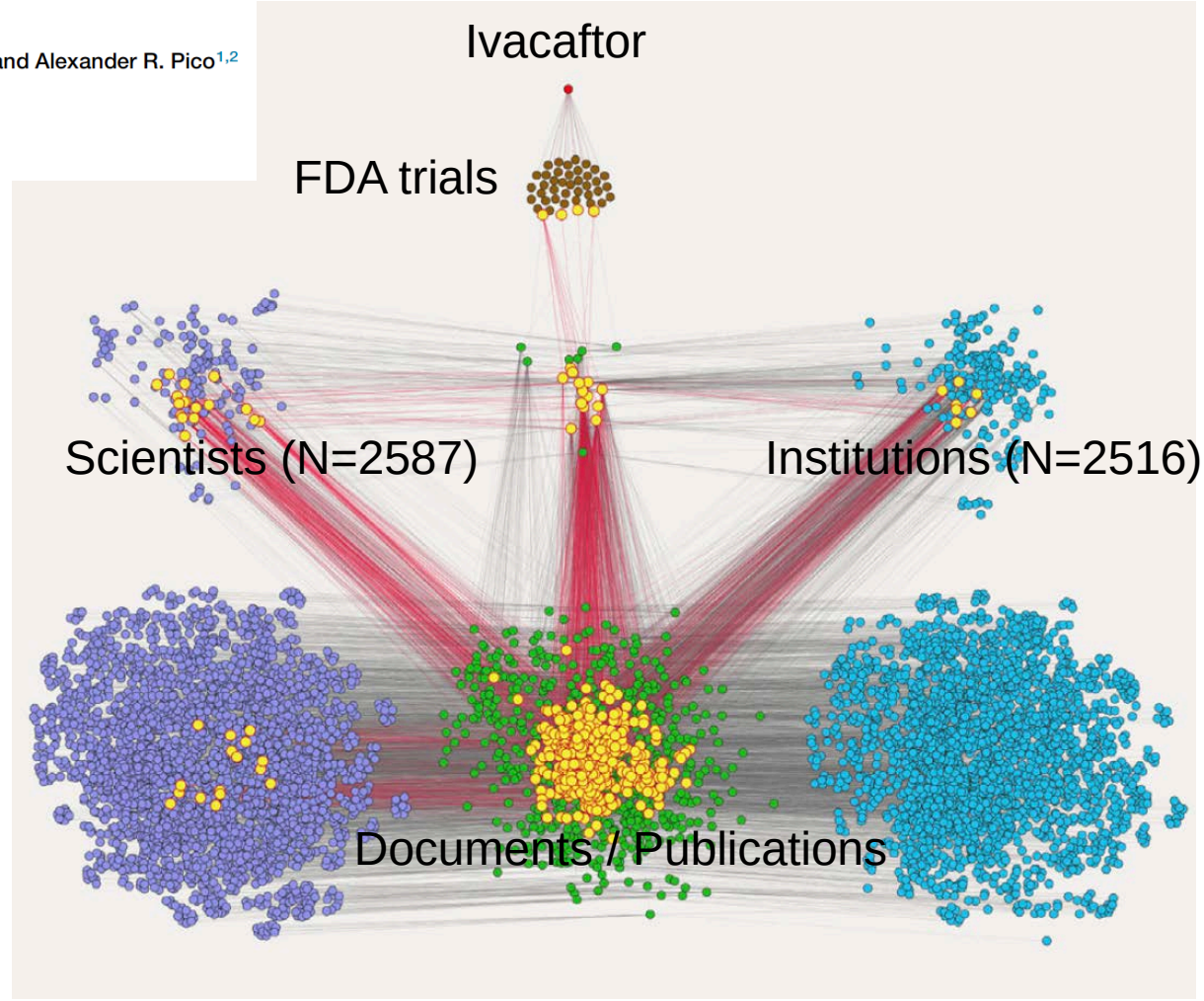
*Antman et al; Nature Reviews Drug Discovery***16**, 663–664 (2017)

Data Access Benefits Society

From Scientific Discovery to Cures: Bright Stars within a Galaxy

R. Sanders Williams,[1,2,*] Samad Lotia,[1] Alisha K. Holloway,[1,2] and Alexander R. Pico[1,2]
[1]Gladstone Institutes, San Francisco, CA 94158, USA
[2]University of California, San Francisco, San Francisco, CA 94143, USA
*Correspondence: rs.williams@gladstone.ucsf.edu
http://dx.doi.org/10.1016/j.cell.2015.09.007

"We propose that data mining and network analysis utilizing public databases can identify and quantify relationships between scientific discoveries and major advances in medicine (cures). Such approaches could enhance decision making..."



