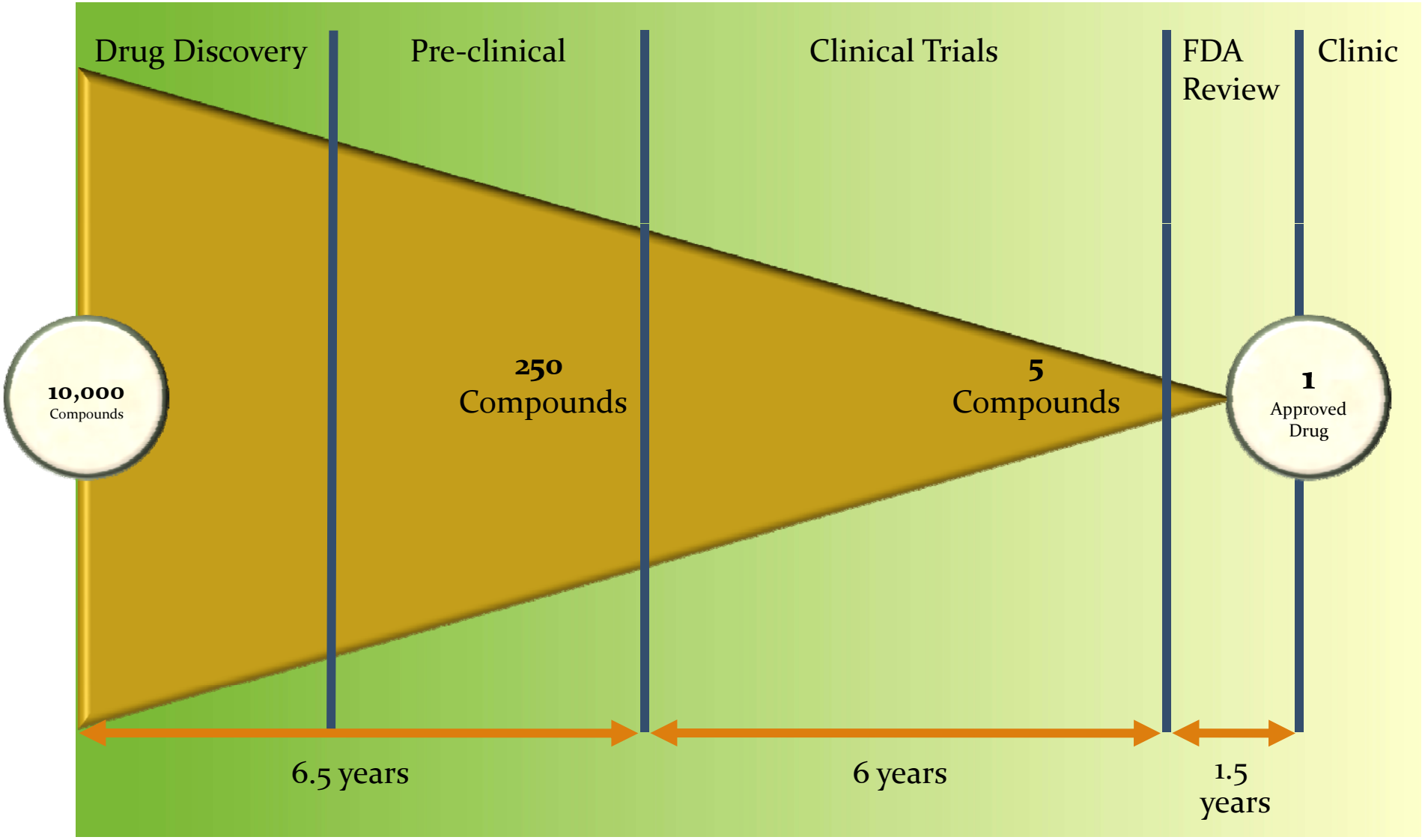
Update on the National Center for Advancing Translational Sciences (NCATS)

