

U.S. Partnerships in Translational Medicine

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Consortium

Foundation for the NIH



24th Annual
EuroMeeting
26-28 March 2012
Copenhagen, Denmark



Foundation for NIH Overview

- Established by Congress in 1990; incorporated in 1996
- Supports the NIH mission
- 501 (c)(3) non-profit organization
 - Raised over \$560M since 1996
 - 50+ projects
- Non-governmental
 - Directly solicits contributions
 - Flexible donor relationships
 - Creates open, inclusive, objective governance mechanisms
 - Timely, effective grants/contracts/project management

Convergence of multiple factors has led to the emergence of public-private partnerships in translational medicine

Escalating complexity of biomedical science and technologies



Declining productivity in biopharma R&D → “externalization” of research



Decline in government health research budgets → funding gap



Increased Need for Public- Private Partnerships



Regulatory challenges → increasing complexity, limited budgets



Emergence of viable collaborative models → e.g., SNP Consortium, Gates Foundation



Expansion of “pre-competitive” field

As a result, a number of public-private collaborations in translational medicine have emerged in recent years

Some examples:

- Alzheimer's Disease Neuroimaging Initiative (ADNI)
- Gates Foundation initiatives (e.g., vaccine development)
- Critical Path Institute (PSTC)
- Innovative Medicines Initiative
- The Biomarkers Consortium
- NCATS (NIH)

NIH: Steward of Medical and Behavioral Research for the Nation



“Science in pursuit of fundamental knowledge about the nature and behavior of living systems and the application of that knowledge to extend healthy life and reduce the burdens of illness and disability”

New NIH National Center for Advancing Translational Sciences (NCATS)

- Established December 23, 2011 via Consolidated Appropriations Act of the U.S. Congress as a new NIH Center

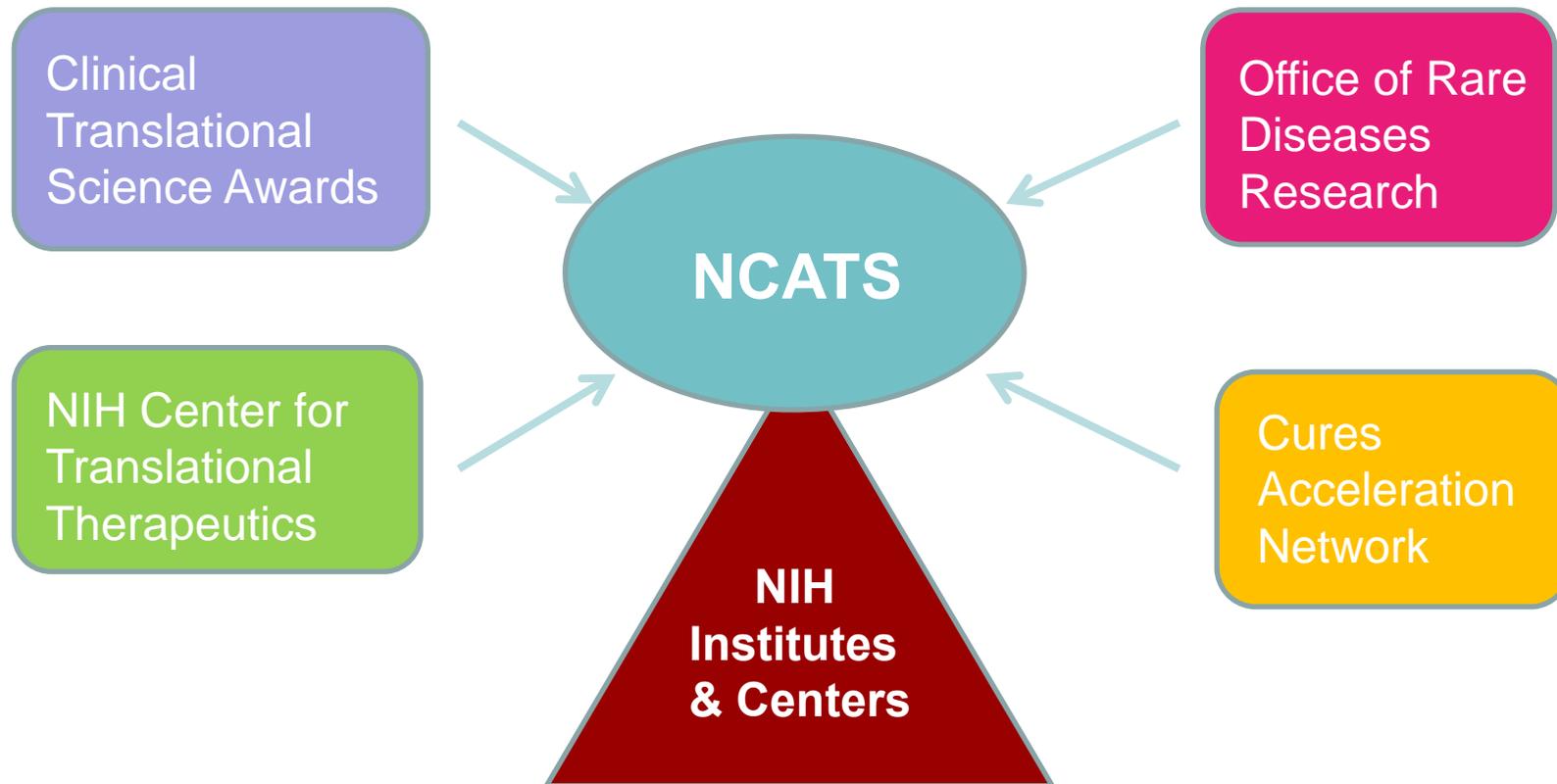
Mission:

“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: How will it work?

- NCATS will:
 - Provide tools and reagents for target validation
 - Create tools to accelerate translation
 - Innovate to improve the process of translation
 - Foster collaboration

NCATS represents a synthesis and expansion of existing capabilities within NIH



NCATS Programs

- **Clinical and Science Translational Awards**
 - Support national consortium of medical research institutions
 - Work together to improve the way clinical and translational research is conducted across the U.S.
 - Aim to accelerate the translational research process
- **Rare Diseases Research and Therapeutics**
 - Therapeutics for Rare and Neglected Diseases
 - Office of Rare Diseases Research

NCATS Programs

NIH Center for Translational Therapeutics : *Re-engineering Translational Sciences*

- NIH Clinical Genomics Center
 - Provides access to large-scale screening capacity to
 - Identify small molecules as chemical probes to study genes, cells, and biochemical pathways in health and disease
 - Validate new therapeutic targets for rare and neglected diseases
- NCATS Pharmaceutical Collection
 - Resource of 3,800 approved and investigational medicines to facilitate drug repurposing

NCATS Initiatives - examples

- **Tox21: Toxicology in the 21st Century (with EPA, FDA)**
 - Screen collection of 10,000 compounds composed of environmental chemicals and drugs approved for use
 - Looks for compounds' potential to disrupt biological pathways that may be toxic
- **Tissue Chip Collaboration**
 - \$70M, 5-year NIH-FDA-Defense Advanced Research Projects Agency (DARPA) collaboration
 - Aims to develop a tissue chip that mimics human physiology to screen for safe, effective drugs
- **NIH-Industry Compound Rescuing Initiative**
 - Industry provides compounds/data; NIH funds grants to generate innovative ideas for new uses

Cures Acceleration Network

- Created to advance development of high need cures
- Reduces barriers to translation in areas the private sector is less likely to pursue
- Funded via:
 - Grant award with/without partnership
 - Flexible Research Awards
- FY 2012 budget = US\$ 10 million

NCATS Will:

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

Website: www.ncats.nih.gov

FNIH Major Research Partnerships

- **Grand Challenges in Global Health** **\$249M**
Partner: Bill & Melinda Gates Foundation
- **Alzheimer's Disease Neuroimaging Initiative (ADNI)** **\$50M**
Partners: NIA, NIBIB & 20 companies/2 non-profits
- **Collaboration for AIDS Vaccine Discovery (CAVD)** **\$50M**
Partners: VRC/NIAID, Bill & Melinda Gates Foundation
- **The Biomarkers Consortium** **\$47M**
Partners: NIH, FDA, CMS, PhRMA, BIO, biopharmaceutical industry/non-profits
- **MAL-ED: The Interactions of Malnutrition and Enteric Infections,
Effect on Childhood Development** **\$30M**
Partner: Bill & Melinda Gates Foundation, Fogarty Institute Center (NIH)
- **Observational Medical Outcomes Partnership** **\$28M**
Partners: FDA, PhRMA, 16 pharmaceutical partners
- **Genetic Association Information Network (GAIN)** **\$26M**
Partners: NHGRI, NLM, Pfizer, Affymetrix, Perlegen Sciences, Broad Institute

