

Director's Report

NCATS Advisory Council and CAN Review Board

CHRISTOPHER P. AUSTIN, M.D.
DIRECTOR, NCATS
JANUARY 12, 2017

NCATS



We would like to save trees, but please let us know in advance of next Council if you would like the Activity Summary document printed

The *All of Us*SM Research Program Progress & NIH Engagement Discussion



NCATS Council
January 12, 2017



RESCHEDULED TO MAY COUNCIL

Eric Dishman
Director, *All of Us*SM Research Program



Outgoing CAN RB/Council Members

Thank you!

- **Freda Lewis-Hall, M.D.**
Executive Vice President
Chief Medical Officer
Pfizer Inc.
- **Geoffrey Ginsburg, M.D., Ph.D.**
Director, Center for Applied Genomics and Precision
Medicine
Duke University School of Medicine
- **Margaret Anderson, M.A.**
Executive Director
FasterCures, a Center of the Milken Institute



Outgoing CAN RB/Council Members

Thank you!

- Robert Beall, Ph.D.*
President and CEO
Cystic Fibrosis Foundation
- Jorge Contreras, J.D.
Associate Professor
University of Utah
- Louis DeGennaro, Ph.D.*
President and CEO
Leukemia & Lymphoma Society



Outgoing CAN RB/Council Members

Thank you!

- Eric Kodish, M.D.
F.J. O'Neill Professor
Director, Center for Ethics, Humanities, and Spiritual Care
Cleveland Clinic
- Ankit Mahadevia, M.D., M.B.A.*
Principal
Atlas Venture
- Bernard Munos, M.B.A.
Founder
InnoThink Center for Research in Biomedical Innovation



Outgoing CAN RB/Council Members

Thank you!

- Robert Tepper, M.D.*
Partner
Third Rock Ventures, L.L.C.



- Scott Weir, Pharm.D., Ph.D.
Director, Institute for Advancing Medical Innovation
University of Kansas Cancer Center



Incoming CAN RB/Council Members

Welcome!

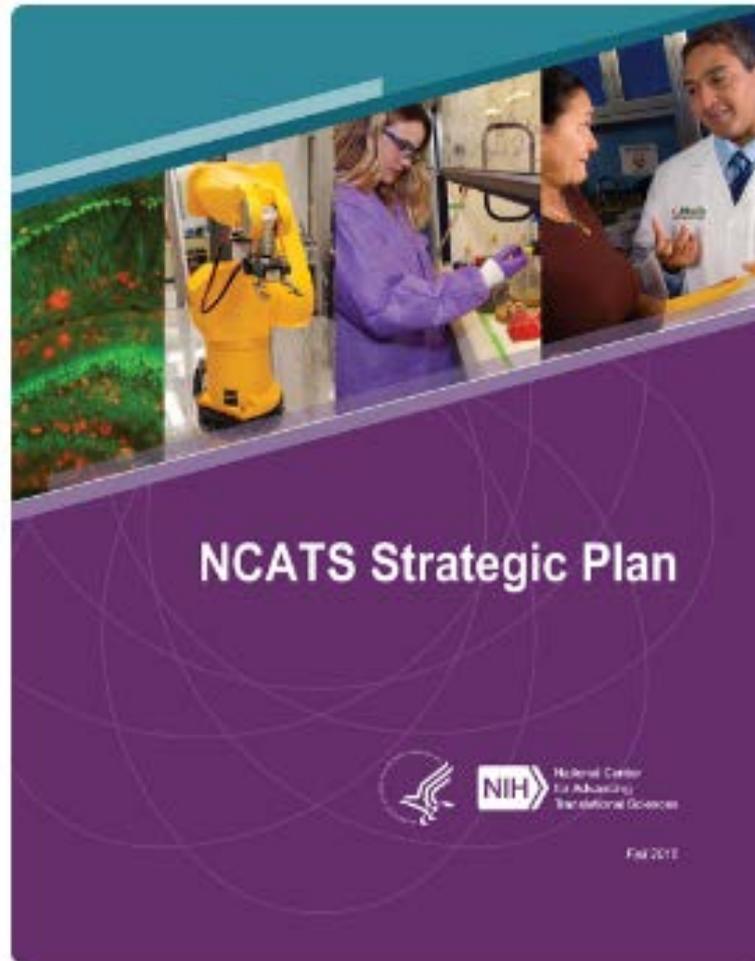
- Daniel Hartman, M.D.
Director
Integrated Development Global Health and Discovery Program
Bill and Melinda Gates Foundation
- Megan O'Boyle, B.A.
Principal Investigator
Phelan-McDermid Syndrome Data Network
- Alan Palkowitz, Ph.D.
Vice President
Discovery Chemistry Research and Technologies
Lilly Research Laboratories, Lilly Corporate Center



Policy and Legislative Updates



NCATS Strategic Plan



Released November 29, 2016

ncats.nih.gov/strategicplan



National Center
for Advancing
Translational Sciences

NCATS Strategic Plan

Strategic Principles

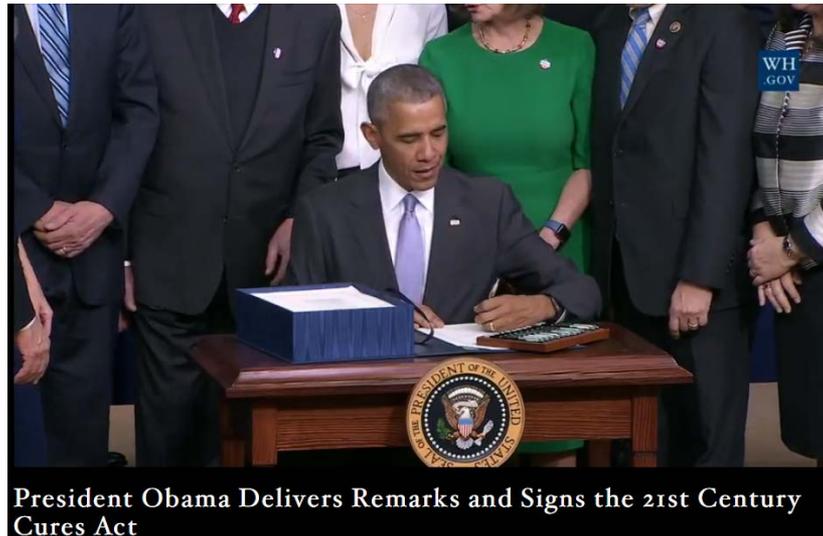
- **Catalytic:** NCATS is a catalyst that enables others to perform more efficient and effective translation.
- **Generalizable Principles:** NCATS uncovers fundamental principles shared among diseases and translational processes; widespread implementation of such generalizable principles will accelerate translation.
- **Innovative:** NCATS programs lead to profound improvements in translational understanding and effectiveness, producing innovation that establishes fundamentally new ways of doing translation that are multiplicative in their effects.
- **Collaborative:** Translational research endeavors require the expertise of multiple people and groups, particularly as the research is carried across through different phases of the translational science spectrum. NCATS approaches translation as a “team sport.”
- **Patient-focused:** At all phases of translational science, NCATS is committed to patients and their communities and looks for opportunities to include the patient perspective. The ultimate goal of translation is tangible improvement in health, so the perspectives of and partnerships with patients are crucial.
- **Measurable:** NCATS continuously improves translational effectiveness, so programs must be designed and implemented with explicit indicators of success for translational progress.

FY 2017 Budget

- FY2017 President's Budget request, Feb. 9, 2016
 - NCATS' request is \$685.417 million (same as FY 16)
- FY2017 Appropriation bills
 - House and Senate Appropriation committees each passed a Labor, HHS, and Education bill
 - Neither bill was voted on by the full chamber
- FY2017 began Oct. 1, 2016
 - Operating since Oct. 1 under a Continuing Resolution (CR) that runs through Apr. 28, 2017
 - Extends government funding at FY2016 level

21st Century Cures Act

- Nov. 30, 2016 - Passed the House by vote of 392-26
- Dec. 5, 2016 - Passed the Senate by a vote of 94-5
- Dec. 13, 2016 - President signed the bill



President Obama Delivers Remarks and Signs the 21st Century Cures Act

21st Century Cures Act

Relieves Administrative Burdens

- **For NIH:**
 - » Exempts NIH research from Paperwork Reduction Act
 - » Exempts NIH from conference and travel requirements in OMB Memo 12-12 & annual appropriations Acts
- **For Grantees:**
 - » OMB must establish Research Policy Board to review regulations across Federal science agencies
 - » HHS must examine financial conflicts of interest & financial expenditure reporting
 - » NIH must consider ways to reduce burden relating to sub-recipient monitoring and care and use of lab animal regs

21st Century Cures Act

Other Provisions of Interest

- Data Access and Privacy
 - » Exempts certain genomic information from FOIA requests
 - » Requires issuance of Certificates of Confidentiality and enhances their protections
 - » Authorizes the NIH Director to require funding recipients to share data
- Requires NIH Strategic Plan every 6 years
- Rigor and Reproducibility ACD Working Group; issue recommendations by Dec. 2017

21st Century Cures Act

Next Steps

- Legislative Implementation Work Group
 - » Comprised of Offices within the OD and ICs
 - » Meets to review statute
 - » Assigns implementation plans to the appropriate IC/OD Office
 - IC/OD office writing plan consults with relevant OD office as the plan is developed
 - Plans contain actions required to implement provisions, address significant policy, procedural or legal issues, describes new or revised regulations, guidelines or delegations needed
 - » Plan submitted to the Work Group for review
 - » Final plans submitted to NIH Director

Year in Review

Fiscal Year 2016



Budget

FY 2016: NIH IC Budgets

NIH IC	Approx FY16 Estimated Actuals (Dollars in Millions)
NCI	\$ 5,206
NIAID	\$ 4,750
NHLBI	\$ 3,109
NIGMS	\$ 2,509
NIDDK	\$ 1,964
NINDS	\$ 1,693
NIA	\$ 1,596
OD	\$ 1,571
NIMH	\$ 1,517
NICHD	\$ 1,338
NIDA	\$ 1,049
NIEHS	\$ 770
NEI	\$ 707
NCATS	\$ 684
NIAMS	\$ 541
NHGRI	\$ 513
NIAAA	\$ 467
NIDCD	\$ 422
NIDCR	\$ 413
NLM	\$ 395
NIBIB	\$ 343
NIMHHD	\$ 280
NINR	\$ 146
NCCIH	\$ 130
FIC	\$ 70