Cell 2015;163:21-23

A Quarter of a Century Ago …

Underreporting Research Is Scientific Misconduct

Iain Chalmers, FRCOG

"Substantial numbers of clinical trials are never reported … Failure to publish is a form of **scientific misconduct** that can lead to inappropriate treatment decisions. Investigators, ethics committees, funding bodies, and scientific editors all have responsibilities to reduce underreporting of clinical trials."

JAMA 1990;263:1405-8

FDAAA 2007

Public Law 110-85 Sept. 27,2007
110th Congress

Title VIII--Clinical Trial Databases, Sec 801

"(C) DATA SUBMISSION.—The responsible party for an applicable clinical trial, including an applicable drug clinical trial for a serious or life-threatening disease or condition, that is initiated after, or is ongoing on the date that is 90 days after, the date of the enactment of the Food and Drug Administration Amendments Act of 2007, shall submit to the Director of NIH for inclusion in the registry data bank the clinical trial information described in of subparagraph (A)(ii) not later than the later of—

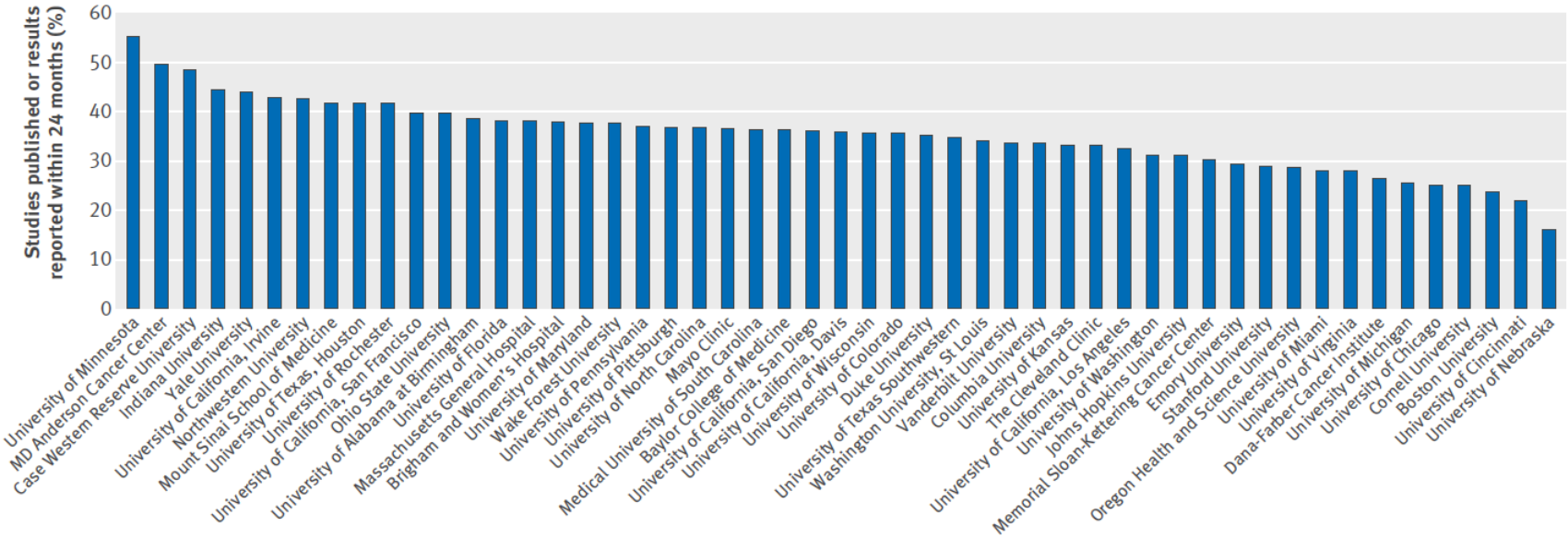
At *Your* Institution

RESEARCH

OPEN ACCESS

Publication and reporting of clinical trial results: cross sectional analysis across academic medical centers