Biomarker qualification: the value of collaboration

- Biomarkers require extensive testing and qualification for practical use
 - Multiple studies to ensure integrity, reproducibility of results
- Qualification is challenging, expensive, and time-consuming
 - Can require large amounts of data: literature, observational studies, clinical trials
- Qualification is based on consensus among the scientific community
 - Deep understanding of and agreement on disease risk, natural history, outcomes
- Qualification is a pre-competitive activity
- Qualification is difficult to accomplish this in a single institutional setting
- → Requires partnerships and a strategic approach

Regulatory agencies encourage public-private collaborations

Guidance for Industry

Qualification Process for Drug Development Tools

DRAFT GUIDANCE

This guidance document is being distributed for comment purposes only.

Comments and suggestions regarding this draft document should be submitted within 90 days of publication in the *Federal Register* of the notice announcing the availability of the draft guidance. Submit comments to the Division of Dockets Management (HFA-305), Food and Drug Administration, 5630 Fishers Lane, rm. 1061, Rockville, MD 20852. All comments should be identified with the docket number listed in the notice of availability that publishes in the *Federal Register*.

For questions regarding this draft document contact (CDER) Shaniece Gathers, 301-796-2600.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)

October 2010
Clinical/Medical

*FDA Biomarker
guidance published
October 22, 2010*

Goals of The Biomarkers Consortium



- Founded in 2006 to facilitate the development and standardization of biomarkers using new and existing technologies
- Qualifies biomarkers for specific applications in diagnosing disease, predicting therapeutic response, or improving clinical practice
- Generates information useful to inform regulatory decision-making
- Makes consortium project results broadly available to the entire scientific community

Contributing Members (42)

For-Profit Companies (18)

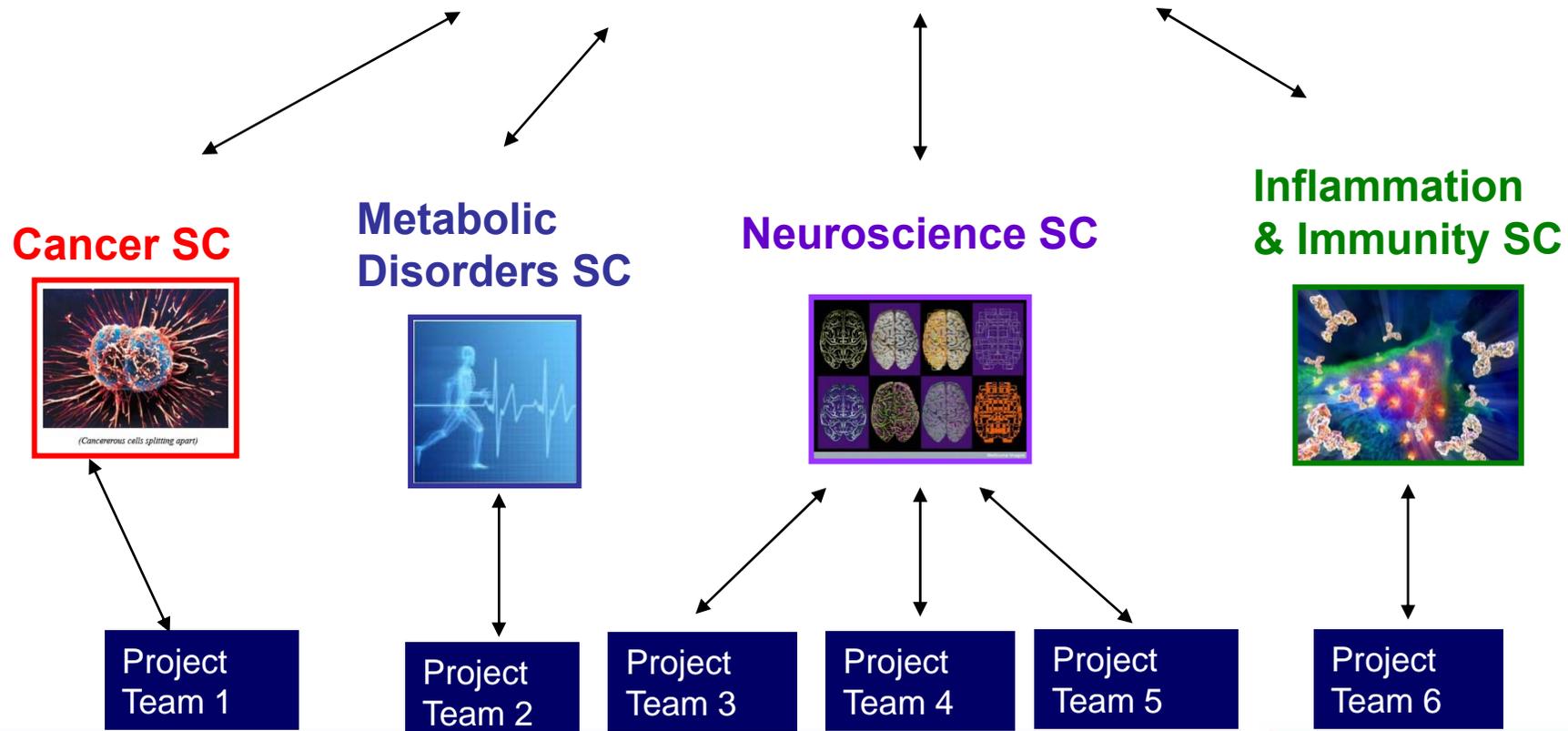
Abbott
Amgen
Amylin Pharmaceuticals
AstraZeneca
Banyan Biomarkers
Boehringer-Ingelheim
Bristol-Myers Squibb
Daiichi-Sankyo, Inc.
Eisai, Inc.
Hoffman-LaRoche/The Roche Group
Johnson & Johnson
Eli Lilly and Company
Merck
Metanomics Health GmbH
Myriad RBM
Pfizer Inc
Sanofi
Takeda