Largest



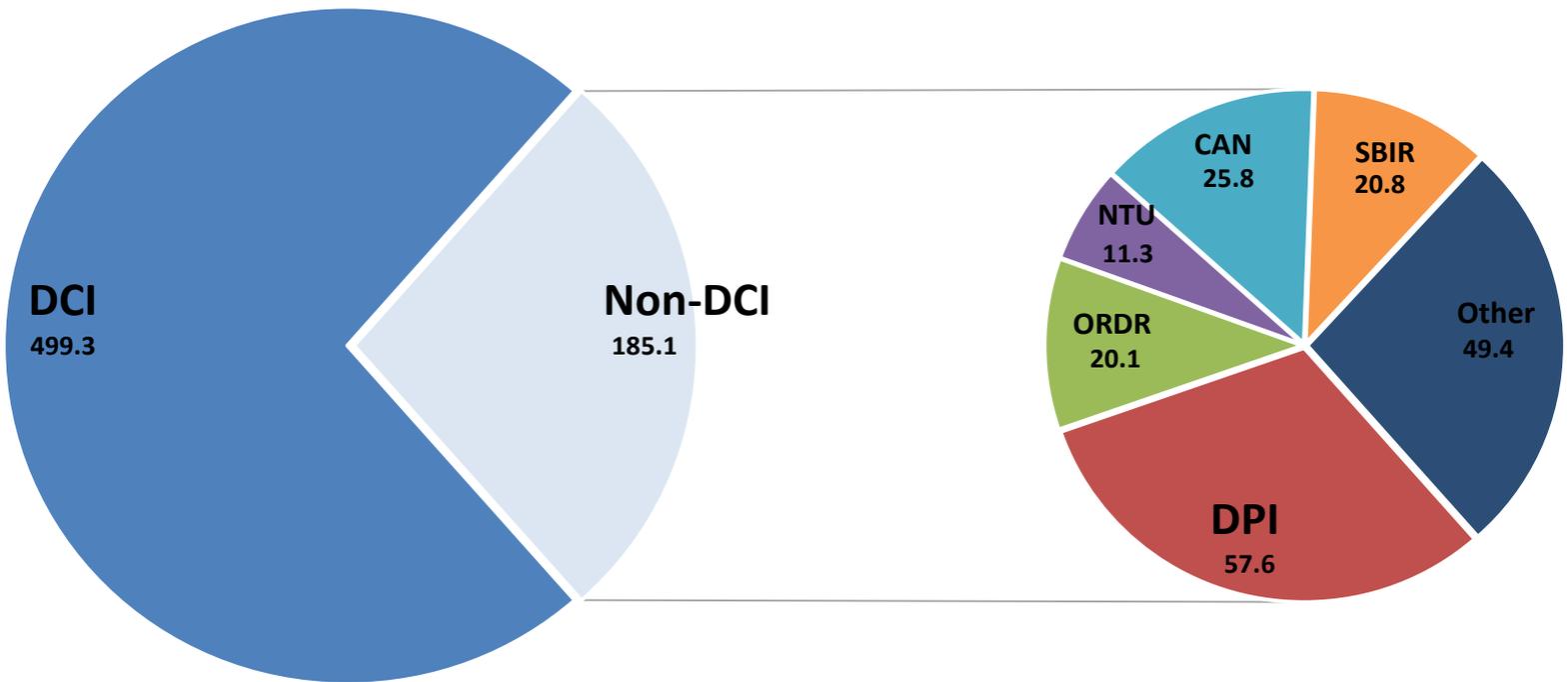
Smallest



Budget

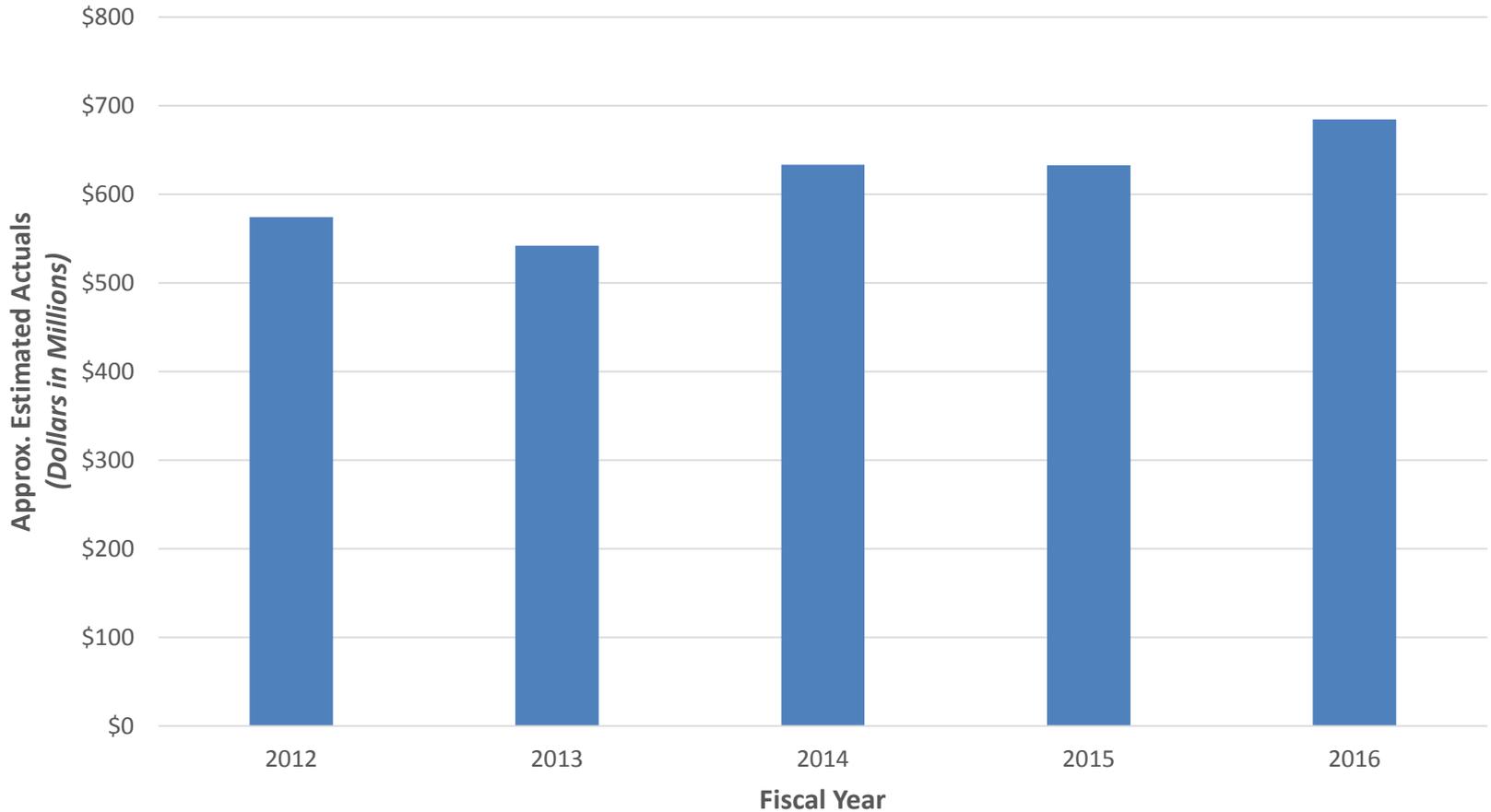
FY 2016: NCATS Appropriation

Total: \$684.4M*



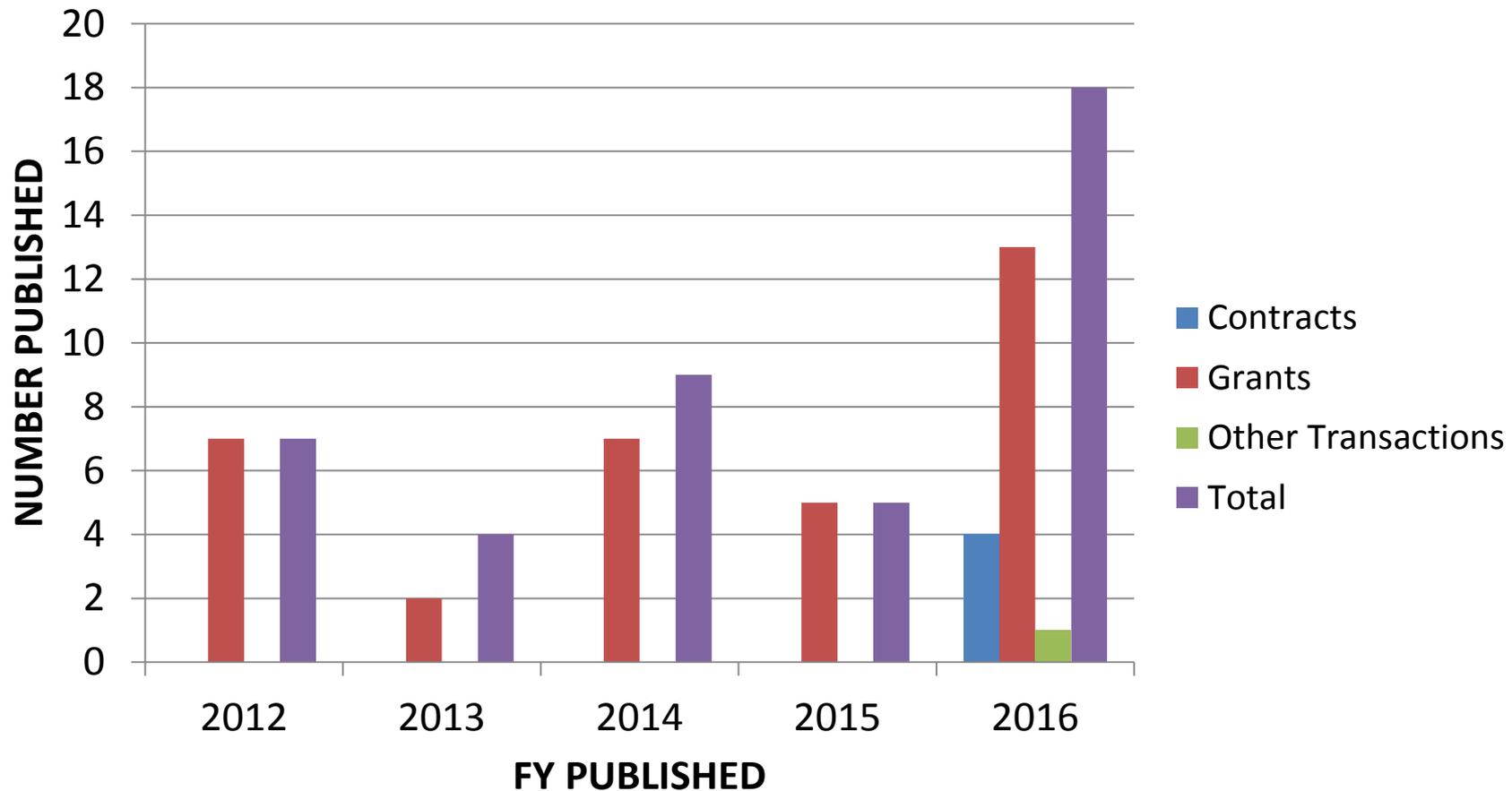
Budget

FY 2012-2016: Trends in NCATS Budget



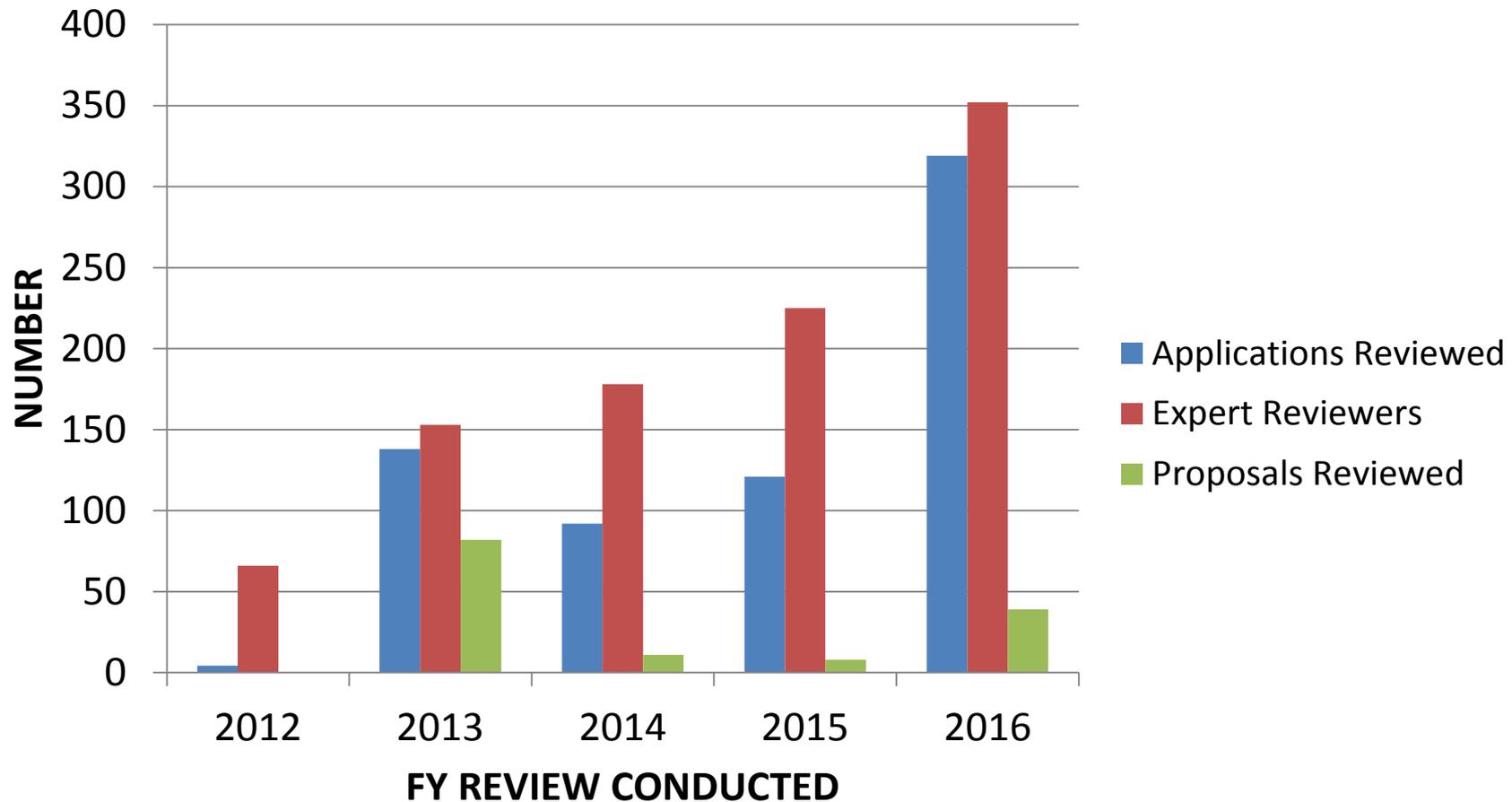
Solicitations

FY 2012-2016: Trends in NCATS Solicitations



Peer Review

FY 2012-2016: Trends in NCATS Peer Review



Evolution of the NCATS Solicitation Ecosystem



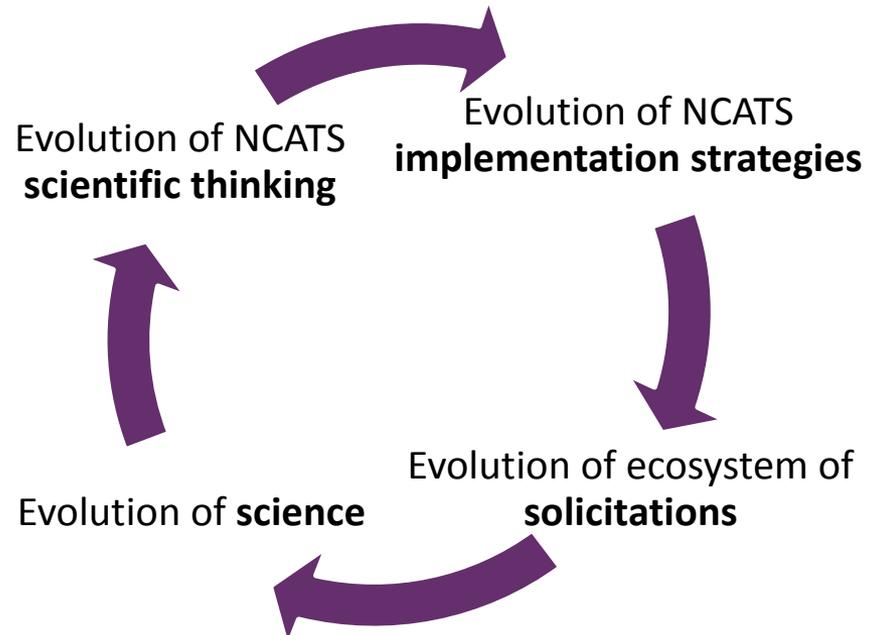
Funding and
Resource Access
Opportunities

Utilization of
Technical Experts
and Post-Review
Outcomes

Grants, Contracts,
Cooperative
Agreements, Other
Transactions,
Collaborations

Evolution of the NCATS Solicitation Ecosystem

