

Director's Update

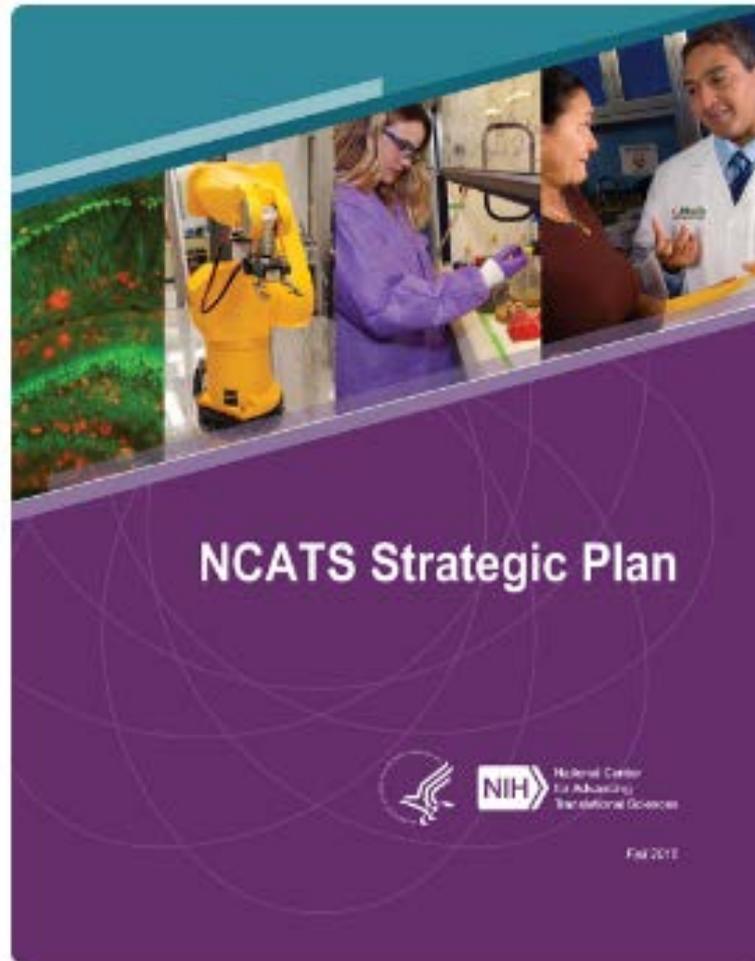
NCATS Cures Acceleration Network Review Board

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17TH MEETING OF THE CAN REVIEW BOARD
DECEMBER 9, 2016

NCATS

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.

NCATS Strategic Plan

Strategic Goals

1. Conduct and support innovative research that uncovers fundamental scientific and operational principles of translational science to catalyze the development and dissemination of novel medical interventions
2. Advance translational team science by fostering innovative partnerships and collaborations with a strategic array of stakeholders
3. Develop and foster innovative translational training and a highly skilled, creative and diverse translational science workforce
4. Enhance good stewardship of public funds by promoting and employing efficient and effective management practices

FY 2017 Budget

- FY2017 President's Budget request, February 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 October 1, 2016
 - Operating since Oct 1 under a Continuing Resolution (CR), which extends government funding at FY2016 level
 - Current CR - Runs through today, December 9
 - Next CR - Runs through April 28, 2017

21st Century Cures Act

- Passed by House (11/30) and Senate (12/7)
 - President expected to sign next week
- Designed to spur medical innovation
 - Primarily affects NIH and FDA
- Key NIH-related Provisions
 - Reauthorizes NIH through FY 2020
 - NIH Innovation Account - \$4.8 billion over 10 years
 - PMI, BRAIN, Cancer Moonshot, Regenerative Medicine
 - Funds come from Prevention and Public Health Fund and Strategic Petroleum Reserve
 - Must be appropriated each year, but does not count against budget caps (i.e. can't be used to offset regular appropriations)

21st Century Cures Act, cont.

- Key NIH-related Provisions (cont'd)
 - Data sharing
 - NIH Director allowed to require awardees to share data
 - Requires NIH Strategic Plan
 - Establishes 5-year renewable terms for IC Directors
 - Requires HHS Director to conduct review to reduce administrative burden for researchers
 - Exempts NIH research from Paperwork Reduction Act
 - Provides Other Transactions Authority (OTA) to PMI and 50% of the Common Fund
 - Report on OTA activities due by September 2020

21st Century Cures Act, cont.

