ACD WORKING GROUP ACTIVITIES:

NATIONAL CENTER FOR ADVANCING TRANSLATIONAL SCIENCES

NIH Advisory Committee to the NIH Director

Maria Freire, Ph.D., Chair

ACD-NCATS: Membership

MARIA FREIRE, PhD *(Chair)*
President, Albert and Mary Lasker Foundation

JULIAN ADAMS, PhD
President of Research and Development, Infinity Pharamceuticals, Inc.

LEE BABISS, PhD
Executive Vice President of Global Laboratory Services, PPD, Inc

BROOK BYERS, MBA
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Executive Dean for Research, Harvard Medical School

SUSAN DESMOND-HELLMANN, MD, MPH
Chancellor, University of California-San Francisco

DAVID GINSBURG, MD
James V. Neel Distinguished University Professor of Internal Medicine and Human Genetics, University of Michigan

VICTORIA HALE, PhD
Chief Executive Officer, Medicines360

HELEN HOBBS, MD
Director of the McDermott Center, University of Texas at Southwestern

ROBERT LANGER, ScD
David H. Koch Institute Professor, Massachusetts Institute of Technology

STELIOS PAPADOPOULOS, PhD
Director and Chairman of the Board, Exelixis

MARY PENDERGAST, JD
President, Pendergast Consulting

MONCEF SLAOUI, PhD
Chairman of Research and Development, GlaxoSmithKline

MARC TESSIER-LAVIGNE, PhD
President, Rockefeller University

DAVID VALLE, MD
Professor and Director of the Institute of Genetic Medicine, Johns Hopkins University School of Medicine

ACD-NCATS: Charge

1. Identify areas in which NIH can contribute to streamlining therapeutic and diagnostic development nationally and globally;

2. Recommend possible ways in which NCATS can maximally tap the strengths of extant programs, the authorities under the Cures Acceleration Network (CAN), and the vast capabilities of partners;

3. Propose new models for how NCATS could build partnerships with external entities, including biotechnology and pharmaceutical companies, to achieve its mission of accelerating translational research;

ACD-NCATS: Charge *(cont.)*

4. **Recommend the subset of scientific and technical challenges along the drug discovery pipeline that NCATS should address;**

5. **Recommend potential areas of translational research that fall outside the drug development process that NCATS should address; and**

6. **Suggest a framework for metrics and timelines by which success of NCATS can be measured.**

ACD-NCATS: Process and Deliverables

- **Hold deliberations at least on a quarterly basis, with the intent of meeting at least three times before October 1, 2011;**
- **The Chair of the Working Group will present Working Group findings to the full ACD (preliminary findings presented at the ACD meeting on June 9-10, 2011); and,**
- **Work will be complete when the Advisory Council for NCATS is formally in place (expected by October 2011).**

ACD-NCATS: Meetings

- ***February 4, 2011*** **– receipt of charge** ***(held in conjunction with the NIH Institute and Center Directors Working Group on NCATS)***
- ***May 24, 2011*** **– existing hurdles and areas amenable to reengineering**
- ***July 15, 2011*** **– project management and cross-sector partnerships (included two expert panels)**
- ***September 14, 2011*** **– review of findings**

ACD-NCATS: Expert Panelists

Jeffrey Bluestone, Ph.D.
Executive Vice Chancellor and Provost
University of California, San Francisco

Jennifer Cook, M.S., M.B.A.
Senior Vice President, Genentech Immunology and Ophthalmology
Genentech

John McKew, Ph.D.
Chief, Therapeutic Development Branch; and
Director of Chemistry
NIH Center for Translational Therapeutics
National Institutes of Health

Jack D. Newman, Ph.D.
Co-Founder and Chief Scientific Officer
Amyris

Torben Straight Nissen, M.Sc., Ph.D.
Managing Director; and
Head of Portfolio Management and Development Strategy
Pfizer Inc.

Beth Seidenberg, M.D.
Partner
Kleiner Perkins Caufield & Byers

Deepak Srivastava, M.D.
The Younger Family Director, Gladstone Institute of Cardiovascular Disease;
Professor, Departments of Pediatrics and Biochemistry & Biophysics; and
Wilma and Adeline Pirag Distinguished Professor in Pediatric Developmental Cardiology
University of California, San Francisco

Lewis T. "Rusty" Williams, M.D., Ph.D.
Executive Chairman, Founder, President, and CEO
FivePrime

Paul G. Yock, M.D.
Martha Meier Weiland Professor of Medicine; and
Director, Biodesign
Stanford University

FINDINGS

Revolutionize Translation

NCATS can be a leader in reengineering the translational process by identifying and overcoming hurdles that decrease the probability of success.