Ruijun Chen,[1] Nihar R Desai,[2,3] Joseph S Ross,[3,4,5,6] Weiwei Zhang,[3] Katherine H Chau,[1] Brian Wayda,[7] Karthik Murugiah,[8] Daniel Y Lu,[9] Amit Mittal,[8] Harlan M Krumholz[2,3,5,6]



"Despite the **ethical mandate** and expressed values of academic institutions, there is poor performance and noticeable variation in the dissemination of clinical trial results across leading academic medical centers."

BMJ 2016;352:i637

“Sharing Results Should Not Be Optional”

OPINION POLICY-ISH

Academic Medical Centers Get An F In Sharing Research Results

February 23, 2016 · 1:59 PM ET

HARLAN KRUMHOLZ

Who will check the study results if they aren't made public?

Simone Golob/Corbis

“Not reporting results violates the basic principle of the scientific method. It hurts patients, society and science. It dishonors the people who gave their consent and bore the risk of participating...

The holding back of the results impedes progress toward scientific breakthroughs, corrupts the medical literature and wastes research funding.”

http://www.npr.org/sections/health-shots/2016/02/23/467712481/academic-medical-centers-get-an-f-in-sharing-research-results

Why it's important

- 70% phase II and 50% phase III trials fail
- Inadequate basic science
- Data integrity issues
- Failure negatively affects patients
- What is needed?
  - "a culture where the facts and data are confronted with brutal honesty"
  - Optimize Phase III trial design -- "Harness the vast amount of data available in public sources (e.g. Clinicaltrials.gov) to determine what worked…."

Grignolo and Pretorius, Applied Clinical Trials; Aug/Sep 2016

Now the Policy Exists… the Final Rule

42 CFR Part 11

[Docket Number NIH-2011-0003]

RIN: 0925-AA55

Clinical Trials Registration and Results Information Submission

Effective Date

This policy is effective January 18, 2017.

Date: September 12, 2016

Francis S. Collins, M.D., Ph.D.

Director

National Institutes of Health

NIH Policy on the Dissemination of NIH-Funded Clinical Trial Information

Notice Number: NOT-OD-16-149

"A fundamental premise of all NIH-funded research is that the results must be disseminated …

In research involving human beings, scientists have **an ethical obligation** to ensure that the burden and risk that volunteers assume comes to something, at the very least by ensuring that others are aware of the study and that its findings contribute…"

https://www.federalregister.gov/documents/2016/09/21/2016-22379/nih-policy-on-the-dissemination-of-nih-funded-clinical-trial-information

Is Your Study a Clinical Trial?

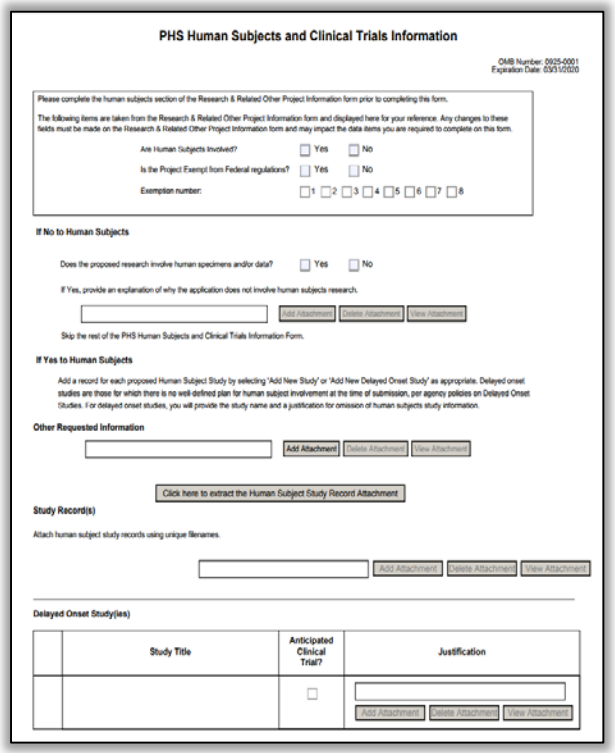
The 4 Questions

- Does the study involve **human participants**?
- Are the participants **prospectively assigned** to an **intervention**?
- Is the study designed to **evaluate the effect** of the intervention on the participants?
- Is the effect that will be evaluated a **health-related biomedical or behavioral outcome**?