Non-Profit Organizations (24)

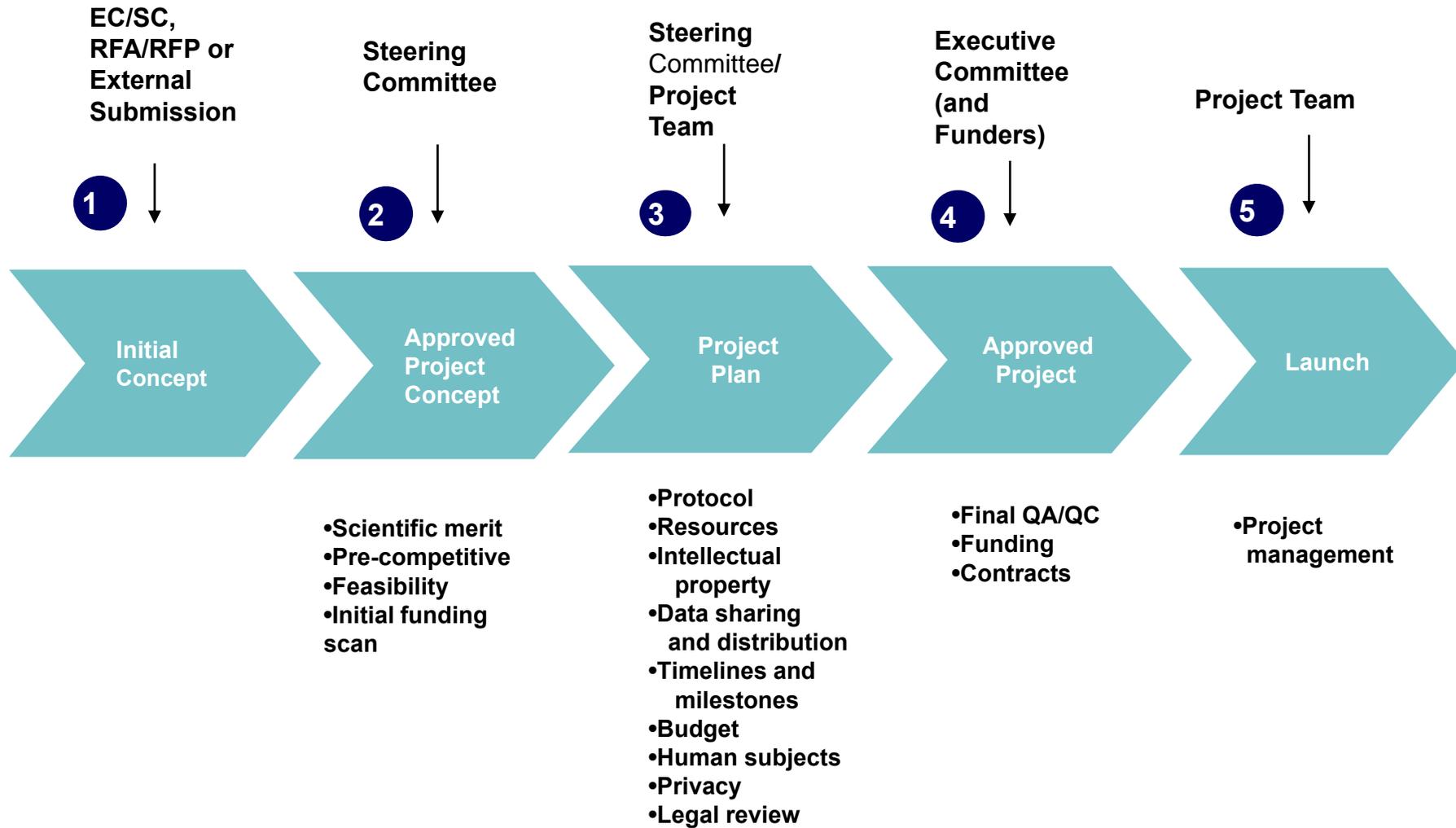
Alzheimer's Association
American Association for Cancer Research
American Diabetes Association
American Orthopaedic Society for Sports Medicine
American Society of Clinical Oncology
American Society for Radiation Oncology
Arthritis Foundation
Association of Clinical Research Organizations
Autism Speaks
Avon Foundation
Biotechnology Industry Organization
CHDI Foundation
Dairy Research Institute
Juvenile Diabetes Research Foundation
Kidney Cancer Association
The Leukemia and Lymphoma Society
Michael J. Fox Foundation for Parkinson's Research
Ontario Cancer Biomarker Network
Osteoarthritis Research Society International
Pharmaceutical Research and Manufacturers of America
PROOF Centre of Excellence
Radiological Society of North America
U.S. Pharmacopeia
University of Illinois