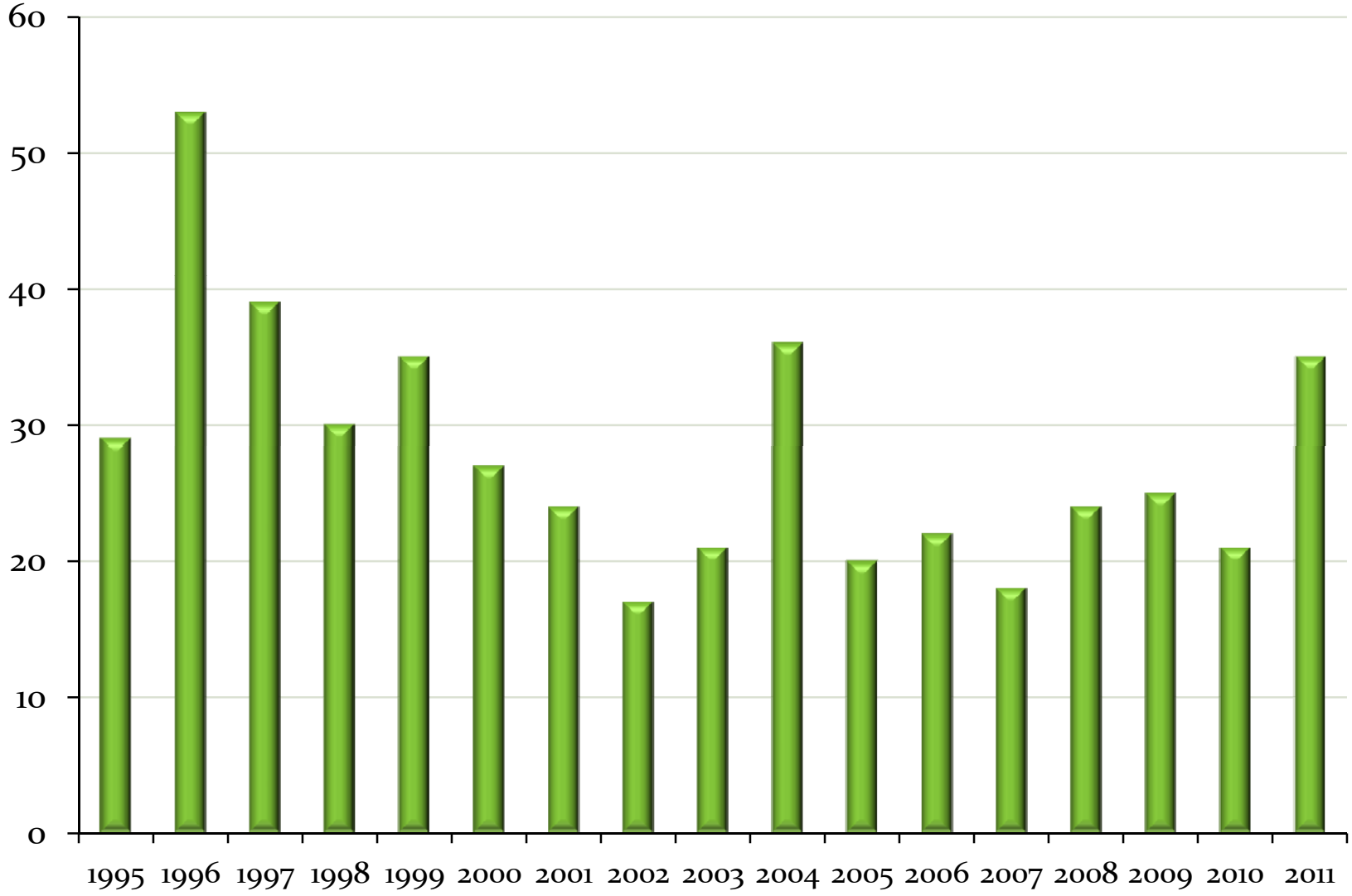
Kathy Hudson, Ph.D.
Deputy Director for Science, Outreach, and Policy, NIH

December 8, 2011

The Problem: Development of New Therapeutics is Slow, Expensive, and Failure-Prone