- **Solicitations:**
 - » Vehicles by which NCATS seeks projects and services to undergo merit review
 - » Issued by both intramural and extramural
 - » They invite:
 - Contract proposals
 - Applications for access to NCATS resources
 - Applications for grants and cooperative agreements
- **3 examples:**
 - » CTSA Program
 - » NTU Program
 - » SBIR Program



CTSA Original RFA

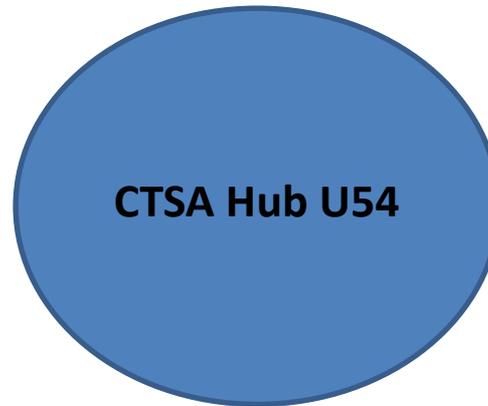


- “Legacy” FOA
- 1 receipt date per year
- Included minor post-IOM modifications

~FY 2012-2014

Current CTSA Hub PAR

CTSA Program



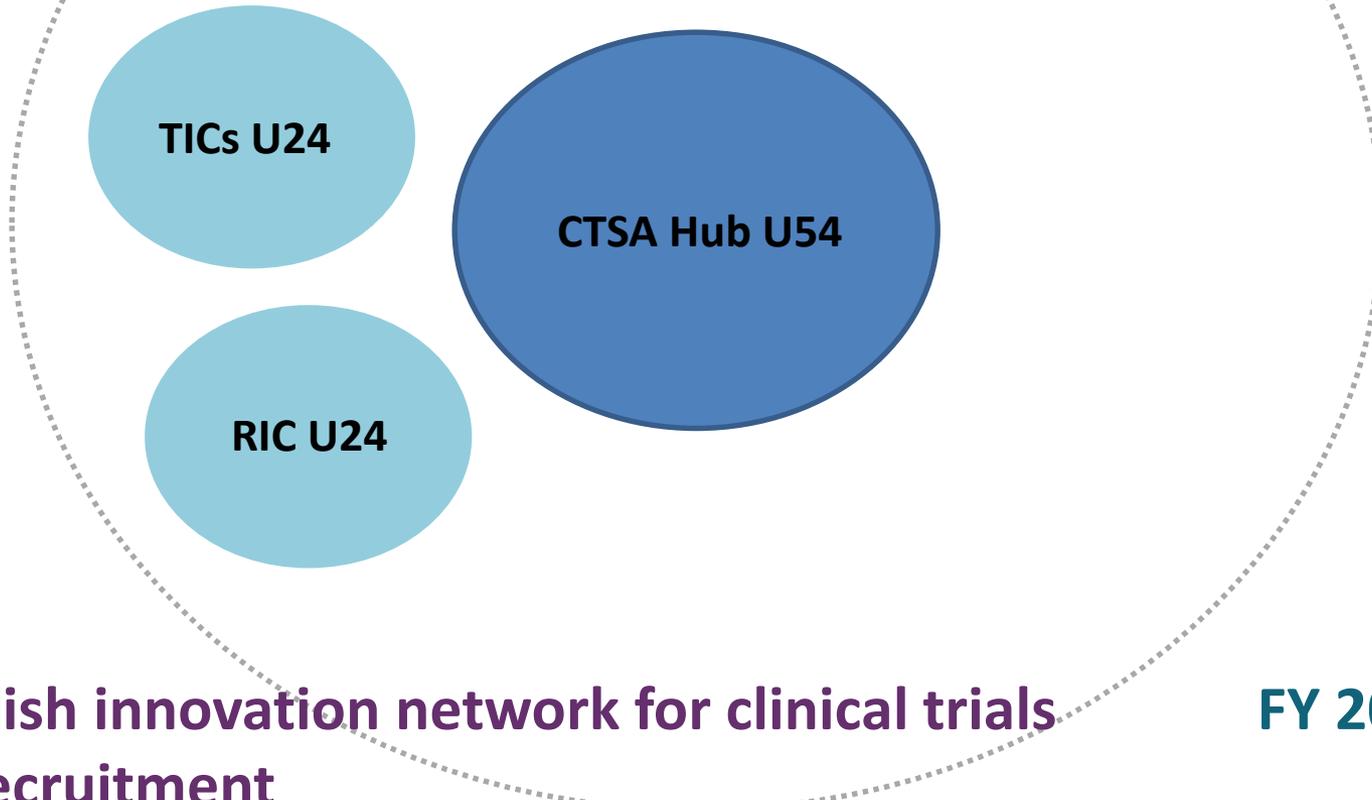
CTSA Hub U54

FY 2015

- **Current FOA(s)**
 - U01, KL2, TL1 components
- **3 receipt dates per year**
- **Open for 3 years**