If "Yes" to ALL of these questions, your study is considered a clinical trial

Clinical Trial Interactive Decision Tree: https://grants.nih.gov/ct-decision/index.htm

New Human Subjects and Clinical Trials Information Form



PHS Human Subjects and Clinical Trials Information

OMB Number: 0925-0001
Expiration Date: 03/31/2020

Please complete the human subjects section of the Research & Related Other Project Information form prior to completing this form.

The following items are taken from the Research & Related Other Project Information form and displayed here for your reference. Any changes to these fields must be made on the Research & Related Other Project Information form and may impact the data items you are required to complete on this form.

Are Human Subjects Involved? ☐ Yes ☐ No

Is the Project Exempt from Federal regulations? ☐ Yes ☐ No

Exemption number: ☐1 ☐2 ☐3 ☐4 ☐5 ☐6 ☐7 ☐8

If No to Human Subjects

Does the proposed research involve human specimens and/or data? ☐ Yes ☐ No

If Yes, provide an explanation of why the application does not involve human subjects research.

Add Attachment | Delete Attachment | View Attachment

Skip the rest of the PHS Human Subjects and Clinical Trials Information Form.

If Yes to Human Subjects

Add a record for each proposed Human Subject Study by selecting 'Add New Study' or 'Add New Delayed Onset Study' as appropriate. Delayed onset studies are those for which there is no well-defined plan for human subject involvement at the time of submission, per agency policies on Delayed Onset Studies. For delayed onset studies, you will provide the study name and a justification for omission of human subjects study information.

Other Requested Information

Add Attachment | Delete Attachment | View Attachment

Click here to extract the Human Subject Study Record Attachment

Study Record(s)

Attach human subject study records using unique filenames.

Add Attachment | Delete Attachment | View Attachment

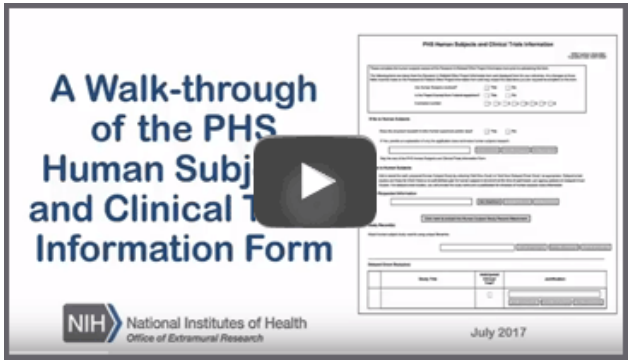
Delayed Onset Study(ies)

	Study Title	Anticipated Clinical Trial?	Justification
		☐	Add Attachment | Delete Attachment | View Attachment

Must apply to specific trials' announcements

NOT-OD-16-147

Take a video tour of the new form.



Review High Level Summary of Form Changes: FORMS-E to learn about other form changes.

NIH National Institutes of Health
Office of Extramural Research

Starting with application due dates on or after Jan. 25, 2018

NIH National Institutes of Health
Office of Extramural Research

Accountability, Ethical Mandate, Transparency

VIEWPOINT

Toward a New Era of Trust and Transparency in Clinical Trials

Kathy L. Hudson, PhD
National Institutes of Health, Bethesda, Maryland.

Michael S. Lauer, MD
National Institutes of Health, Bethesda, Maryland.

Francis S. Collins, MD, PhD
National Institutes of Health, Bethesda, Maryland.

"To realize the benefits of a clinical trial, the data must be broadly shared quickly. The DHHS has released a regulation for registration and summary results reporting. The NIH **will withhold clinical trial funding** if the agency is unable to verify adequate registration and results reporting…"

JAMA 2016 (online September 16, 2016)

What We Care About

Thanks to Brian Haugen, Cindy Danielson, George Chacko and Samet Keserci

Thank you