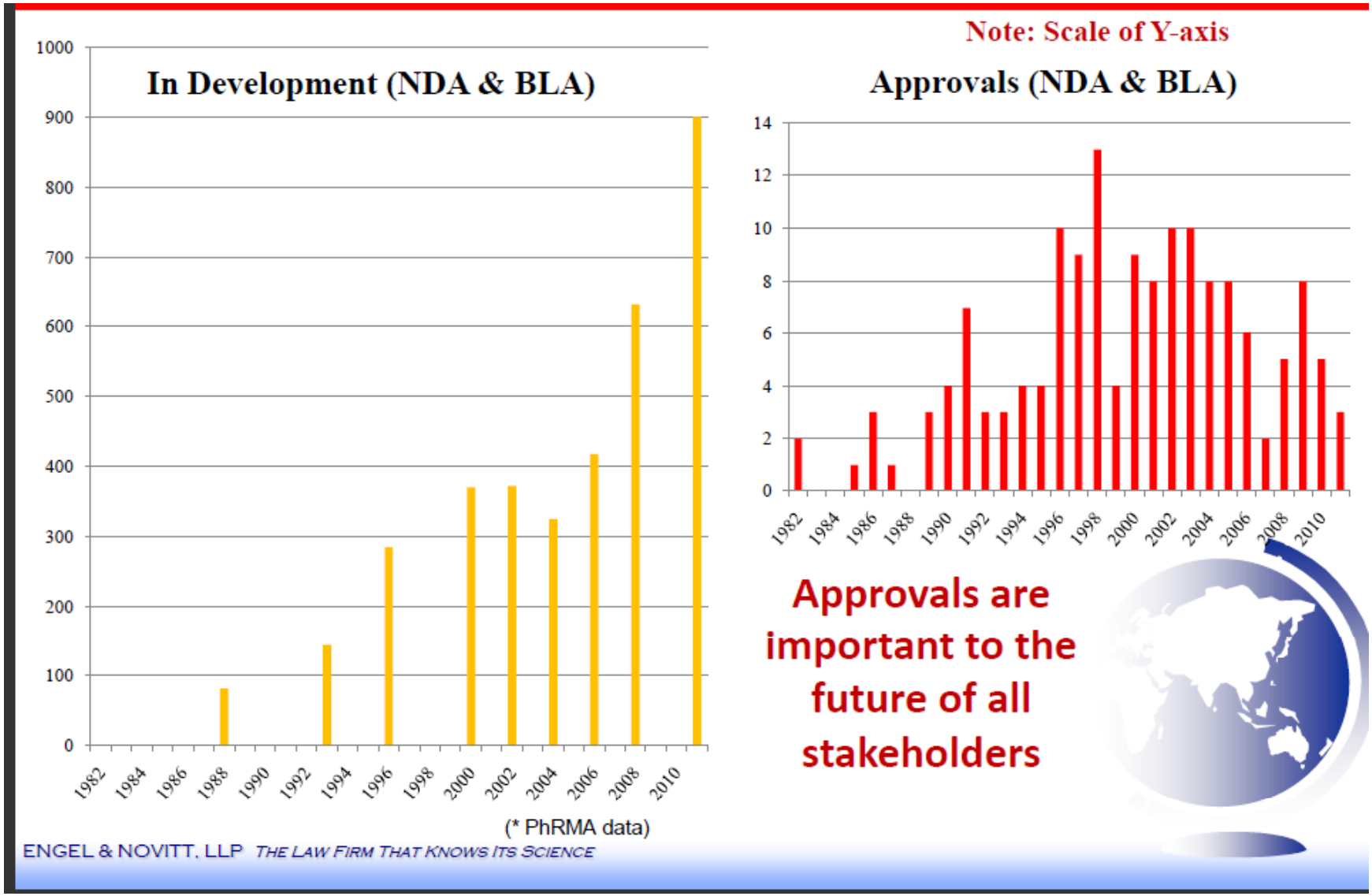


New Medicines in Your Medicine Cabinet: New Molecular Entities Entering Marketplace