CTSA Hub PAR + RIC, TICs

CTSA Program



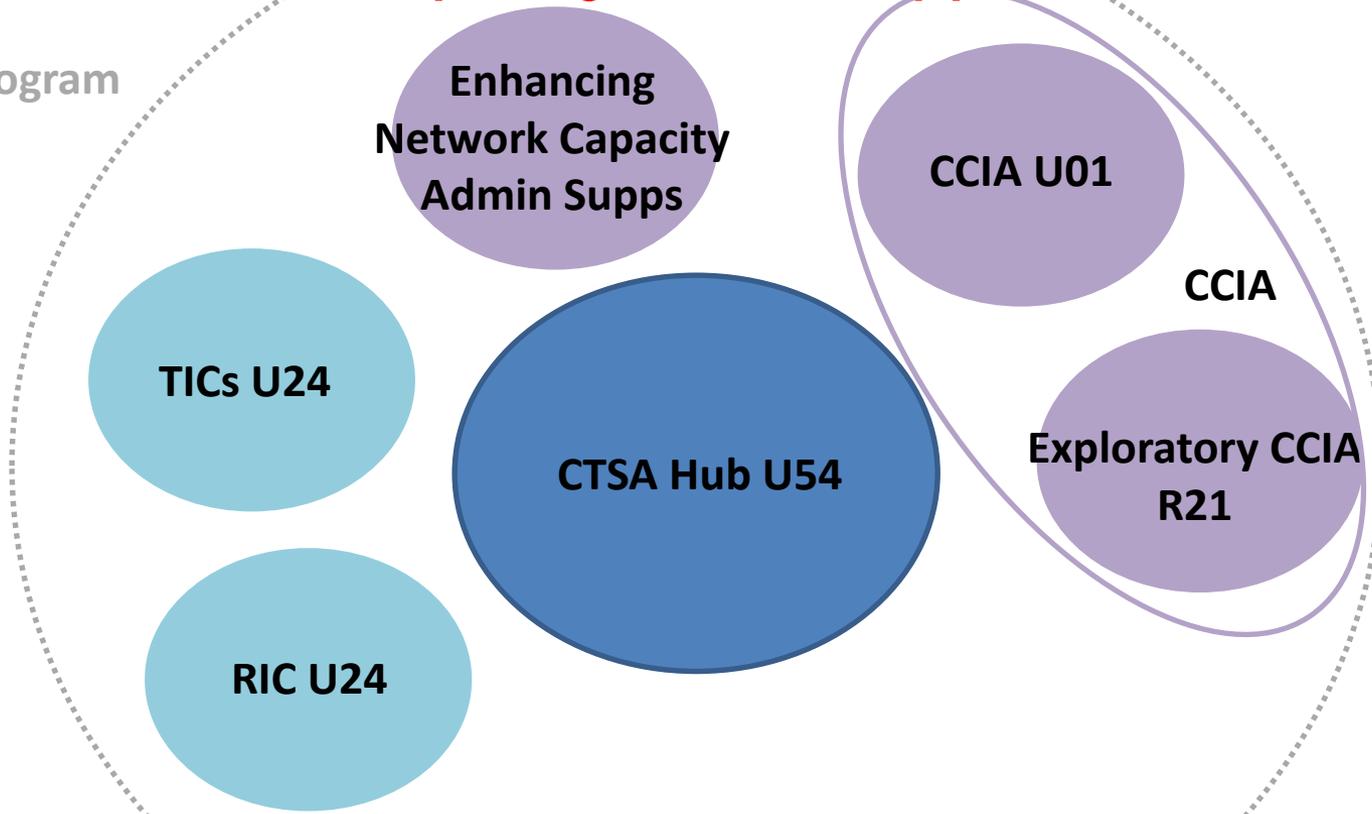
- **Establish innovation network for clinical trials and recruitment**

FY 2015

- **Trial Innovation Centers (TICs)**
- **Recruitment Innovation Center (RIC)**

CTSA Hub PAR + RIC, TICs + CCIA, Enhancing Network Capacity Admin Supps

CTSA Program

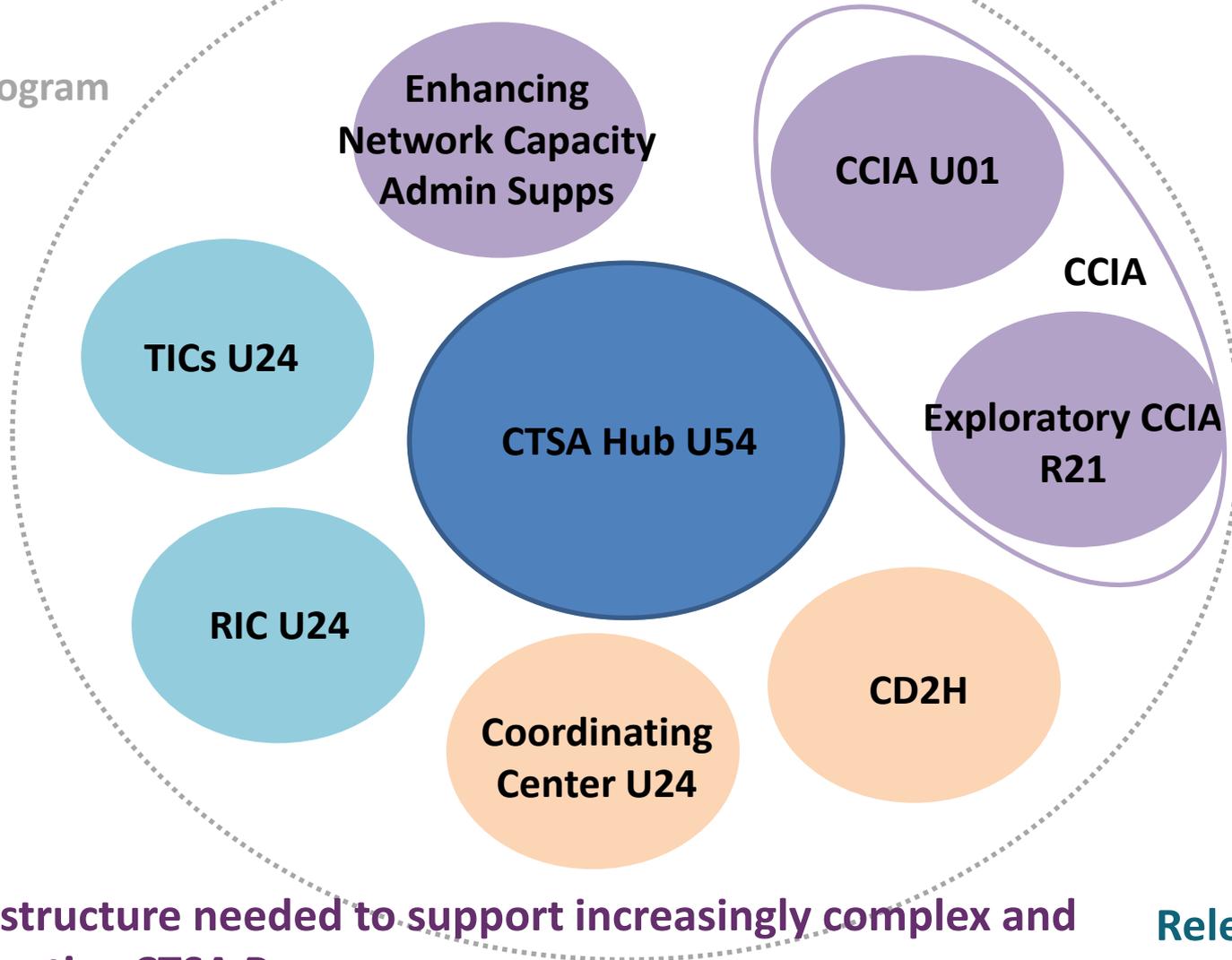


- Encourage innovation among CTSA awardees and allow non-CTSA awardee participation
 - CTSA Collaborative Innovation Award (CCIA) U01
 - Exploratory CCIA R21
- Enhance network capacity and encourage collaboration via admin supplements

FY 2015-2016

CTSA PAR + RIC, TICS + CCIA, Net Capacity + Coord Center, CD2H

CTSA Program



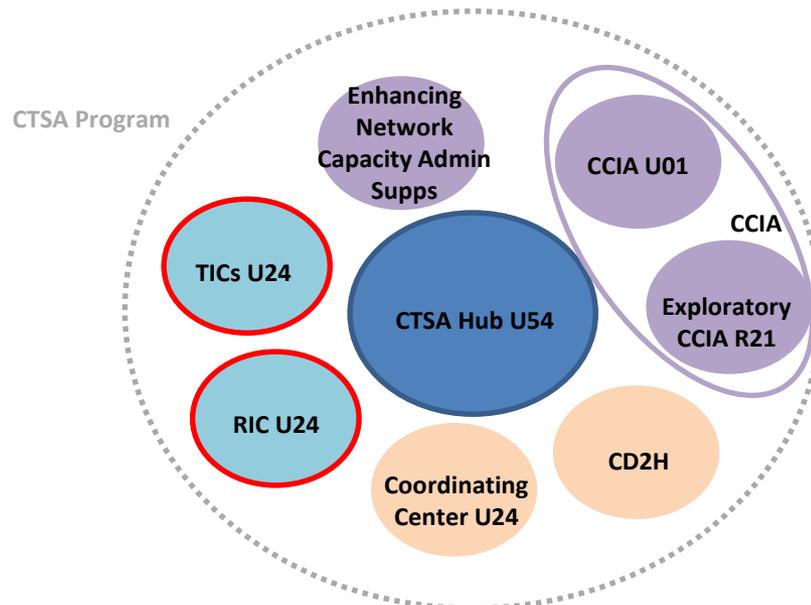
- **Infrastructure needed to support increasingly complex and productive CTSA Program**
 - **CTSA Program Coordinating Center U24**
 - **CTSA Program Data to Health (CD2H) FOA**

**Released
FY 2016-2017**

DCI Awards

FY 2016: Recruitment Innovation Center (RIC) & Trial Innovation Centers (TICs)

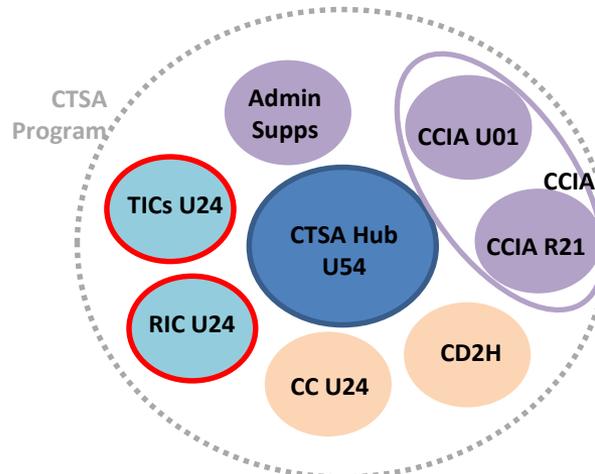
Title	PI	Grant Mechanism	Institution
RIC	Harris and Wilkins	U24	Vanderbilt University Medical Center
TICs	Benjamin and Bernard	U24	Duke University and Vanderbilt University Medical Center
	Dean	U24	University of Utah
	Hanley, Ford, and Selker	U24	Johns Hopkins University (Tufts University)



DCI Awards

FY 2016: Administrative Supplements for RIC & TICs

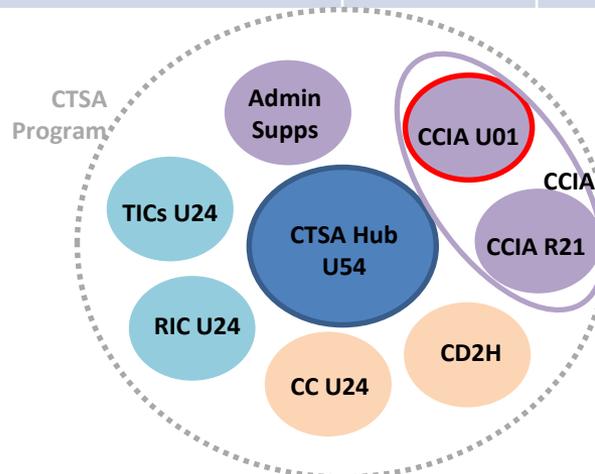
Title	PI	Parent Grant and Mechanism	Institution
Enhancing Recruitment of Minority Populations in Clinical Trials	Harris and Wilkins	Recruitment Innovation Center (U24)	Vanderbilt University Medical Center
Enhance, Innovate, and Harmonize Data Management, Data Collection, and DSMB Approaches	Benjamin and Bernard	Trial Innovation Center (U24)	Duke University and Vanderbilt University Medical Center
	Dean	Trial Innovation Center (U24)	University of Utah
	Hanley	Trial Innovation Center (U24)	Johns Hopkins University and Tufts University
Administrative Support to Foster Collaboration Across Trial Innovation Network	Benjamin and Bernard	Trial Innovation Center (U24)	Duke University and Vanderbilt University Medical Center



