



# FNIH

Foundation for the  
National Institutes of Health

## Private Public Partnerships: Perspectives from the Foundation for the NIH

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# FNIH

*Building partnerships for discovery and innovation to improve health.*

## PURPOSE

Support the mission of the NIH  
Advance collaboration with biomedical researchers from universities, industry, not-for-profit organizations

## STRUCTURE

501(c)(3) not-for profit foundation created by Congress  
Independent Board of Directors with NIH Director and FDA Commissioner as *ex-officio* Board members

## HIGHLIGHTS

Raised over \$800 million since 1996  
Supported nearly 500 projects  
94 cents of every dollar spent directly funds programs

# Our role...

*What we do, how we do it...*



## Identify:

- Important scientific problems
- Key players
- Resources required and sources of support
- Neutral convener; trusted party to provide safe harbor for discussions

## Establish:

- Highest level of ethical standards
- Clear goals and milestones
- Effective mechanism to generate scientific consensus
- Nimble infrastructure and project expert project management

## Facilitate:

- Discussions with key opinion leaders and regulatory decision makers
- Integrated approach to cross-sector partnerships
- Communications; ensure all partners' voices are heard;

## Enable:

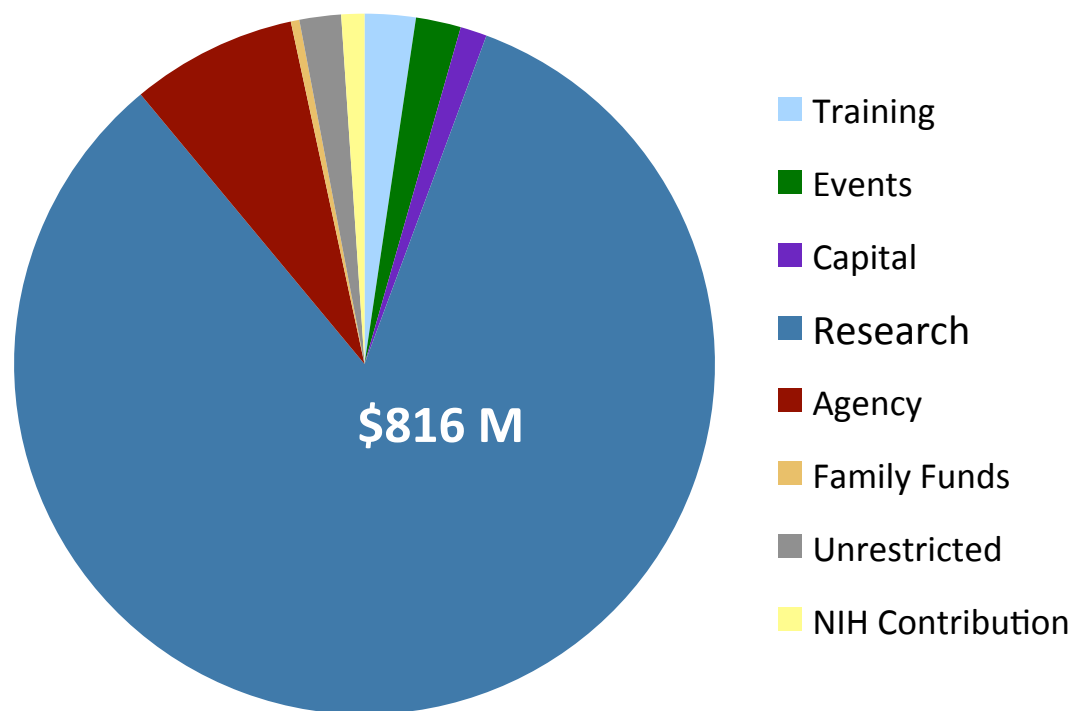
- Sharing of data and expertise to collaboratively address medical needs
- Resource mobilization
- Manage grants, contracts, and projects; oversee and conduct research

# Funding History

*Support for over 500 projects*



Our history of support for hundreds of projects since 1996 offers many models for partnerships between the public and the private sectors, ranging from simple fund transfers to highly complex and interactive programs.