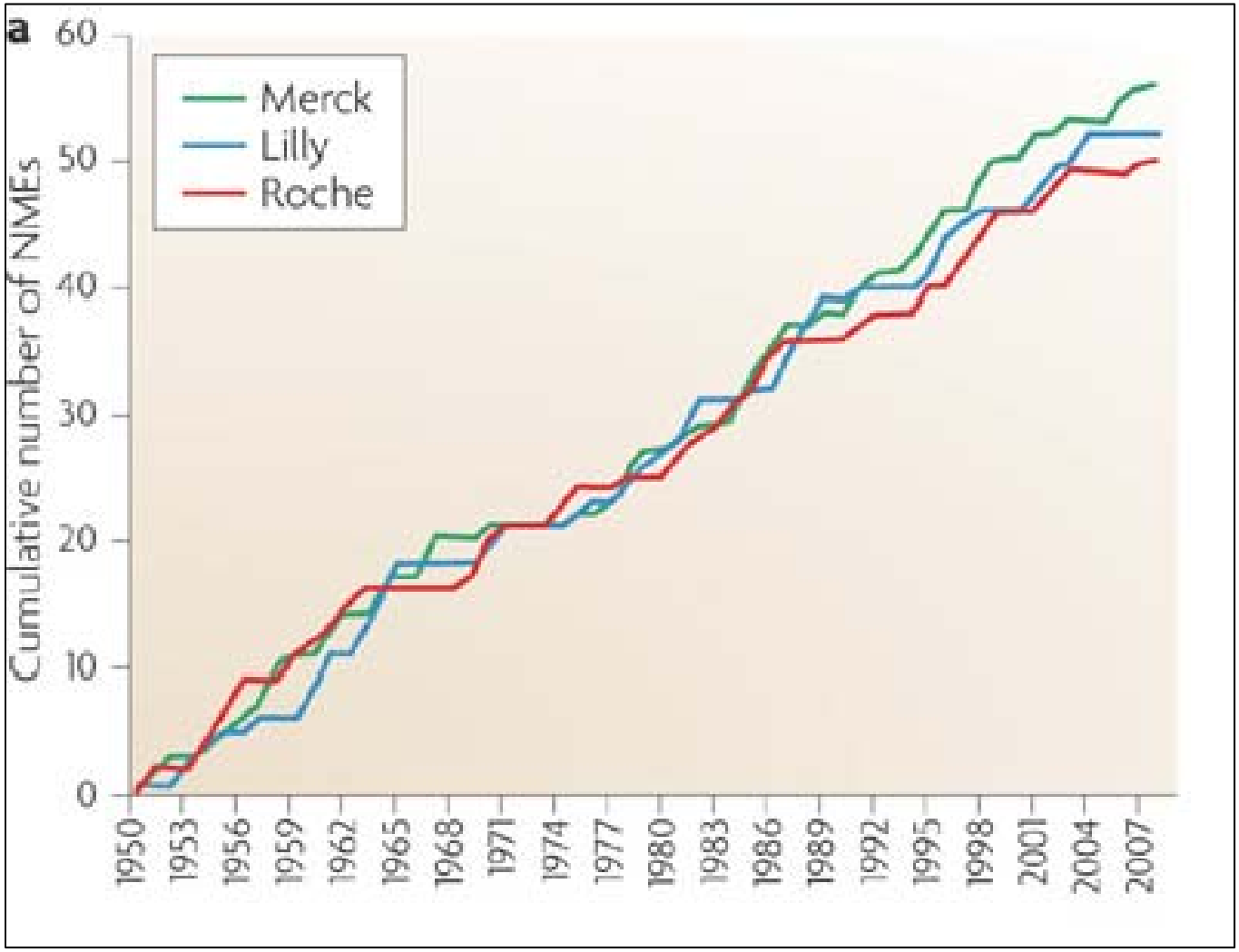


Source: FDA

Recombinant Biotech Medicines

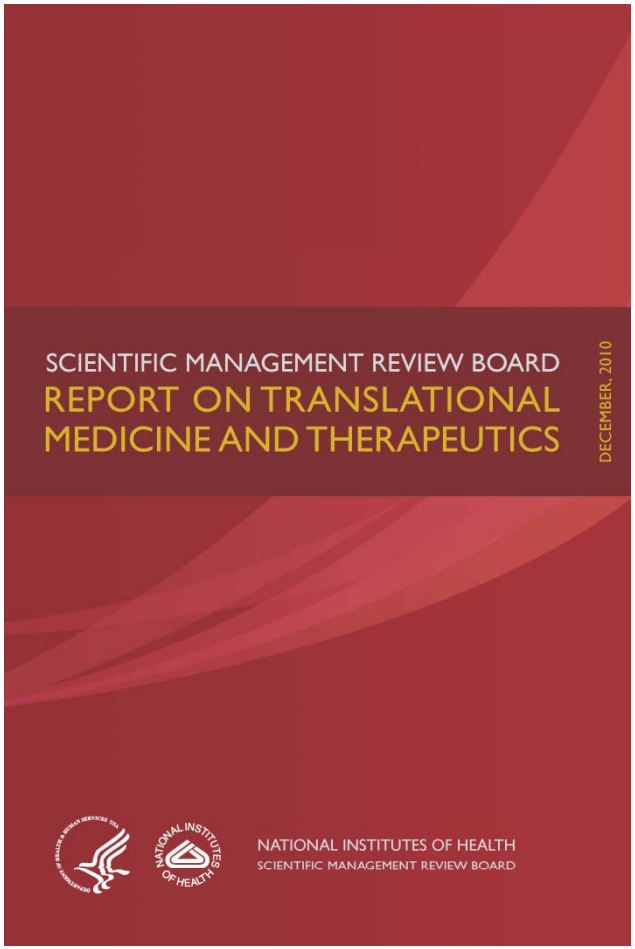


Dynamics of Drug Innovation



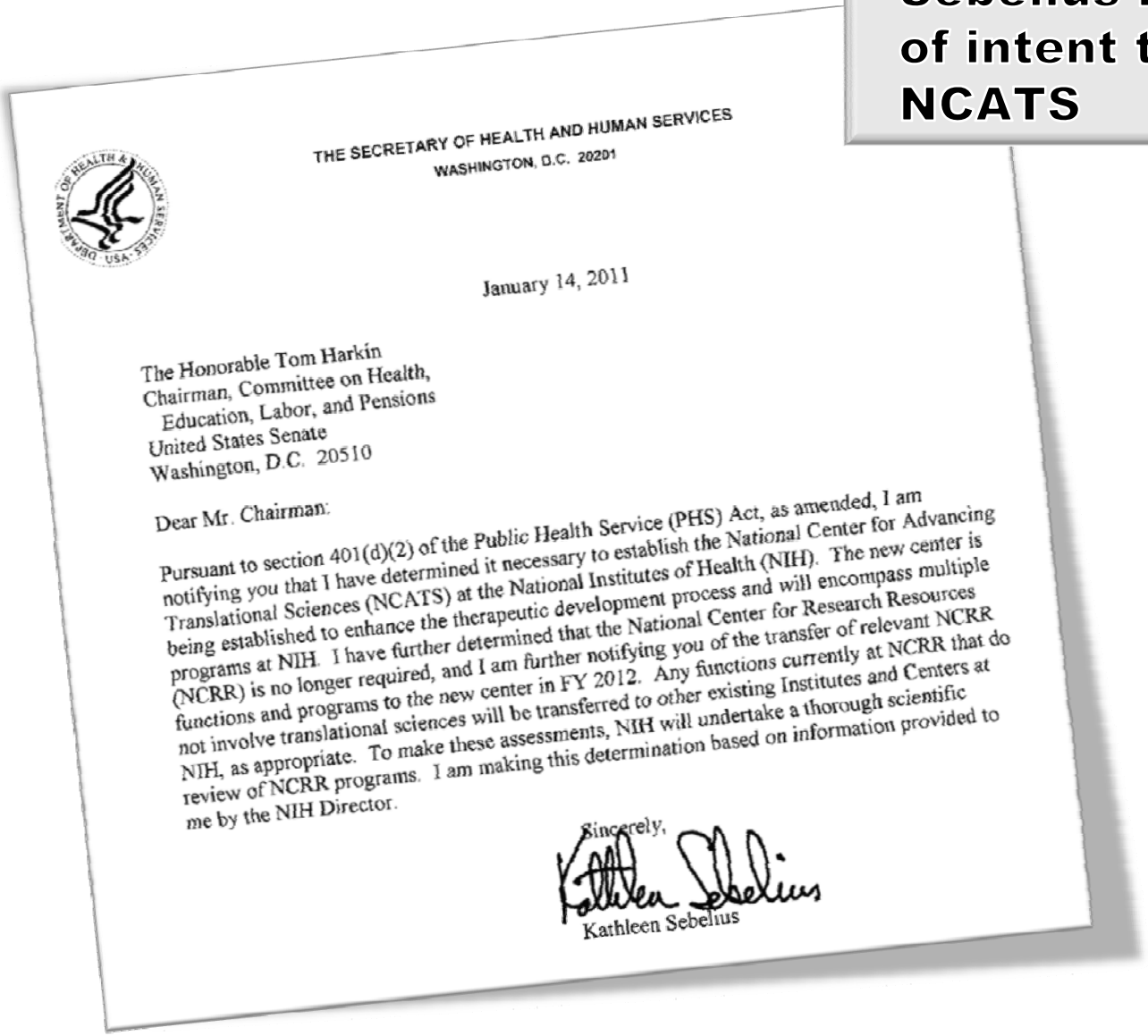
Nature Reviews Drug Discovery 8, 959-968, 2009

Scientific Management Review Board Recommendations



- May 2010
  - SMRB asked to determine how NIH could better support translational and therapeutic sciences.
- December 2010
  - SMRB recommends that a new translational medicine and therapeutics center be created.
  - SMRB also recommends NIH undertake a more extensive and detailed analysis through a transparent process to evaluate the new center's impact.