DCI Awards

FY 2016: CTSA Program Collaborative Innovation Award (CCIA) U01s

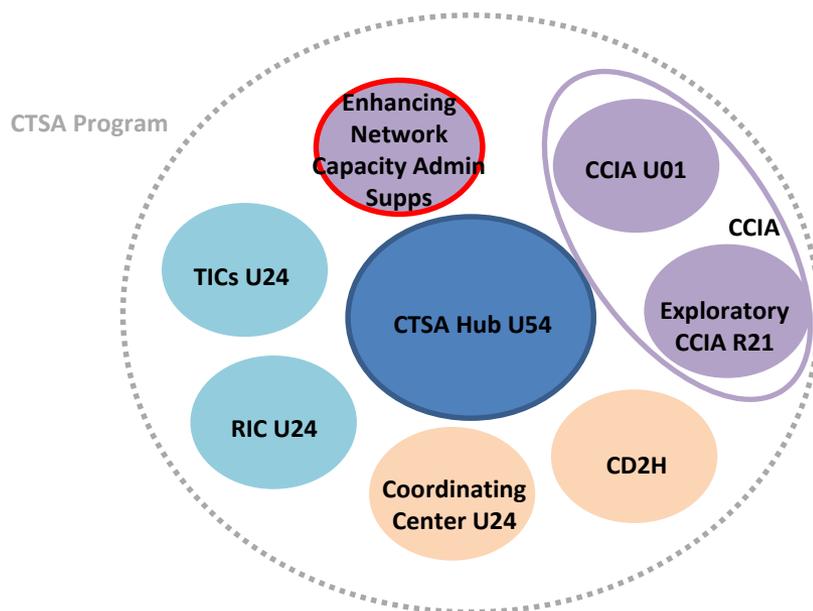
Title	Grant Mechanism	Collaborating Institutions
National IPS Cell Network with Deep Phenotyping for TR	U01	Boston U Med Campus, Harvard, U Chicago, U Penn
Disseminating Curative Biological Therapies for Rare Ped. Dz.	U01	Boston Children's, UCLA, U Cincinnati
Early Check: A Collaborative Innovation to Facilitate Pre-symptomatic Clinical Trials in Newborns	U01	Duke, Research Triangle Inst (UNC Chapel Hill), Wake Forest
Leveraging Existing Registry Resources to Facilitate Clinical Trials	U01	Duke, Johns Hopkins U, Vanderbilt
Improving PRO Data for Research Through Seamless Integration of the PROMIS Toolkit into EHR Workflows	U01	Harvard, Northwestern U, U Alabama Birmingham, U Chicago, U Florida, U Kentucky, U Illinois Chicago, USC, U Utah
Strengthening Translational Research in Diverse Enrollment (STRIDE)	U01	U Mass Medical School Worcester, U Alabama Birmingham, Vanderbilt U
Transformative Computational Infrastructures for Cell-Based Biomarker Diagnostics	U01	J. Craig Venter Inst, Stanford, UC Irvine



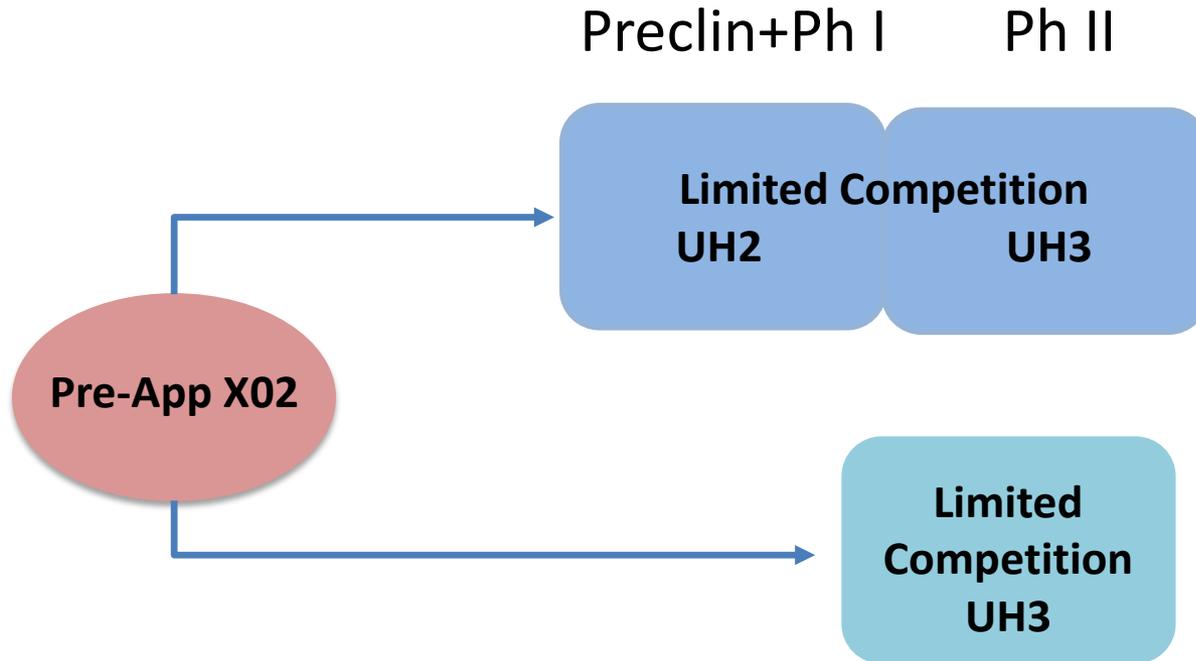
DCI Awards

FY 2016: Administrative Supplements for Enhancing Network Capacity

Title	PI	Grant Mechanism	Parent Grant Title	Institution
Optimizing Translational Veterinary Trials to Advance Human Outcomes	Jackson	UL1 (Admin Supp)	Ohio State U Center for Clinical and Transl. Sci.	Ohio State University
Real-time Genomic Analysis using iobio	Dere	UL1 (Admin Supp)	U of Utah Center for Clinical and Transl. Sci.	University of Utah
Disseminating Methods and Tools for the Design and Analysis of Randomized Clin Trials	Ford	UL1 (Admin Supp)	Institute for Clinical and Translational Research	Johns Hopkins University



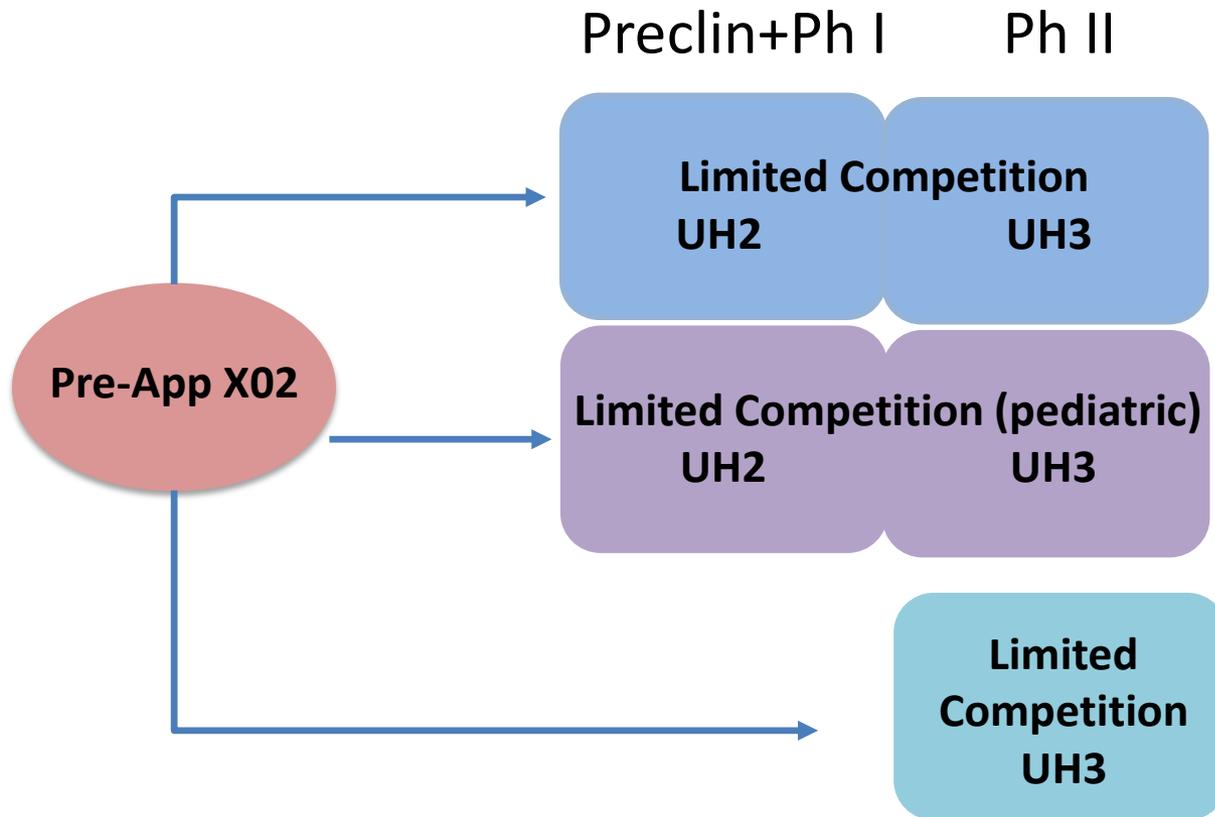
NTU Original Solicitation Suite



- Pre-applications required
- Afterward, 2 paths:
 - UH2/UH3 for Phase I/II (bi-phasic awards)
 - UH3 for Phase II only
- Transition milestones; 1 receipt date/year