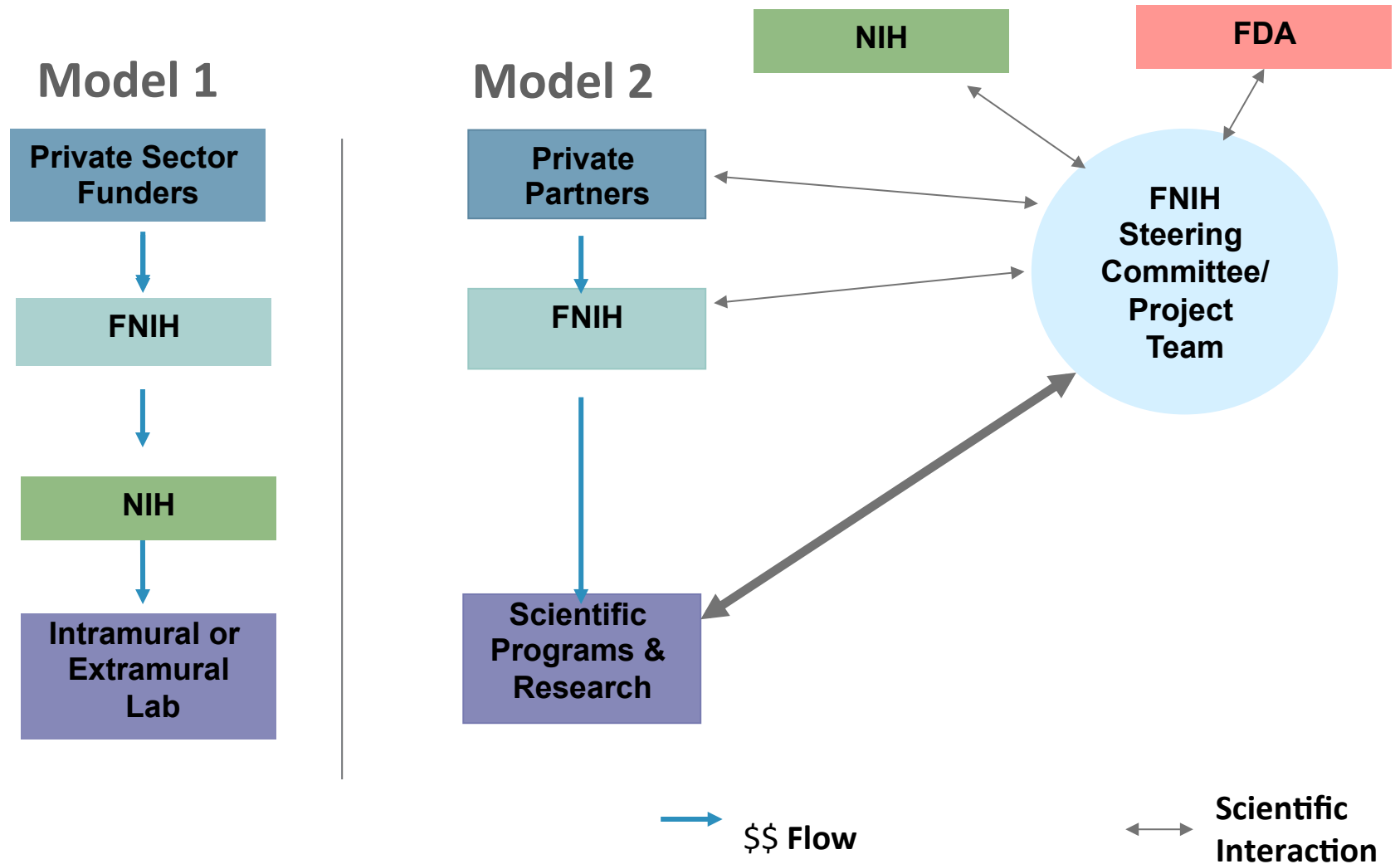


# How we Fund Our Programs

*FNIH has no endowment*



# Funding & Partnership Models

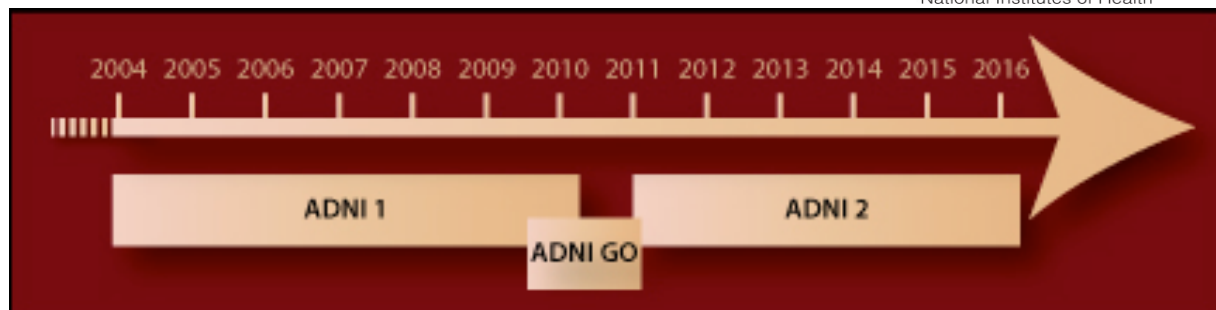


# Major FNIH Research Partnerships

- |  |               |
|--|---------------|
| <ul style="list-style-type: none"> <li> <b>Accelerating Medicines Partnership</b><br/>           Partner: NIH (OD), NIA, NIAMS, NIDDK, 10 companies, 7 non-profits         </li> </ul>   | <b>\$230M</b> |
| <ul style="list-style-type: none"> <li> <b>Grand Challenges in Global Health (GCGH)</b><br/>           Partners: Bill &amp; Melinda Gates Foundation         </li> </ul>   | <b>\$201M</b> |
| <ul style="list-style-type: none"> <li> <b>LungMAP: Master Lung Protocol Trial</b><br/>           Partners: NCI (SWOG), FDA, Friends of Cancer Research, 5 companies to date         </li> </ul>                                     | <b>\$163M</b> |
| <ul style="list-style-type: none"> <li> <b>Alzheimer's Disease Neuroimaging Initiative (ADNI)</b><br/>           Partners: NIA, NIBIB &amp; 20 companies/2 non-profits         </li> </ul>   | <b>\$148M</b> |
| <ul style="list-style-type: none"> <li> <b>Vector-Based Control of Emission (VCTR)</b><br/>           Partners: Bill &amp; Melinda Gates Foundation         </li> </ul>  | <b>\$78M</b>  |
| <ul style="list-style-type: none"> <li> <b>The Biomarkers Consortium</b><br/>           Partners: FDA, NIH, CMS, PhRMA, BIO, 17 companies, 16 non-profits         </li> </ul>  | <b>\$60M</b>  |
| <ul style="list-style-type: none"> <li> <b>Comprehensive T Cell Vaccine immune Monitoring Consortium (CT-VIMC)</b><br/>           Partners: VRC/NIAID, Bill &amp; Melinda Gates Foundation, NIAID         </li> </ul>                | <b>\$50M</b>  |
| <ul style="list-style-type: none"> <li> <b>MAL-ED: The Interactions of Malnutrition and Enteric Infections, Effect on Childhood Development</b><br/>           Partner: Bill &amp; Melinda Gates Foundation, FIC         </li> </ul> | <b>\$46M</b>  |