January 2011: HHS Secretary Kathleen Sebelius notified Congress of intent to establish NCATS



THE SECRETARY OF HEALTH AND HUMAN SERVICES
WASHINGTON, D.C. 20201

January 14, 2011

The Honorable Tom Harkin
Chairman, Committee on Health,
Education, Labor, and Pensions
United States Senate
Washington, D.C. 20510

Dear Mr. Chairman:

Pursuant to section 401(d)(2) of the Public Health Service (PHS) Act, as amended, I am notifying you that I have determined it necessary to establish the National Center for Advancing Translational Sciences (NCATS) at the National Institutes of Health (NIH). The new center is being established to enhance the therapeutic development process and will encompass multiple programs at NIH. I have further determined that the National Center for Research Resources (NCRR) is no longer required, and I am further notifying you of the transfer of relevant NCRR functions and programs to the new center in FY 2012. Any functions currently at NCRR that do not involve translational sciences will be transferred to other existing Institutes and Centers at NIH, as appropriate. To make these assessments, NIH will undertake a thorough scientific review of NCRR programs. I am making this determination based on information provided to me by the NIH Director.

Sincerely,

Kathleen Sebelius

Kathleen Sebelius

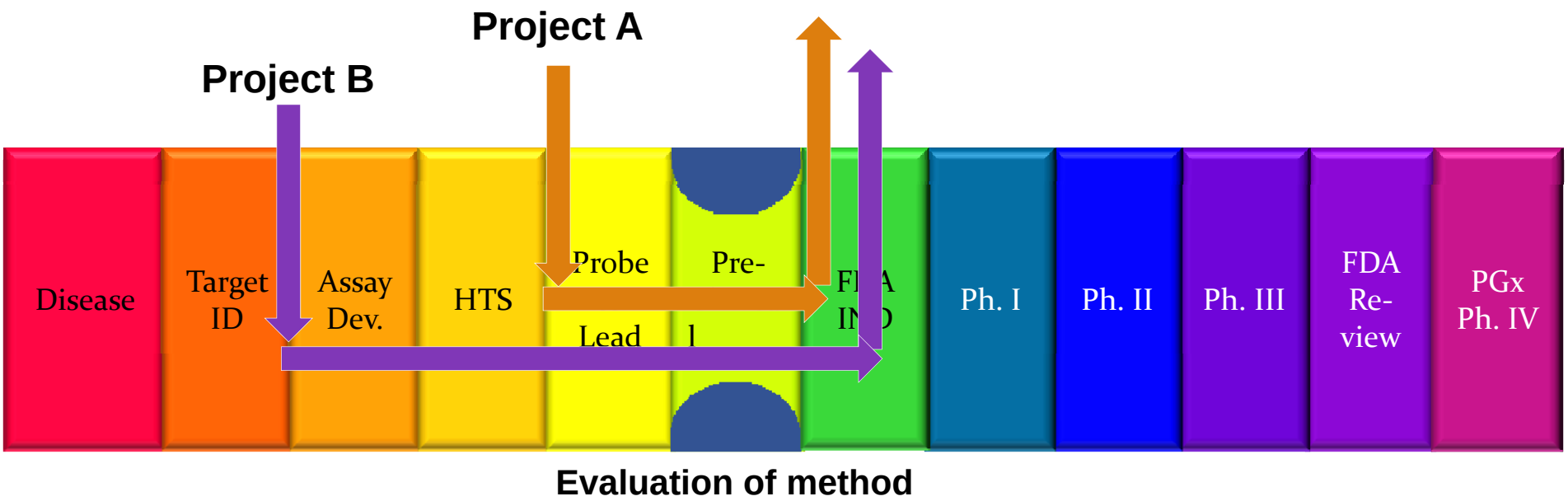
NCATS: Pursuing Opportunities for Disruptive Innovation

Mission:

To catalyze the generation of innovative methods and technologies that will enhance the development, testing, and implementation of diagnostics and therapeutics across a wide range of human diseases and conditions

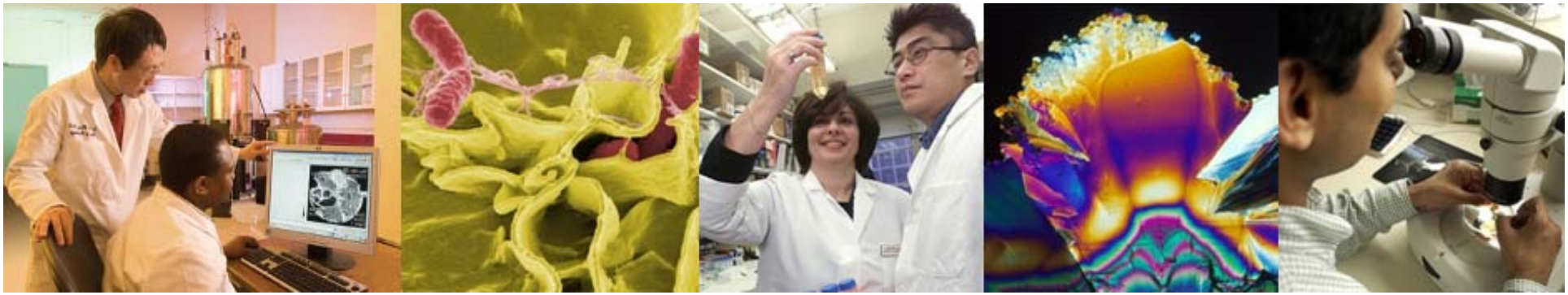
NCATS
Advancing the Science of Translation