The Biomarkers Consortium Governance Structure

The Biomarkers Consortium Executive Committee
[NIH / FDA / CMS / industry / general public / Foundation for NIH]



Project Development Process



Accomplishments to Date

- 14 launched projects
 - 2 completed projects:
 - Utility of Adiponectin as a Biomarker to Predict Glycemic Efficacy
 - Alzheimer's Disease Targeted Plasma Proteomics
 - 4 projects scheduled to complete in 2012
- 3-4 additional projects in the 'pipeline'
- Marked progress toward projects focused on regulatory qualification
- Stable membership base/operations core
- Widely regarded as a model public-private partnership

Consortium projects address a broad range of biomarker strategies and target diseases

- *Early stage biomarker development*
 - Alzheimer's disease, Diabetes
- *Biomarker-driven disease definition*
 - Sarcopenia
- *Biomarker/platform/assay standardization*
 - Diabetes (beta cell mass/function), Carotid MRI, FDG-PET
- *Clinical studies to evaluate and qualify biomarkers*
 - Drug-induced kidney toxicity, Lung cancer/Lymphoma
- *Defining and qualifying clinical endpoints*
 - Bacterial pneumonia, Acute skin infection
- *In-silico disease modeling*
 - Atherosclerosis
- *Advanced clinical trial design (biomarkers, adaptive design)*
 - Breast cancer

I-SPY 2 Breast Cancer Trial

THE SATURDAY ESSAY | OCTOBER 2, 2010

A New Rx for Medicine

Fed up with slow drug trials, cancer patients and doctors are testing a fast track to personalized treatments.

THE WALL STREET JOURNAL
WSJ.com

By **RON WINSLOW**



When 37-year-old Kerry Landreth discovered a lump in her breast last April, she was told it would take three weeks to get a doctor's appointment to have it checked.

"I don't do three weeks," she recalls saying. "How about today?"

By the end of the day, she had talked her way into a doctor's appointment, a mammogram and a biopsy to determine whether the suspicious lump was a tumor. A few days later came the diagnosis: stage 2 invasive ductal breast cancer, a particularly aggressive form of the disease. When a surgeon recommended a double mastectomy, she decided to consider other options.

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