- Key NCATS-related Provisions
 - Allows NCATS to support clinical trials through phase IIB (instead of IIA) and phase III (instead of IIB) for rare diseases
 - Changes NCATS' Annual Report to a biennial report
 - Report must include list of methods and tools developed, and whether they are being used by FDA

New Administration - Update

- HHS Secretary nomination - Rep. Tom Price
 - Representative from Georgia's 6th Congressional District (since 2005)
 - Chairs the House Budget Committee
 - Serves on the House Ways and Means Committee
 - Trained as an orthopedic surgeon
- *NIH has a long history of bi-partisan support and stands ready to work with the new Administration to improve people's health and reduce the burden of disease through biomedical research.*

Origins and Establishment of the Cures Acceleration Network (CAN)

Cures Acceleration Network (CAN)

Background

- The Patient Protection and Affordable Care Act (P.L. 111-148), which was signed into law on March 23, 2010, amended the Public Health Service Act to authorize the Cures Acceleration Network (CAN)
- CAN specifically directs the NIH to *accelerate the development of high-need cures*, by reducing the time it takes to move new drugs and therapies from the lab to the marketplace
- CAN was originally within the Office of the NIH Director and was moved into NCATS upon its inception in 2011

CAN

Functions (from Statute Language)

1. Conduct and support revolutionary advances in basic research, translating scientific discoveries from bench to bedside
2. Award grants and contracts to eligible entities to accelerate the development of high need cures
3. Provide the resources necessary for government agencies, independent investigators, research organizations, biotechnology companies, academic research institutions, and other entities to develop high need cures

CAN

Functions (from Statute Language, *cont.*)

4. Reduce the barriers between laboratory discoveries and clinical trials for new therapies
5. Facilitate review in the FDA for the high need cures funded by the CAN, through activities that may include:
 - a) Facilitation of regular and ongoing communication with FDA regarding the status of activities
 - b) Ensuring that activities are coordinated with the approval requirements of FDA, to expedite the development and approval of countermeasures and products
 - c) Connecting interested persons with additional technical assistance

CAN

Funding Mechanisms

- **Grant Awards** - Awards up to \$15M per project for the first fiscal year; potential additional funding in subsequent years
- **Matching Funds** - As a condition for receiving an award under this provision, an eligible entity shall contribute to the project non-federal funds in the amount of \$1 for every \$3 awarded
- **Flexible Research Authority** - To use other transaction (OT) to fund projects in accordance with the terms and conditions of this section, awards made under OT for a fiscal year shall not exceed 20% of the total funds appropriated under CAN for such fiscal year*

CAN

Budget Funding History

- FY 11 - \$0
- FY 12 - \$9.9 M
- FY 13 - \$9.4 M
- FY 14 - \$9.8 M
- FY 15 - \$9.8 M
- FY 16 - \$25.8 M
- *FY 17 - \$25.8 M (request)*

Five Years of CAN Thinking & Projects

The NCATS Recipe



CAN Project Criteria

- As recommended by the CAN Review Board (RB)...
 - Collaborative
 - Discrete and measureable outcomes
 - Broad and significant impact
 - Focused on a compelling disease
 - Timeline for completion of each project should be shorter than 5 years

CAN Project Selection Process

Timeline: 2014

- **January 2014**
 - NCATS reviewed previous translational-need landscaping exercises as starting points for potential CAN focus areas
- **May 2014**
 - ~10 CAN RB-generated broad focus areas based on previous landscaping exercises were presented
- **June 2014**
 - CAN RB members voted and commented on the scientific impact of those ~10 broad focus areas
 - NCATS selected three of the broad focus areas that had received wide support

CAN Project Selection Process

Timeline: 2014, cont.

- **July-August 2014**

- Three focus groups, including CAN RB members and NCATS staff members, were formed around these top areas:
 - **Micro-awards for researchers who need to get past small preclinical hurdle**
 - Rick Kodish (RB)
 - Scott Weir (RB)
 - Dorit Zuk
 - Xin Xu - *ad hoc*
 - Sitta Sittampalam
 - Christine Cutillo
 - **Devices and sensors to detect clinical outcomes**
 - Todd Sherer (RB)
 - Paul Yock (RB)
 - Elaine Collier
 - Todd Wilson
 - Dan Tagle
 - Christine Cutillo
 - **Insufficient access to compounds, toxicology/PK, patient populations**
 - Bob Beall (RB)
 - Ankit Mahadevia (RB)
 - Christine Colvis
 - Asaf Alimardanov - *ad hoc*
 - Carson Loomis
 - Christine Cutillo
- The focus groups met and worked on potential concept ideas within those broad focus areas that would lend themselves to the CAN project criteria

CAN Project Selection Process

Timeline: 2014-2016

- **September 2014**
 - Concept clearance presentations
 - Discussion of scientific ideas of all concept clearances
- **December 2014**
 - NCATS considered concepts based on input received, feasibility, impact in the long-run, and implementation strategies
- **December 2015**
 - Revisited 3 CAN-generated and 4 NCATS-generated concepts
 - In order to fill pipeline if CAN were to receive funding
 - Prioritization by CAN RB
- **2016**
 - CAN RECEIVES ADDITIONAL FUNDING!
 - NCATS initiated 3-D Bioprinting and Translator project (using OTA)

CAN Approved Concepts

- Via 2014 CAN RB project selection process
 - Increasing Access to Compounds and Tox Data
 - Proof of Principle (POP) Awards
 - Sensors and Devices to Detect Clinical Outcomes
- Via 2015 NCATS-generated concept clearances
 - Tissue Chip Testing Centers: Validating Microphysiological Systems
 - SaME Therapeutics: Targeting Shared Molecular Etiologies underlying multiple diseases to accelerate translation
 - 3-D Bioprinting of Human Tissues for Drug Screening
 - Proteomic Profiling for Clinical Applications