INSTITUTE OF MEDICINE
OF THE NATIONAL ACADEMIES

Board on Health Sciences Policy — *Forum on Drug Discovery, Development, and Translation*

Maximizing the Goals of the Cures Acceleration Network to Accelerate the Development of New Drugs and Diagnostics: An Institute of Medicine Workshop

DRAFT AGENDA

June 4–5, 2012

National Academy of Sciences
Keck Building, Room 100
500 5th Street, N.W.
Washington, DC 20001

Background and Meeting Objectives:
Recent years have seen both extraordinary opportunity and complex challenges in pharmaceutical innovation. New biomedical technology platforms are creating novel avenues for research and new opportunities for the discovery and clinical development of innovative diagnostics and therapies. Yet despite these advances, the pathway from basic science to new therapeutics faces challenges on many fronts. The translational divide results in only a small fraction of investigational new drugs reaching FDA approval and the patients who need them. New paradigms for discovering and developing drugs are being sought to bridge the ever-widening gap between scientific discoveries and translation of those discoveries into life-changing medications. New collaborative approaches within the federal agencies, academia, and industry are directing focused attention on the advancement of the drug development enterprise. Among these initiatives is the Cures Acceleration Network (CAN), which was originally authorized in the Patient Protection and Affordable Care Act (P.L. 111-148) and was subsequently amended by the Consolidated Appropriations Act, FY 2012 (P.L. 112-74), which moved CAN to the newly authorized National Center for Advancing Translational Sciences (NCATS).

This public workshop will consider options and opportunities to maximize the usefulness and impact of the CAN program in order to advance translational sciences. In addition to providing suggestions to NCATS, the workshop is, in part, in response to Congressional interest in CAN expressed in the FY 2012 appropriations act conference report. The workshop will inform NIH/NCATS in its efforts to implement CAN and advance translational sciences, and will also inform the public, policy community, and other stakeholders as all of these parties continue to work to enhance the development and testing of therapies and diagnostics to patients. The summary will be provided to NCATS and the newly established CAN Board to help it identify ways to accelerate and expand the number of cures.

The workshop objectives are to:

- Identify and catalog potential tools, methods, and approaches that hold promise for accelerating translational science.
    - Consideration of such promising approaches will draw from the experiences of existing activities at other federal agencies related to the goals of CAN (e.g., FDA, CDC, AHRQ).
- Discuss the authorities conferred to CAN and identify strategies for effectively using those authorities.
    - Consideration of the CAN authorities will specifically explore the flexible research, or "other transactions", authority and will reference existing efforts in which such authority is currently applied across other federal agencies (e.g., DARPA, DTRA, BARDA).
- Explore promising models for public-private collaborations that could be strengthened or facilitated by activities under CAN.
    - Discuss barriers to such collaborations and identify opportunities and potential solutions for moving past the identified barriers.
    - Discuss the respective roles of multiple sectors, including, e.g., biopharma, biotech, venture capital/private equity, and patient/disease advocacy.
- Identify barriers and potential solutions to facilitate coordination of activities under CAN with the FDA regulatory review process and timelines.

Day One

8:30 a.m. Opening Remarks

Workshop Co-Chairs

CAROLYN COMPTON
President and Chief Executive Officer
Critical Path Institute

LOUIS DEGENNARO
Executive Vice President and Chief Mission Officer
The Leukemia & Lymphoma Society

SESSION I: OVERVIEW OF NIH TRANSLATIONAL SCIENCES AND THE CURES ACCELERATION NETWORK

Session Objectives:

- Provide an overview of the translational science initiatives at NIH.
- Provide an overview and description of the Cures Acceleration Network program goals and authorities.

8:40 a.m. Background and Session Objectives

SUDIP PARIKH, *Session Chair*
Vice President, Health Policy
Managing Director
Centers for Public Health Research & Evaluation
Battelle Health & Life Sciences

8:45 a.m. **Keynote Address: Introduction to NCATS and Overview of Translational Science Initiatives and CAN**

TOM INSEL
Acting Director
National Center for Advancing Translational Sciences
National Institutes of Health

9:05 a.m. **Plenary Discussion: Introduction to CAN Authorities**

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

BARBARA MCGAREY
Deputy Associate General Counsel for Public Health
Office of General Counsel
National Institutes of Health

KATHY HUDSON
Acting Deputy Director, NCATS
Deputy Director for Science, Outreach, and Policy, NIH Office of the Director
National Institutes of Health

9:25 a.m. **Discussion with Speakers and Audience:**

- Relationship of CAN to NCATS, other NIH Institutes/Centers
- Identification of types of activities that are likely to be undertaken under CAN
- Discussion of role of CAN Board

SESSION II: APPROACHES TO ACCELERATING TRANSLATIONAL SCIENCE

Session Objectives:

- Through discussion of case examples and other mechanisms, identify potential approaches that hold promise for accelerating translational science, highlighting approaches that could potentially benefit from the new CAN authorities.
- Discuss and identify attributes of success stories and failures.