FY 2012

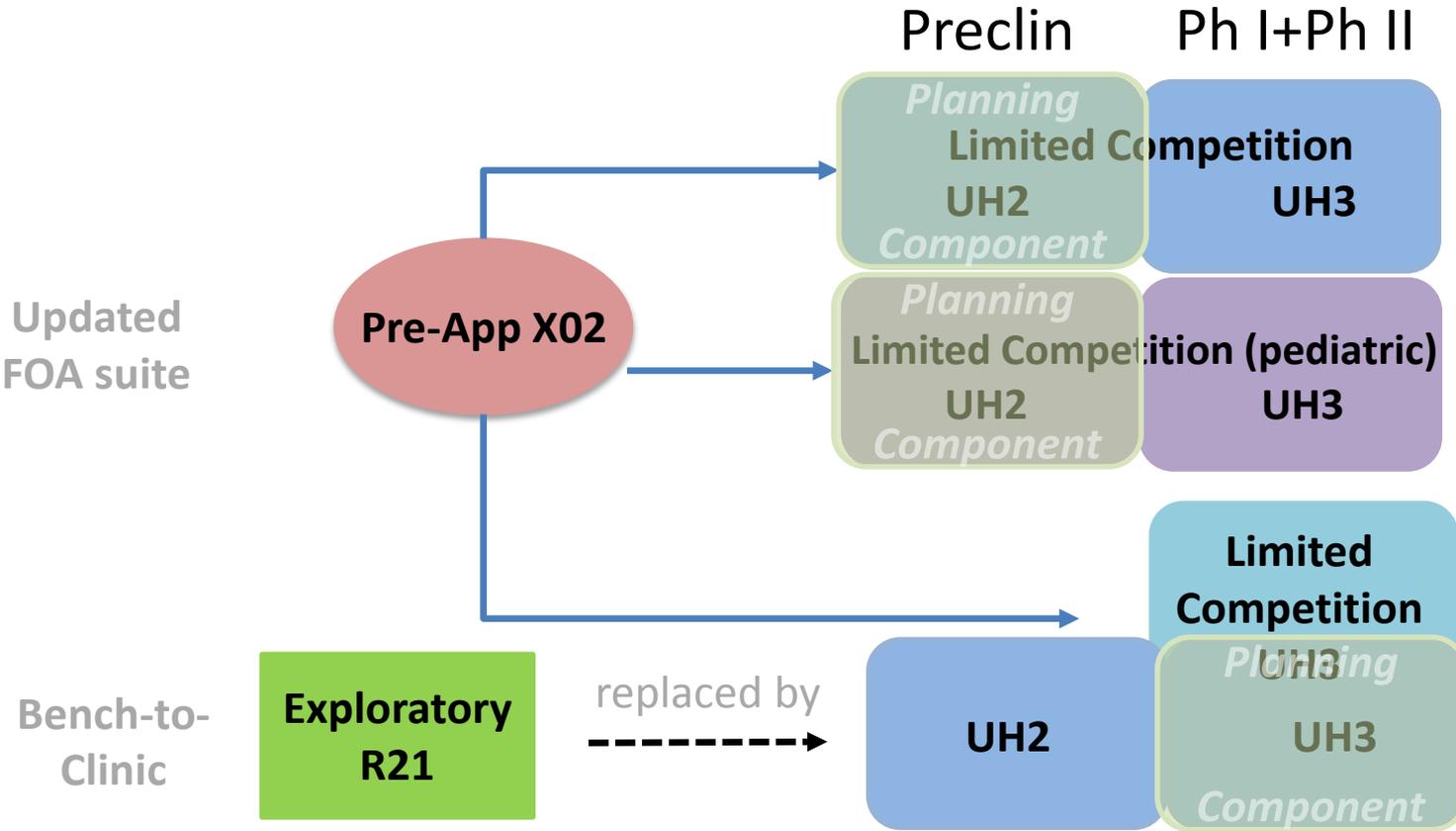
NTU Updated Solicitation Suite



- Original FOA suite reissued
- Added additional path for pediatric focus
 - UH2/UH3 for Phase I/II (bi-phasic awards) with additional year

FY 2014

NTU Current Solicitation Suite



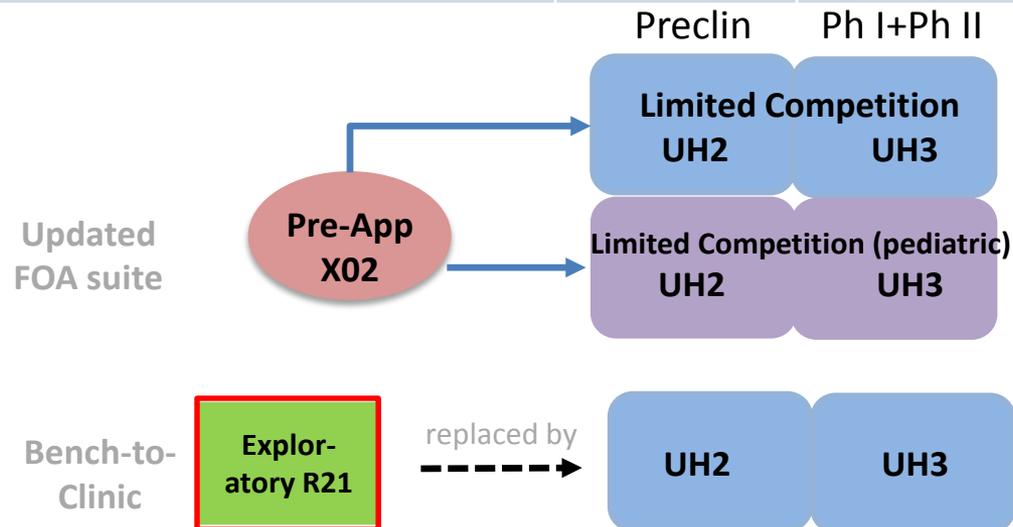
- Previous FOA suite to be published with updates
 - Adding planning component to awards for clinical research
 - Removing single phase awards (UH3)
- Added Bench-to-Clinic initiatives
 - Exploratory awards (R21); being phased out for bi-phasic awards (UH2/UH3)
 - Includes computational algorithms and marketed therapeutics

**Released
FY 2016-2017**

NTU Awards

FY 2016: Bench-to-Clinic exploratory R21s

Title	PI	Project #	Institution
Misoprostol for Clostridium difficile colitis	Aronoff	R21	Vanderbilt University
Lesogaberan for type 1 diabetes	Kaufman	R21	UCLA
Ketorolac and related NSAIDs for Ovarian Cancer	Wandinger-Ness	R21	UNM Health Sciences Center
Neutrophil elastase inhibitor for IBD	Kokkotou	R21	Beth Israel MC
CXCR antagonism for type 2 diabetes	Giannoukakis	R21	Allegheny-Singer Res. Inst.
Pyronaridine for Ebola virus	Davey	R21	Collaborations Pharma
Anti-virulence drug repurp. using structural systems pharmacol.	Xie	R21	Hunter College
Cethromycin for liver stage malaria	Sullivan	R21	Johns Hopkins Univ.
Network-driven approaches for coronary artery dz	Bjorkegren	R21	Icahn at Mount Sinai
Drug repositioning in diabetic nephropathy	Sarwal	R21	UCSF
Benserazide for colon cancer	Szabo	R21	UT Med. Br. Galveston



SBIR Program Objectives



Drive awareness of NCATS SBIR & STTR programs and conduct targeted outreach to women-owned and minority small biotech businesses and researchers

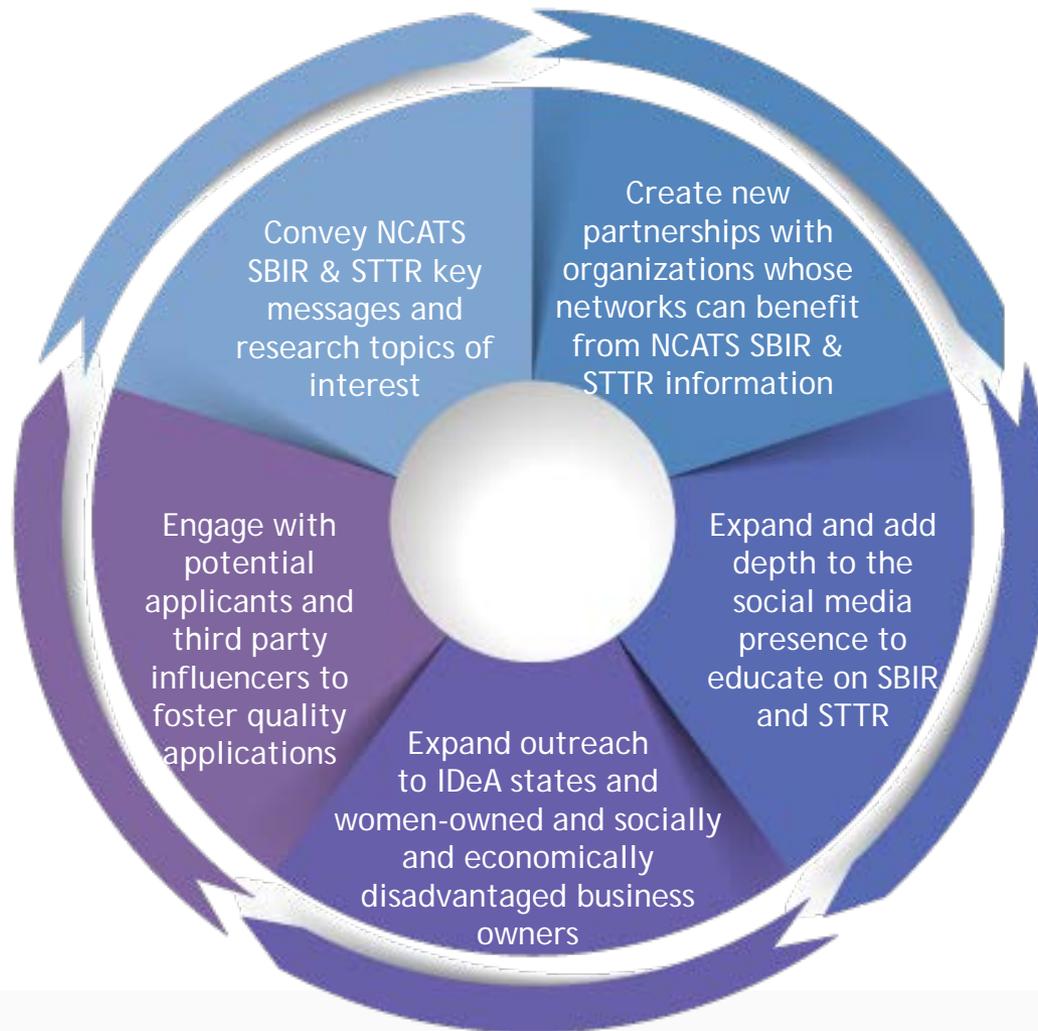


Increase the number of high-quality applications



Advance small businesses innovation among NCATS priority areas: drug development, discovery, research tools and technologies to improve translational research and patient care

Strategic Approach



Connecting the Dots - Bringing in a New Applicant

Ensuring Center's scientific priorities are reflected in funding focus areas

Creating opportunities to engage with researchers and entrepreneurs

Making program officers and SBIR program staff available to serve as a resource



Prospective Applicants

- Webinars
- Conferences
- 1: 1 Meetings
- Direct Email



Internal Partners

- NCATS Program Directors
- SBIR Leaders at other ICs



Successful Applicant

Expanding Engagement through New Partner Channels



Join #NCATSSbir, @AWISNational, & @NCISbir on 3/2 for a webinar on funding opportunities for women. Register here: attendee.gotowebinar.com/register/79060...



RT @ncats_nih_gov Prepare for #NCATSSbir & #STTR Jan. 5 funding app deadlines & review top submission errors. 1.usa.gov/1zZHGsx



Focusing on Priority Audiences and Targeted Opportunities

Women
Entrepreneurs
&
Researchers

Professional
Societies

Media

AWIS **AWIS**
@AWISNational

Follow

RT @JKWinnovation Join #NCATSSbir, @AWISNational & @NCISbir on 3/2 for webinar on funding opps for women. Register: goo.gl/cY2Tqe

An Evening With NIH

Monday, February 9, 6:30 - 8:30 pm

Join **Chris Austin**, Director, National Center for Advancing Translational Sciences (NCATS) as he chairs a special evening event focused on the array of programs, services and capabilities of the National Institutes of Health and how to best navigate the organization. Presentations include:

- **Facilitated Translation Within NCATS' TRND and BRiDGs Programs** Presenter: Nora Yang, National Institutes of Health (NIH)
- **NIH-Industry Partnership to Discover New Therapeutic Uses for Existing Molecules** Presenter: Christine Colvis, National Institutes of Health (NIH) *Learn more in ELN*
- **National Center for Advancing Translational Sciences (NCATS) SBIR and STTR Programs: Valuable Resources for Small Businesses** Presenter: Lili Portilla, National Institutes of Health (NIH)
- **NCI Experimental Therapeutics Program** Presenter: Barbara Mroczkowski, National Institutes of Health (NIH)



Chris Austin



Stakeholder Partnerships with a Focus on State Outreach and Women in Biotech

AWIS

ASSOCIATION FOR WOMEN IN SCIENCE
YOUR NETWORK, YOUR RESOURCE, YOUR VOICE

AZBIO

ARIZONA BIOINDUSTRY ASSOCIATION

BioNJ
The Gateway to Health

CLSA

California Life Sciences Association

CSBI

ColoradoBioScience
ASSOCIATION

CURE
Connecting Connecticut's Science Community

iBIO INSTITUTE
PUTTING SCIENCE TO WORK®

ihif
INDIANA HEALTH
INDUSTRY FORUM

ALBUQUERQUE BUSINESS FIRST
INNOVATION
NEW MEXICO

MassBio
MASSACHUSETTS BIOTECHNOLOGY COUNCIL

MICH
BIO

MONTANA
BIO SCIENCE
ALLIANCE

south dakota
biotech
Connect. Collaborate. Innovate.