# Alzheimer's Disease Neuroimaging Initiative (ADNI)

## GOALS



1. To detect AD at the earliest stage possible and identify ways to track the disease through biomarkers.
2. To support advances in AD intervention, prevention and treatment through the application of new diagnostic methods to apply at the earliest stages technically possible - when intervention may be most effective.
3. To continually develop ADNI's data access policy and continuously improve and expand the unprecedented data sharing model.





# Current PPSB Partners for ADNI2



**Private Partner Scientific Board (PPSB):** Independent, open, and pre- competitive forum



# Accelerating Medicines Partnership: AMP

Transform the current model for developing new diagnostics and treatments by jointly identifying and validating promising biological targets of disease.

THE WALL STREET JOURNAL. U.S.

U.S. NEWS

## Drug Companies Join NIH in Study of Alzheimer's, Diabetes, Rheumatoid Arthritis, Lupus

Ten Drug Companies Form Pact With NIH to Find Path

By MONICA LANGLEY and JONATHAN D. ROCKOFF

USA TODAY  
A GANNETT COMPANY

NEWS SPORTS LIFE MONEY TECH TRAVEL OPINION

## Ten rival drug companies partner with government

Liz Szabo, USA TODAY 6:01 p.m. EST February 4, 2014

*Rival drug companies plan to collaborate on research against key diseases.*



(Photo: Jack Gruber, USA TODAY)

In an unprecedented move designed to jump-start medical science, 10 rival drug companies that normally compete ferociously against each other will now cooperate not just with government researchers and non-profits, but with each other.

The White House  
Office of the Press Secretary

For Immediate Release

February 04, 2014

## Statement by the President on the Accelerating Medicines Partnership

Today, my Administration is taking action to accelerate the development of life-saving drugs and to help identify new treatments and cures for diseases like Alzheimer's, diabetes, rheumatoid arthritis, and lupus. The Accelerating Medicines Partnership - innovation of our private sector companies to meet the health challenges.