9:45 a.m. Background and Session Objectives

BILL CHIN, *Session Chair*
Executive Dean for Research
Harvard Medical School

9:50 a.m. **Opening Plenary: Challenges and Needs in Translational Science – Industry Perspective**

JOSHUA BOGER
Founder, Vertex Pharmaceuticals

10:10 a.m. **Opening Plenary: Challenges and Needs in Translational Science – Academic Perspective**

R. SANDERS WILLIAMS
President
The Gladstone Institutes
University of California, San Francisco

10:30 a.m. BREAK

10:45 a.m. **Brief Presentations: Product Development/Commercialization Efforts**

JAMES BRADNER
Assistant Professor, Harvard Medical School
Instructor in Medicine, Dana-Farber Cancer Institute

STEPHEN SEILER
Founder and Chief Executive Officer
AesRx

LOUIS DEGENNARO
Executive Vice President and Chief Mission Officer
The Leukemia & Lymphoma Society

11:25 a.m. **Discussion with Speakers and Audience:**

- What are the translational science needs?
- What approaches have failed with existing authorities? What can we learn from these failures?
- What approaches/efforts could potentially benefit from new CAN authorities?

12:05 p.m. LUNCH

SESSION III: APPLICATION OF MATCHING AUTHORITY

Session Objectives:

- Explore existing efforts in which the matching authority, or similar match-type requirement, is currently applied across other federal and state agencies.
- Examine benefits and advances that have been achieved through use of this authority.
- Discuss how barriers to application and use of those authorities have been overcome.

12:35 p.m. Background and Session Objectives

NANCY SUNG
Senior Program Officer
Burroughs Wellcome Fund

12:40 p.m. **Series of Brief Presentations: Application of "Matching" or Similar Authority**

MICHAEL WEINGARTEN
Director, SBIR Development Center
National Cancer Institute
National Institutes of Health

KRISTEN DOYLE
General Counsel
Cancer Prevention and Research Institute of Texas

ELLEN FEIGAL
Senior Vice President, Research & Development
California Institute for Regenerative Medicine

1:25 p.m. **Panel Discussion with "Matchers"**

JENS ECKSTEIN
President, SR One
GlaxoSmithKline

MARTIN LEHR
Osage Partners

MICHAEL GUTCH
Managing Director
MedImmune Ventures

Issues for Discussion:

- What are the necessary conditions for the matching program to encourage and successfully de-risk investment decisions on part of matching funders?

2:00 p.m. **Discussion with Speakers and Audience**

Issues for Discussion:

- What are the lessons learned from previous experiences?
- What are the respective roles of the various sectors (e.g., biopharma/biotech, venture capital/private equity, and patient/disease advocacy)?
- What are models for public-private collaborations that could be strengthened or facilitated by the matching authority? What are the barriers and opportunities and potential solutions for moving past those barriers?

2:30 p.m. BREAK

SESSION IV: APPLICATION OF FLEXIBLE RESEARCH AUTHORITY

Session Objectives:

- Explore existing efforts in which the flexible research (or similar) authority is currently applied across other federal agencies.
- Examine benefits and advances that have been achieved through use of these authorities.
- Discuss how barriers to application and use of those authorities have been overcome.

2:50 p.m. Background and Session Objectives

WILLIAM WARREN, *Session Chair*
Vice President, VaxDesign Campus
Sanofi Pasteur

2:55 p.m. **Series of Presentations: Agencies**

SCOTT ULREY
Deputy Director, Contracts Management Office
Defense Advanced Research Projects Agency
Department of Defense

JASON PARAGAS
Special Assistant to the Director
Defense Threat Reduction Agency
Department of Defense

GERALD KOVACS
Director, Division of Chemical, Biological, Radiological, and Nuclear Countermeasures
Biomedical Advanced Research and Development Authority
Office of the Assistant Secretary for Preparedness and Response

PEDER MAARBJERG
Assistant Director for External Communication
Advanced Research Projects Agency – Energy (ARPA-E)
Department of Energy

4:15 p.m. **Panel Discussion with Speakers, Discussants, and Audience: Comparing Flexible Research Authority to Existing NIH Authorities**

Discussants:

DAN WATTENDORF
Program Manager, Defense Sciences Office
Defense Advanced Research Projects Agency

ROBI BLUMENSTEIN
President
CHDI Management

Issues for Discussion:

- How can the other transactions authority (OTA) be most effectively applied in the biomedical/life sciences space?
  - What kind of science/projects should be funded under exercise of OTA? What are the attributes of a promising project or science?
  - How should the research needs be defined and executed?
- What are potential barriers that could impede the successful exercise of the OTA by NCATS/CAN? How can these barriers be overcome?
- What are the conditions for success?
- What are the respective roles of the various sectors (e.g., biopharma/biotech, venture capital/private equity, and patient/disease advocacy)?
- What are models for public-private collaborations that could be strengthened or facilitated by the flexible research authority? What are the barriers and opportunities and potential solutions for moving past those barriers?