Tech Council of MD
EMPOWERING LIFE SCIENCE AND TECHNOLOGY

virginia**bio**

Grew and strengthened relationships with 16 industry stakeholders

Historically Black Colleges & University Research/ Tech Transfer Centers



Grew and strengthened relationships with 20 historically black colleges and universities

Creating New Resources to Share NCATS SBIR/STTR News

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@nih.gov
- View current funding opportunities and spread the word: ncats.nih.gov/smallbusiness

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.

Learn more, view current funding opportunities and spread the word: ncats.nih.gov/smallbusiness

NCATS
National Center for Advancing Translational Sciences

Advancing Small Business Innovation
NCATS SBIR and STTR Programs

NCATS seeks to streamline the translational science process so that new prevention, diagnosis and treatment interventions can be advanced to patients faster. Through its Small Business Innovation Programs (SBIR and STTR) and Small Business Technology Transfer (STTR) program, NCATS invites small business entrepreneurs to research and develop, or prototype, offering grants, contracts and business assistance to small businesses and research organizations.

Funding Benefits

- Stable and predictable; one of the largest funding sources of small business
- IP rights are retained by the small business
- Not a loan; non-dilutive capital
- Points of contact are available to assist with the funding process

Funding Types

- Discovery Grant Substitution Applications due January 1, April 1 and September 1
- Contracts
- Applications typically due in October/November
- Grants in Targeted Areas
- Application deadlines vary

Have Questions? Need Help?

Submit a pre-award meeting to discuss a project idea. Email NCATS-SBIRSTTR@nih.gov for pre-award.

SBIR and STTR Program Structure

- Phase I: Establish technical merit, feasibility and potential for commercialization. Support: Usually \$150,000 over six months, with proper justification, you can propose more than one award for the project.
- Phase II: Continue research and R&D efforts initiated in Phase I. Support: Usually \$1 million over 3 years for SBIR and \$250,000 over six months for STTR. Note: Small businesses can apply for this award in Phase II if they have performed Phase I research using other funding.
- Phase III: Commercialization using non-NCATS/NIH funds to pursue Phase I and II goals.



Confirming an Appetite for Information from Key Audiences

Women Entrepreneurs & Researchers

Want Information and
Intend to Apply

AWIS Webinar: Based on
feedback from survey
respondents,

74 %

noted that they intend
to apply to the SBIR and
STTR programs.

Webinars Deliver Useful Information

CSBI Webinar: Overall,

98 %

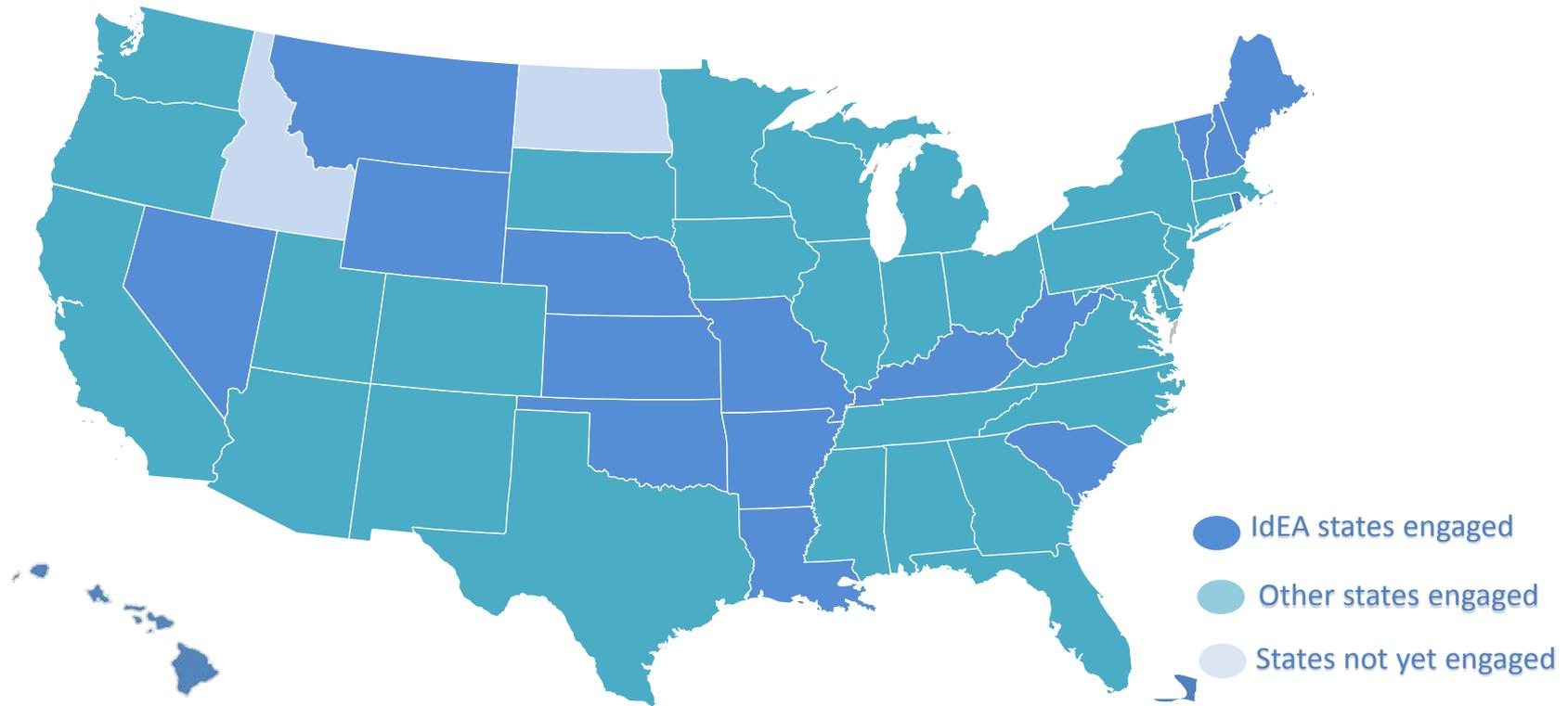
of respondents noted
that the presentation
helped them understand
more about the SBIR
and STTR programs.

Ongoing Outreach to IDeA States Makes a Difference

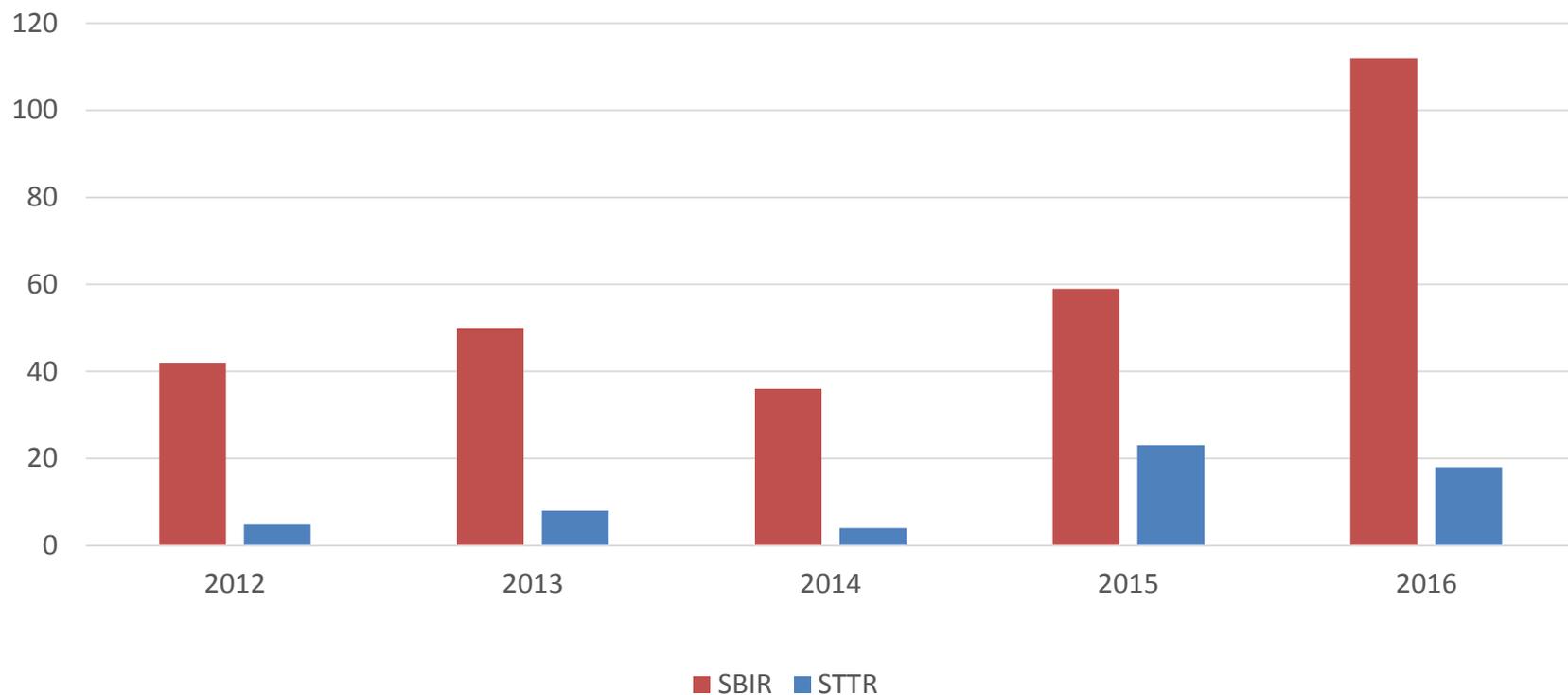
CSBI Webinar:
Registrants from
15 IDeA states
& Puerto Rico