- Study the steps in the diagnostics & therapeutics development pipelines and identify bottlenecks amenable to re-engineering
- Experiment with innovative methods to streamline the process
- Evaluate novel methods with compelling therapeutic projects



NCATS will:

- Facilitate – not duplicate – the translational research activities supported and conducted by the ICs
- Complement – not compete with – the private sector
- Reinforce – not reduce – NIH's commitment to basic science research



NCATS Research Programs

- Components of Molecular Libraries Program
- Therapeutics for Rare and Neglected Diseases
- Office of Rare Diseases Research
- Rapid Access to Interventional Development
- Clinical and Translational Science Awards
- FDA-NIH Regulatory Science
- Cures Acceleration Network

Cures Acceleration Network (CAN)

- Established by the Affordable Care Act
- Included in the Senate Report for FY12 at $20M
- CAN will advance the development of "high need cures" and reduce barriers between research discovery and clinical trials
- Funding flexibilities:
  - Large Grant Awards: Up to $15M per award per fiscal year
  - Partnership Awards: $1 for every $3 from NIH
  - Flexible Research Awards: DARPA-like authority

The ACD-NCATS Working Group

Provide high-level advice on the best ways that NCATS can speed the entire effort of getting effective medicines to patients.

- **Maria C. Freire, Ph.D. *(Chair)***
  Albert and Mary Lasker Foundation
- **Julian Adams, PhD**
  Infinity Pharmaceuticals, Inc.
- **Lee E. Babiss, PhD**
  Global Laboratory Services PPD, Inc.
- **Brook Byers, MBA**
  Kleiner Perkins Caufield & Byers
- **William W. Chin, MD**
  Harvard Medical School
- **Susan Desmond-Hellmann, MD**
  UCSF
- **David Ginsburg, MD**
  University of Michigan
- **Victoria Hale, PhD**
  Medicines360
- **Helen H. Hobbs, MD**
  University of Texas at Southwestern
- **Robert S. Langer, ScD**
  Massachusetts Institute of Technology
- **Stelios Papadopoulos, PhD**
  Exelixis
- **Mary K. Pendergast, JD**
  Pendergast Consulting
- **Moncef Slaoui, PhD**
  GlaxoSmithKline
- **Marc Tessier-Lavigne, PhD**
  Genentech, Inc.
- **David L. Valle, MD**
  Johns Hopkins

ACD-NCATS Report Findings
NCATS Can:

- Catalyze translation by promoting innovative research
- Galvanize and support new partnerships
- Support and augment the discipline of regulatory science and its application
- Expand the precompetitive space
- Harness the power of the CTSA Program
- Transformation through training
- Streamline Administrative Processes

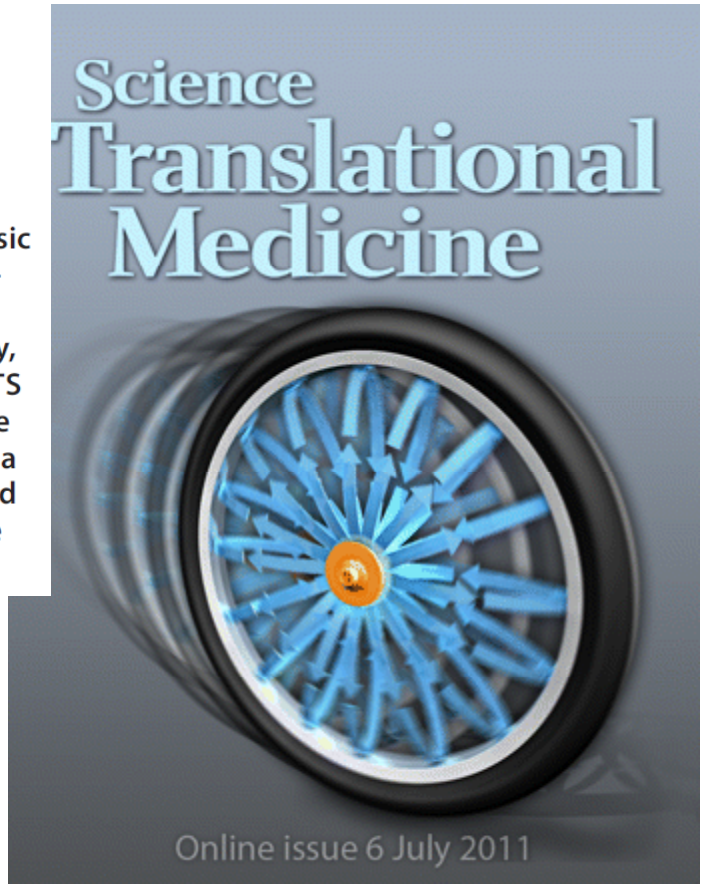
Pursuing Opportunities for Disruptive Translational Innovation