5:30 p.m. Adjourn day 1

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OF THE NATIONAL ACADEMIES

Maximizing the Goals of the Cures Acceleration Network to Accelerate the Development of New Drugs and Diagnostics

June 5, 2012

8:20 a.m. Welcome and Introductions

Workshop Co-Chairs

CAROLYN COMPTON
President and Chief Executive Officer
Critical Path Institute

LOUIS DEGENNARO
Executive Vice President and Chief Mission Officer
The Leukemia & Lymphoma Society

SESSION V: SITUATING CAN WITHIN THE DRUG DEVELOPMENT ECOSYSTEM

Session Objectives:

- Identify potential approaches to facilitate coordination of activities under CAN with FDA regulatory science initiatives and activities.
- Discuss existing activities in multiple sectors and address ways to maximize CAN impact in the drug development ecosystem.
- Explore promising models for public-private collaborations that could be strengthened or facilitated by activities under CAN. Discuss barriers to such collaborations and identify opportunities and potential solutions for moving past the identified barriers.

8:30 a.m. Background and Session Objectives

MARGARET ANDERSON, *Session Chair*
Executive Director
FasterCures

8:35 a.m. **FDA Presentations: Intersection of CAN with FDA Regulatory Science Initiatives and Activities**

JESSE GOODMAN
Chief Scientist, Food and Drug Administration

SHAAVHRÉE BUCKMAN-GARNER
Director, Office of Translational Sciences
Center for Drug Evaluation and Research
Food and Drug Administration

9:05 **Roundtable Discussion: Identification and Discussion of Regulatory Science Priorities That Are Important for Drug Development**

Panelists

SHAAVHRÉE BUCKMAN-GARNER
Director, Office of Translational Sciences
Center for Drug Evaluation and Research
Food and Drug Administration

ELIZABETH MANSFIELD
Director, Personalized Medicine, Office of In Vitro Diagnostics
Center for Devices and Radiological Health
Food and Drug Administration

THOMAS KALIL
Deputy Director for Policy
Office of Science and Technology Policy
Executive Office of the President

CAROLYN COMPTON
President and Chief Executive Officer
Critical Path Institute

GARRY NEIL
Corporate Vice President, Science and Technology
Johnson & Johnson

Panel Moderator:

MARGARET ANDERSON
Executive Director
FasterCures

9:45 a.m. **Roundtable Discussion: Role of CAN in Advancing Cross-Sector and Other Collaborative Translational Science Activities**

Panelists

ELLEN SIGAL
Founder and Chair
Friends of Cancer Research

DAVID WHOLLEY
Director, Biomarkers Consortium
Foundation for the NIH

JANE REESE-COULBOURNE
Executive Director
Reagan-Udall Foundation for FDA

FREDA LEWIS-HALL
Chief Medical Officer
Pfizer Inc.

DOUG THROCKMORTON
Deputy Director
Center for Drug Evaluation and Research

Panel Moderator:

MYRL WEINBERG
President
National Health Council

Issues for Discussion:

- Coordination of efforts across funding agencies/sources (e.g., HHS, DoD, NGOs, industry) and with other established partners in translational research activities (e.g., FNIH, Reagan-Udall Foundation, C-Path), and reduction of duplication.
- Implications of CAN for academic translational science – career paths, funding opportunities, etc.

10:30 a.m. BREAK

SESSION VI: CONCLUDING PANEL DISCUSSION: PRINCIPLES FOR DEPLOYMENT OF CAN AUTHORITIES

Session Objectives:

- Discuss key themes from the workshop.
- Based on workshop presentations and discussions, identify principles for deployment of CAN authorities.

10:45 a.m. Closing Discussion with Panelists and Audience: Led by Workshop Co-Chair(s)

CAROLYN COMPTON
President and Chief Executive Officer
Critical Path Institute

LOUIS DEGENNARO
Executive Vice President and Chief Mission Officer
The Leukemia & Lymphoma Society

10:50 a.m. **Presentation of Key Themes/Suggested Paths from Workshop Session Chairs**

SUDIP PARIKH, *Session I Chair*
Vice President, Health Policy
Battelle Memorial Institute

BILL CHIN, *Session II Chair*
Executive Dean for Research
Harvard Medical School

NANCY SUNG, *Session III Chair*
Senior Program Officer
Burroughs Wellcome Fund

DAN WATTENDORF, *Session IV Rapporteur*
Program Manager, Defense Sciences Office
Defense Advanced Research Projects Agency

MARGARET ANDERSON, *Session V Chair*
Executive Director
FasterCures

11:40 a.m. **Reflecting on Potential Approaches to Maximize the Goals of CAN: Panel Discussion with Session Chairs, Panelists, and Audience**

Discussants

JOSHUA BOGER
Founder, Vertex Pharmaceuticals

KATHY HUDSON
Acting Deputy Director, NCATS
Deputy Director for Science, Outreach, and Policy, NIH Office of the Director
National Institutes of Health

CAROL MIMURA
Assistant Vice Chancellor for Intellectual Property & Industry Research Alliances
University of California, Berkeley

ROBERT O'NEILL
Senior Statistical Advisor
Center for Drug Evaluation and Research
Food and Drug Administration

12:20 p.m. **Closing Observations from NCATS**

KATHY HUDSON
Acting Deputy Director, NCATS
Deputy Director for Science, Outreach, and Policy, NIH Office of the Director
National Institutes of Health

12:30 p.m. ADJOURN