ACD WORKING GROUP ACTIVITIES:
NATIONAL CENTER FOR ADVANCING TRANSLATIONAL SCIENCES
ACD-NCATS: Membership

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

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

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MONCEF SLAOUI, PhD
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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 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;
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.
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.
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.
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
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