

**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

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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 whereby 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 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 on a quarterly basis, or more often if agreed upon by the Working Group, with the intent of meeting at a minimum three times before October 1, 2011;**
- **The Chair of the Working Group will present preliminary findings to the full ACD;**
- **Work will be complete when the Advisory Council for NCATS is formally in place (expected by October, 2011).**

ACD-NCATS: Process and Deliverables (cont.)

- **First meeting held on February 4, 2011 in conjunction with the NIH Institute and Center Directors' Working Group on NCATS;**
- **Second meeting held on May 24, 2011;**
- **Third meeting will be held on July 15, 2011.**

Discussion Highlights

Framing the Issues

CHARGE: Streamlining Diagnostic and Therapeutic Development

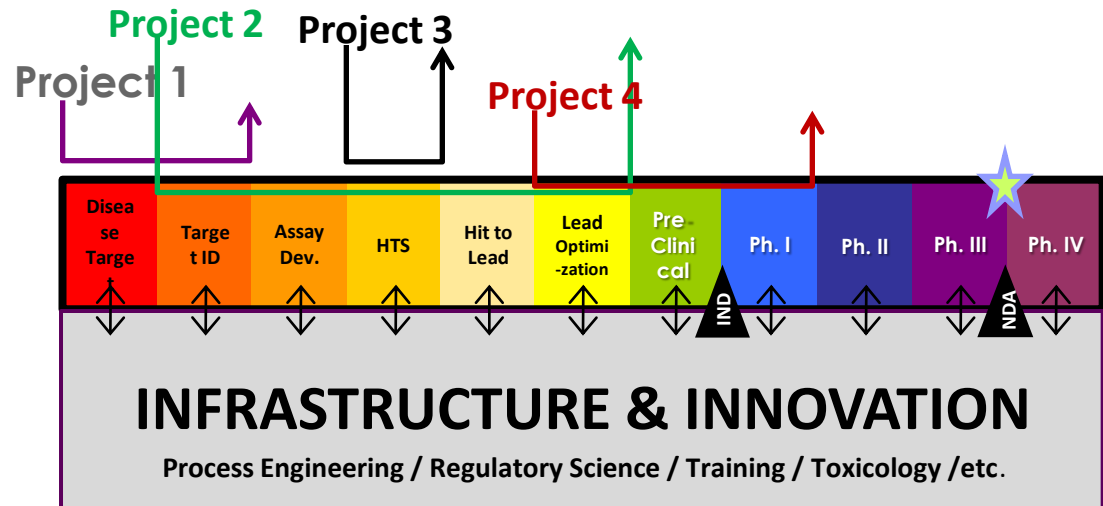
- **Innovate by supporting and enabling high-risk, high-reward projects;**
- **Address barriers and gaps in translational science by bringing together diverse expertise;**
- **Focus on areas of greatest need;**
- **Discover novel and innovative strategies for improving and accelerating the process;**
- **Promote and facilitate an open exchange of information to expand the precompetitive space;**

CHARGE: Streamlining Diagnostic and Therapeutic Development *(cont.)*

- **Open the lines of communication between FDA and researchers to address regulatory hurdles;**
- **Elevate, promote, and provide education and training in the translational sciences;**
- **Provide a neutral forum for sectors to convene and discuss challenges;**
- **Collect/analyze data and lessons learned and promote sharing of failures.**

CHARGE: Streamlining Diagnostic and Therapeutic Development *(cont.)*

- Any new effort should NOT:
- Recapitulate therapeutics development efforts within NIH or the private sector;
- Overextend capacity by funding projects across the entire pipeline;
- Unnecessarily separate intra-/extramural research.



△ Application to FDA
★ FDA Approval

CHARGE: Scientific and Technical Challenges Along the Pipeline

- **Systematic process for target validation;**
- **Predictive models of drug toxicity (e.g., cell-based organelles, IPS cells);**
- **Understanding of systems pharmacology;**
- **Effective biomarkers and accurate disease phenotyping;**
- **Non-invasive imaging for understanding of drug response, metabolism, and distribution.**

CHARGE: Scientific and Technical Challenges Along the Pipeline *(cont.)*

- **Understanding of chemical space:**
 - **Clean/green chemistry (no solvent);**
 - **Independent of carbon (e.g., boron chemistry);**
 - **Combinatorial chemistry.**
- **Enhance relationships with regulatory agencies.**

Additional Discussion

General Considerations

ADDITIONAL DISCUSSION:

Foci for NCATS

- **Members agreed upon several critical areas of research that NCATS should address:**
 - **Research that could be done in greater capacity and more efficiently;**
 - **Research that is not being conducted elsewhere but is important to pursue;**
 - **Research that would enable and enhance the capabilities of others to conduct research.**

ADDITIONAL DISCUSSION:

NCATS Leadership

- **NCATS leadership will need to possess:**
 - **Intimate awareness of private sector mechanics;**
 - **Knowledge of or background in academics;**
 - **Diplomatic skills and capabilities;**
 - **An eagerness to “change the world.”**

NEXT STEPS: Future Meeting Discussions

- **NCATS priority setting and pipeline challenges**
- **Models for partnerships with external entities**
- **Areas of translational research that fall outside the drug development process that NCATS should address**
- **NCATS communication strategies**

QUESTIONS AND DISCUSSION

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