POLICY www.ScienceTranslationalMedicine.org 6 July 2011 Vol 3 Issue 90 90

Reengineering Translational Science: The Time Is Right

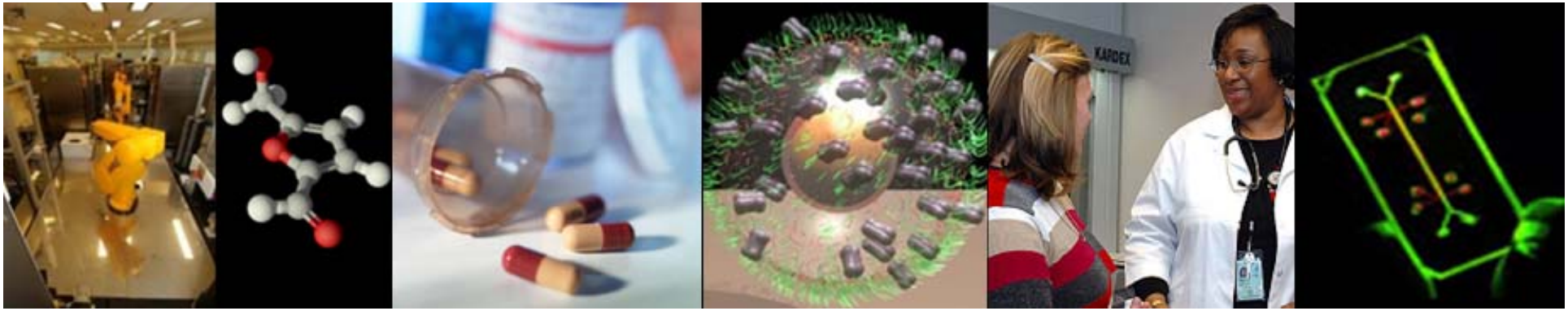
Francis S. Collins

Despite dramatic advances in the molecular pathogenesis of disease, translation of basic biomedical research into safe and effective clinical applications remains a slow, expensive, and failure-prone endeavor. To pursue opportunities for disruptive translational innovation, the U.S. National Institutes of Health (NIH) intends to establish a new entity, the National Center for Advancing Translational Sciences (NCATS). The mission of NCATS is to catalyze the generation of innovative methods and technologies that will enhance the development, testing, and implementation of diagnostics and therapeutics across a wide range of diseases and conditions. The new center's activities will complement, and not compete with, translational research being carried out at NIH and elsewhere in the public and private sectors.



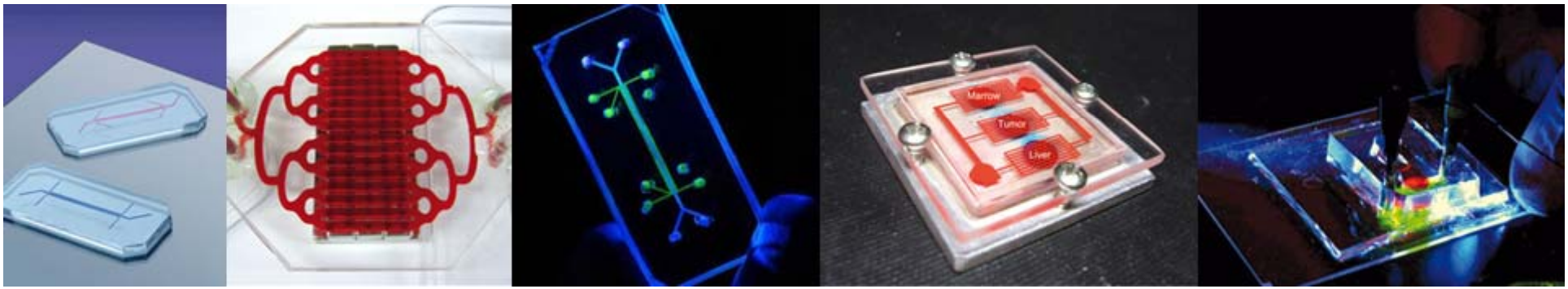
Examples of Translational Challenges

- Therapeutic target validation
- Chemistry
- Virtual drug design
- Preclinical toxicology
- Biomarkers
- Efficacy testing
- Phase zero clinical trials
- Rescuing and repurposing
- Clinical trial design
- Post-marketing research



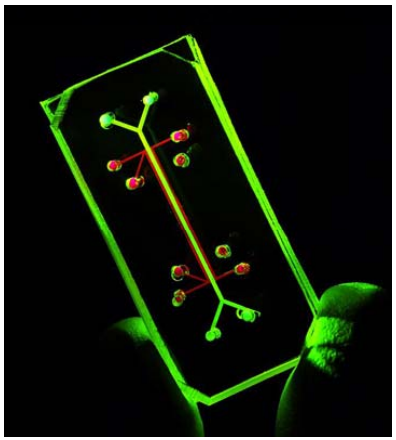
NIH-DARPA-FDA Collaboration: Better Ways to Predict Drug Safety and efficacy

- Part of President's "Lab to Market" initiatives
- Goal: Develop chip to screen for safe, effective drugs
  - Liver, heart, lung, other cell types
  - Designed for multiple different readouts
- NIH, DARPA to commit ~$70 million each over 5 years; NIH and DARPA funded scientists will work very closely
- FDA to offer guidance
- RFA published in mid-November; applications due January 26th
  - Seeking best ideas in engineering, biology, toxicology



NIH-DARPA-FDA Collaboration: Better Ways to Predict Drug Safety and efficacy

- NIH Funding Opportunities: RFAs
  - Integrated Microphysiological Systems for Drug Efficacy and Toxicity Testing in Human Health and Disease (UH2/UH3)
  - Stem/Progenitor Cell-Derived Human Micro-organs and –tissues (U18)
- Ten microphysiological systems on an integrated platform
- Must replicate known responses to drugs and toxins
- Must allow sustained analysis – up to 4 weeks – with multiple read-outs
- Should be extendable to include mimics of pathology
  - Stem/progenitor cell differentiation for diverse lineages
  - Genetic diversity