Revolutionize the Process of Translation

Critical need to catalyze, enable, and implement ground-breaking advances in translational sciences.

NIH possess rich expertise and has significantly advanced our understanding of human biology.

By complementing existing efforts at NIH and across sectors, NCATS can fill gaps in our understanding of the translational process.

Goals for NCATS

- ***Catalyze translation by promoting innovative research***
  - **Support and enable high-risk, high-reward projects that experiment with novel and innovative strategies.**
  - **Serve as an incubator space for innovative science that is too risky or too early in development for commercial investment.**
  - **Recruit diverse expertise to address barriers and gaps.**

Goals for NCATS *(cont.)*

- ***Galvanize and support new partnerships***
  - **Utilize convening power to stimulate communication and promote partnerships across sectors to address barriers and provide solutions.**
  - **Promote cross-sector partnerships by leveraging funds and providing access to tools, technologies, and innovative platforms.**

Goals for NCATS *(cont.)*

- ***Support and augment the discipline of regulatory science and its application***
  - **Collaborate with FDA to design/undertake studies informing regulatory approval processes.**
  - **Help develop regulatory pathways incorporating innovative designs.**
  - **Strengthen communication between regulatory agencies and researchers across sectors to develop a more nimble regulatory process.**

Goals for NCATS *(cont.)*

- ***Expand the precompetitive space***
  - **Promote and facilitate open exchange of information and lessons learned.**
  - **Encourage grantees to submit data regarding failures.**
  - **Encourage the collection of information on failures, and analyze data to capture lessons learned.**
  - **Establish an open-access repository for collecting information.**

Goals for NCATS *(cont.)*

- ***Harness the power of the CTSA Program***
  - **Capitalize on the capabilities of the CTSA program.**
  - **Afford individual CTSAs flexibility to cultivate unique strengths.**
  - **Provide incentives for forming a strong national CTSA consortium.**
  - **Develop programs to train clinicians in translational science.**

Goals for NCATS *(cont.)*

- ***Transformation through training***
  - **Catalyze educational programs in translational sciences, especially in under-represented fields.**
  - **Promote novel training mechanisms such as a drug development apprenticeship for early-stage investigators.**
  - **Provide incentives for physician scientists to seek cross-training in human biology and new drug, diagnostic, and device discovery; explore cross-training of physicians and scientists between and among industry, academia, and government labs.**

Goals for NCATS *(cont.)*

- ***Streamline Administrative Processes***
  - **Identify and overcome roadblocks that hinder the ability to rapidly and effectively fund, manage, and terminate projects.**
  - **Establish clear pathways for developing agreements between NIH and other sectors to allow for prompt turnaround and timely funding.**

FINDINGS

Points to Consider

NCATS should focus its efforts on areas of research that broadly and profoundly impact the entire translational discipline.

Points to Consider

- **NCATS should not duplicate translational research efforts existing in and/or funded by NIH Institutes and Centers or already underway throughout industry.**
- **NCATS should leverage its infrastructure to form strategic collaborations with diverse sectors to minimize scientific and administrative redundancy.**
- **Projects should be supported only until they attract commercial investment and not across the entire pipeline.**
- **NCATS leadership will be responsible for prioritizing goals and activities.**

Areas Amenable to Reengineering - Examples

- Target Validation
- Pharmacology
- Imaging
- Devices and Diagnostics
- Chemical Space
- Toxicology
- Biomarkers
- New Uses for Established Compounds
- Implementation Science

FINDINGS

Ensuring Success

NCATS leadership – its staff and its governing body – will play a defining role in the new Center's success.

Qualities of Effective Leadership, Governance, and Staff

- ***NCATS Director***
  - **Has a unique blend of expertise and experience that transcends a singe field or discipline.**
  - **Has experience in both academia and the private sector.**
  - **Can identify and overcome hurdles.**
  - **Demonstrates political sensitivity when working with diverse stakeholders and constituencies.**
  - **Can convene multiple partners working towards common goals.**
  - **A visionary willing to engage in "disruptive innovation."**

Qualities of Effective Leadership, Governance, and Staff *(cont.)*

- ***Research Staff***
  - **Innovative thinkers, technically sophisticated, and able to work independently and collaboratively in teams.**
  - **Primary responsibility should be focused on team projects and not daily administration of NCATS.**
- ***Advisory Board***
  - **Composed of stakeholder representatives with expertise in sectors of relevance to the NCATS mission.**

CONCLUDING REMARKS

Moving Forward

With visionary leadership, Agency support, and in collaboration with the NIH Institutes and Centers, the FDA, and industry, NCATS can transform the way we develop new medicines, diagnostics, and devices.

QUESTIONS AND DISCUSSION

NIH Advisory Committee to the NIH Director

Maria Freire, Ph.D., Chair