AWIS Webinar:
Registrants from
11 IDeA states

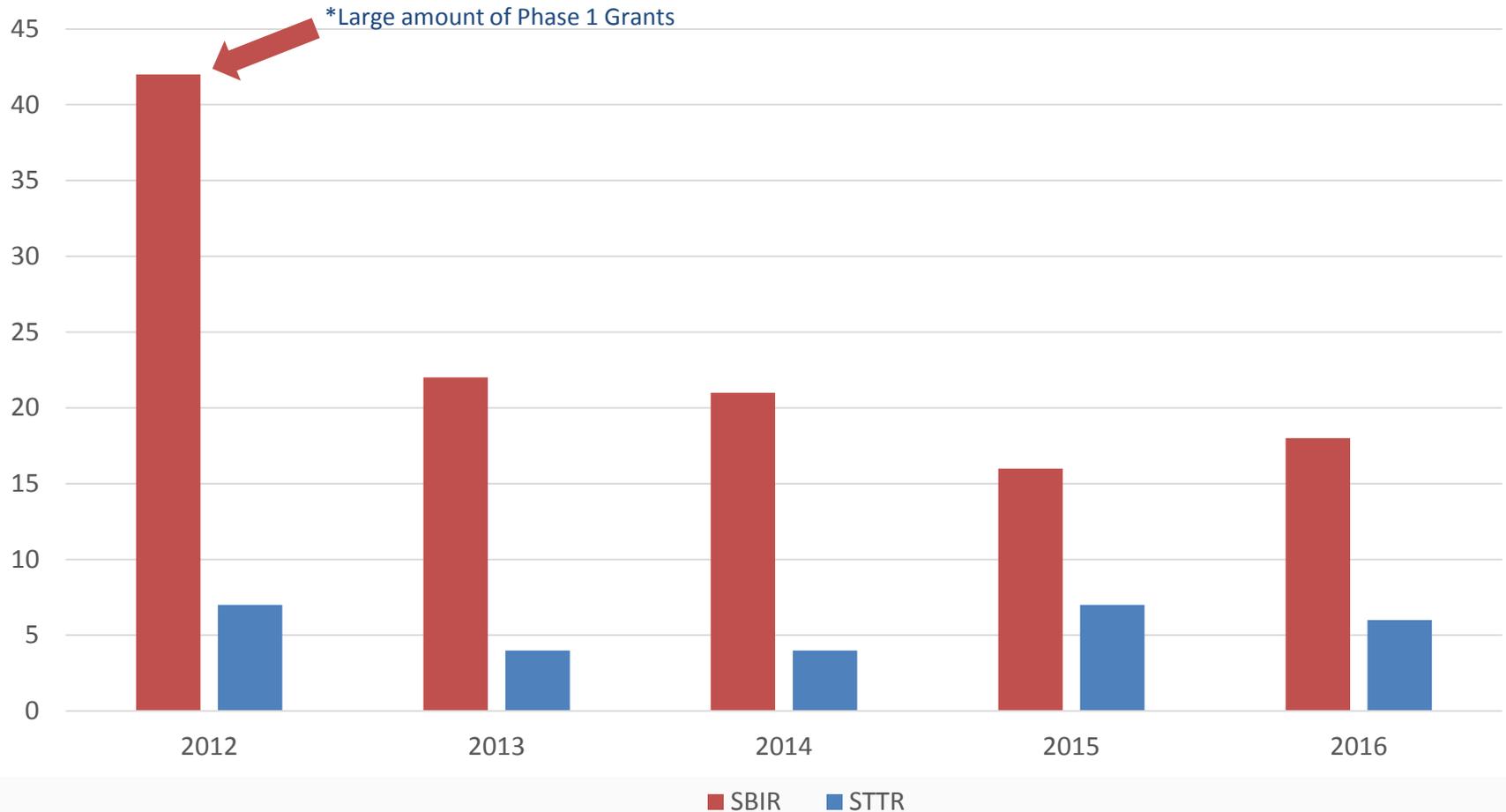
Disseminating Info about NCATS SBIR/STTR Across the Country



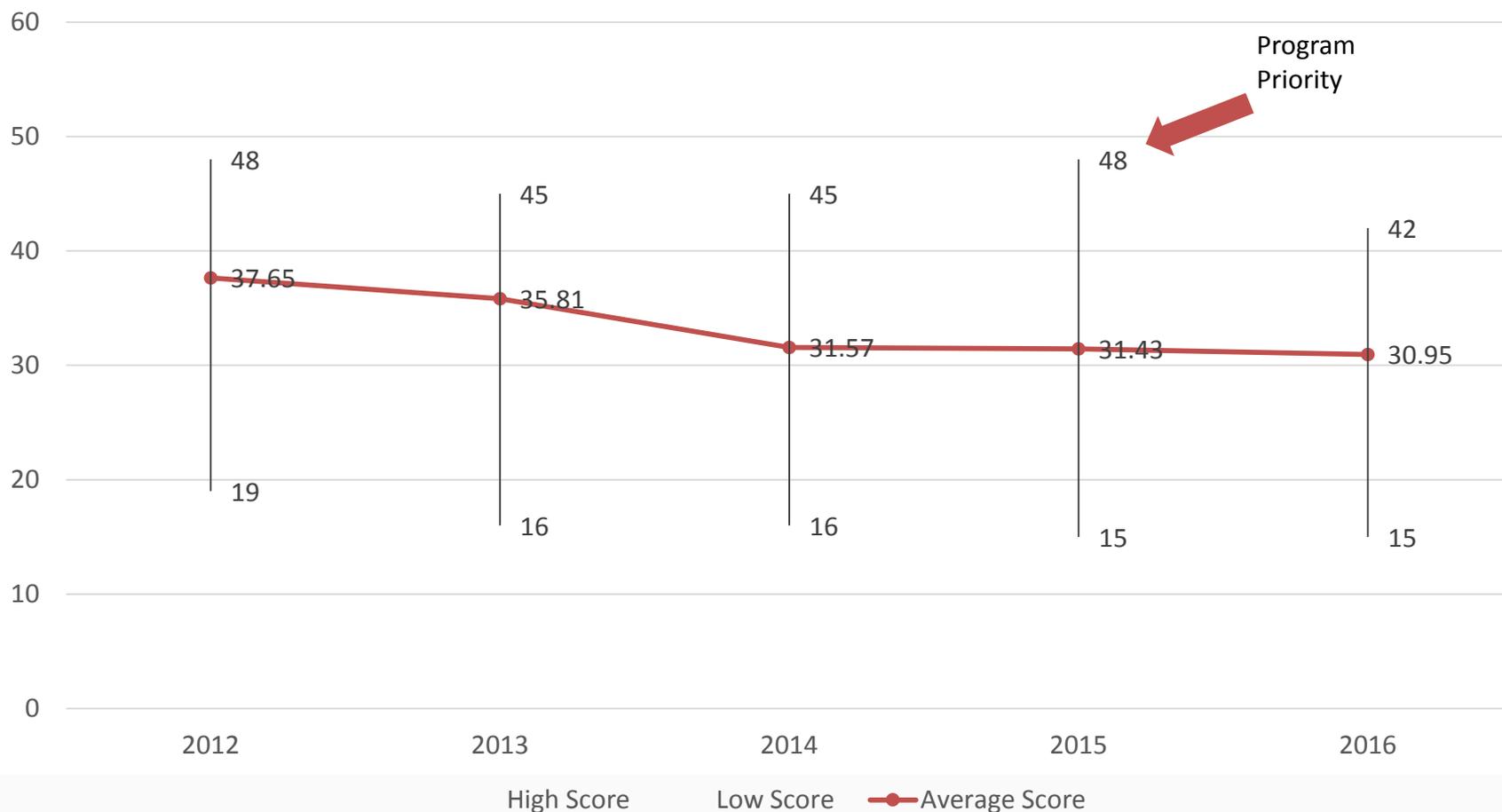
Number of Primary NCATS SBIR/STTR Applications Received 2012-2016



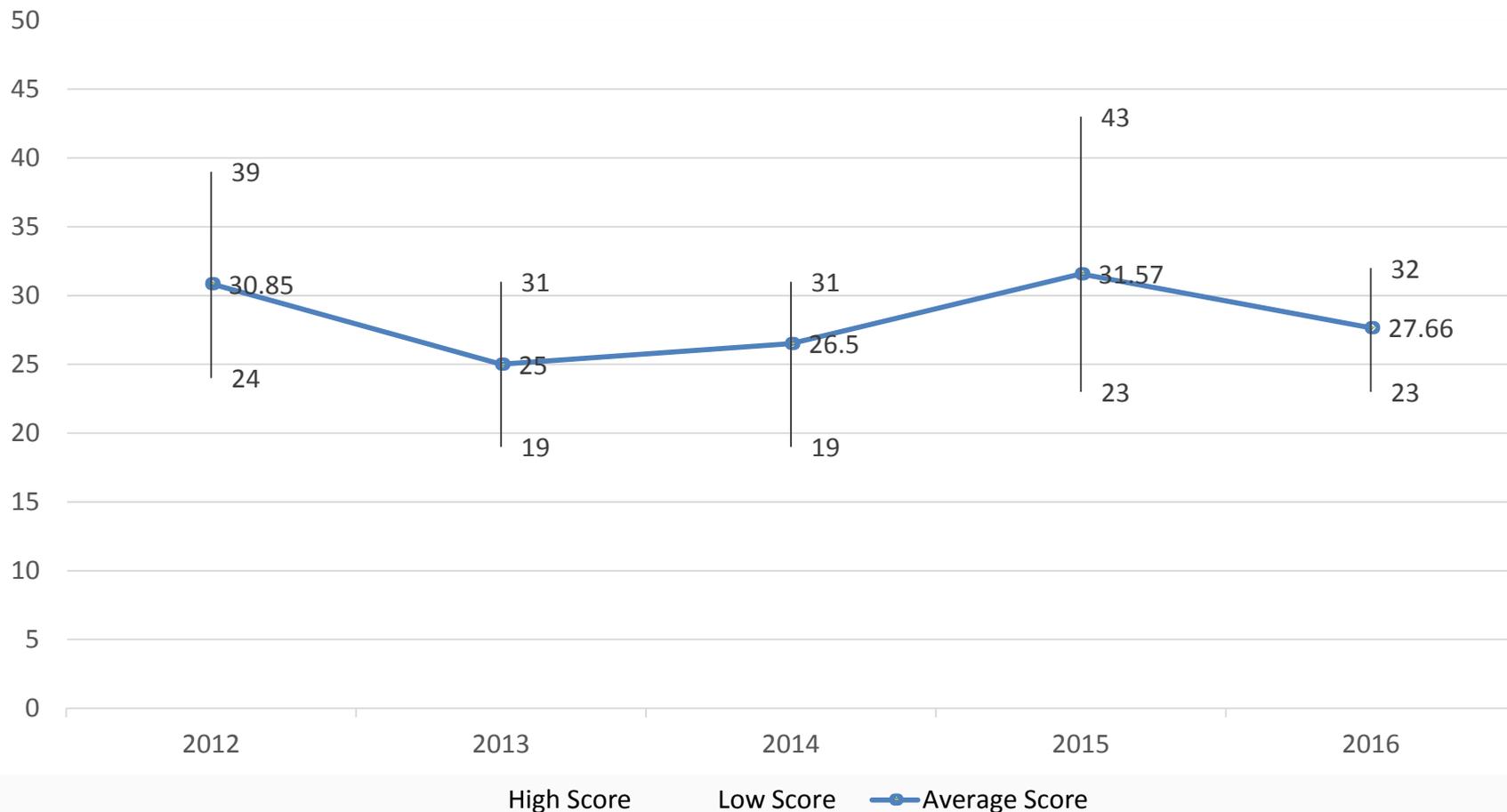
NCATS Awarded SBIR and STTR Applications 2012-2016



NCATS Awarded SBIR Applications 2012-2016 - Average Score by Funded Year



NCATS Awarded STTR Applications 2012-2016 - Average Score by Funded Year



NCATS Awardees Gaining Recognition



NCATS' Recursion Pharmaceuticals, a SBIR awardee, is featured as an SBIR awardee for its new platform for rare disease drug discovery



NCATS Plate Washing initiative, led by IonField, which saves money, keeps plastic out of landfills featured as a success story on the NCATS website



NCATS awardee AiCure develops smartphone application that confirms medication ingestion, making sure patients are taking the right meds at right time.

Summary: Key NCATS SBIR/STTR Achievements

Increased quantity and quality of applications; apps coming directly to NCATS vs other institutes

Increased social media education and engagement

Increased targeted engagement with women researchers and entrepreneurs

Increased SBIR leadership engagement with potential applicants & WOSB leadership role among NIH SBIRs

Increased awareness of the NCATS SBIR and STTR programs and interest in partnerships and applications

Discussion