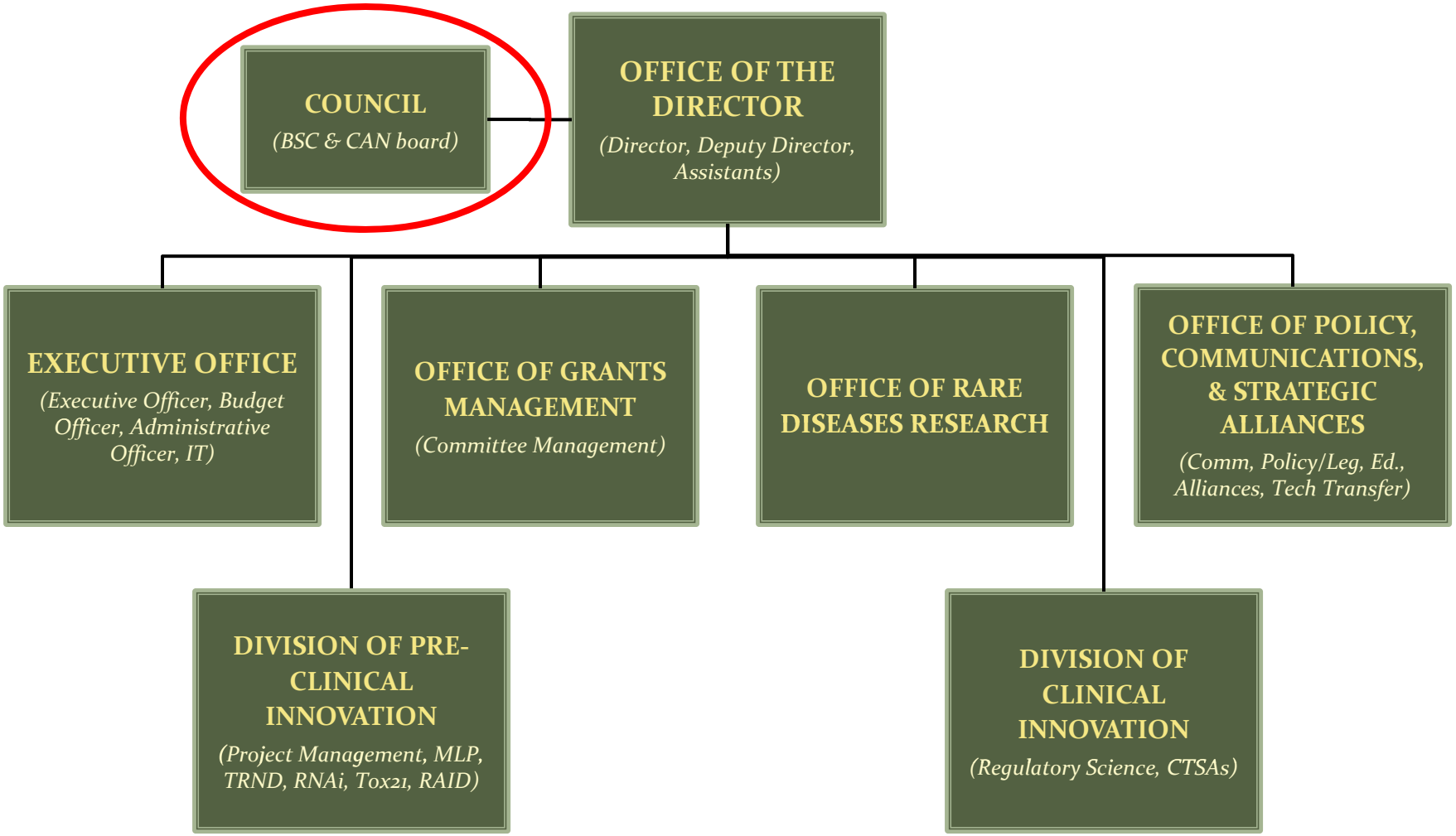
NIH-pharma Collaboration: Rescue and Repurposing

- Match compounds from pharma's "virtual medicine cabinet" with innovative ideas for new indications from NIH extramural scientists
- NIH provides: funding, RFA, review
- Pharma provides: compounds and pertinent data
- NIH-Industry working group – more partners following successful pilot?

NIH-pharma Collaboration: Rescue and Repurposing

- Pilot: 4-8, 2 year projects
- Request for applications and review process:
  - Pre-application (X02) based on limited information on the compounds; peer review
  - Successful applicants get more data on the compounds – submit full application; peer review and Council review
- MOU between NIH and pharma partner
- Model CDA and template agreement would be available

NCATS Organization



CAN Board (and NCATS Council and BSC)

Voting members	
Venture Capitalist	4 Individuals
Disease Advocate	8 Individuals
Basic research	
Medicine	
Biopharmaceuticals	
Discovery and delivery of medical products	
Bioinformatics and gene therapy	
Medical instrumentation	
Regulatory review	
Not specified	5 Individuals

Non-Voting members
National Institutes of Health
Food and Drug Administration
Office of the Assistant Secretary of Defense for Health Affairs
National Science Foundation
Veteran's Health Administration

"My administration is announcing a new center that will help companies reduce the time and cost of developing lifesaving drugs."

President Obama
Signing of America Invents Act
Thomas Jefferson High School
September 16, 2011

NCATS Appropriation for FY2012?