The Washington Post PostTV Politics Opinions Local Sports National

## Health & Science

### NIH announces novel venture with drug companies to fight major diseases

By Ariana Eunjung Cha, Published: February 4

## NIH, drug companies team up to target diseases

The National Institutes of Health is partnering with researchers from 10 rival drug companies to develop new treatments for Alzheimer's, diabetes, rheumatoid arthritis, and lupus.

The partnership will change the way



Science Insider

Breaking news and analysis from the world of science policy

## NIH, 10 Drug Companies Partner to Study Four Diseases

By: Jocelyn Kaiser Tuesday, February 4, 2014 - 3:45pm | 4 Comments

Ramping up its efforts in drug discovery, the National Institutes of Health (NIH) today unveiled what it called an unprecedented \$230 million, 5-year partnership with 10 drug companies aimed at finding new treatments for Alzheimer's disease, diabetes, rheumatoid arthritis, and lupus.

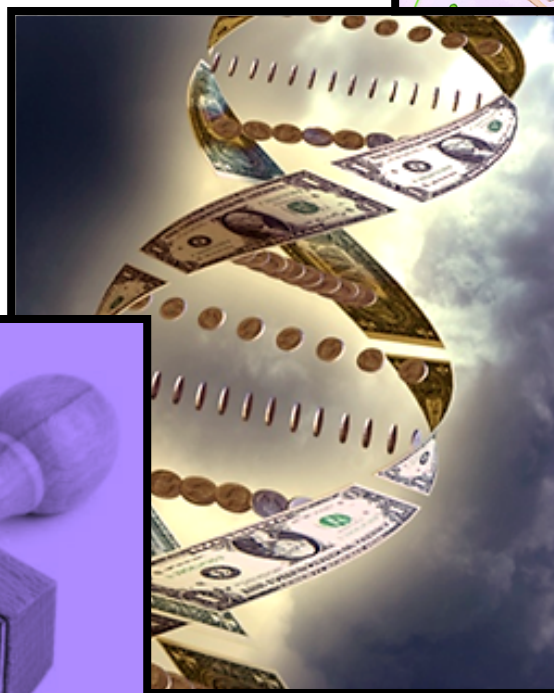
In a room at the Washington, D.C., National Press Club packed with representatives from industry, patient groups, and federal officials, NIH Director Francis Collins described the Accelerating Medicines Partnership (AMP). The goal is to cut down on the more than 95% failure rate for drug candidates. As a result, it now takes some 10 years and more than \$1 billion to develop a



Accelerating Medicines Partnership/Camazine Scott

# AMP: the problem

Developing effective new medicines takes too long, costs too much and fails too often.



# AMP: Improving R&D Efficacy

## Current targets

- Animal models
- Cell lines



## AMP targets

- Emerging Technologies
  - DNA sequencing
  - Proteomics
  - Single-cell analysis
  - Bioengineered cells
  - Imaging
- Extensive Human Data
  - Tissue/blood samples
  - Clinical information
  - Demographics
- Big Data Tools



Lack of efficacy currently accounts for more than half of all drug failures in Phase II clinical studies



AMP's target validation efforts aimed at improving efficacy and increasing success rate

# AMP – Research Initiatives

Disease area	Research plan topics	Deliverables and approach
<b>Alzheimer's disease</b>	Exploratory biomarker validation in clinical trials and network analysis on human tissue	<ul style="list-style-type: none"> <li>• <b>Embed biomarkers in NIH-funded clinical trials</b> to develop biomarkers of disease progression and surrogate endpoints</li> <li>• <b>Conduct network analysis in human brain samples</b> to identify genetic nodes &amp; networks linked to AD to support target identification &amp; validation</li> </ul>
<b>Type 2 Diabetes</b>	Sequencing & phenotyping of targets of interest and a tool to enable easy interrogation of all available data	<ul style="list-style-type: none"> <li>• <b>Create a knowledge portal</b> containing comprehensive genotype/phenotype data sets – apply informatics to identify predictors of risk and potential drug targets</li> <li>• <b>Conduct targeted sequencing/genotyping</b> of high priority targets of interest (as defined by industry)</li> </ul>
<b>RA, SLE &amp; related autoimmune diseases</b>	Immune module deconstruction with blood/tissue and cross-disease comparisons	<ul style="list-style-type: none"> <li>• <b>Conduct extensive profiling of key immune modules</b> in highly refined subsets of relevant cells in informative cohorts to establish pathway/network maps of RA &amp; SLE; high priority targets identified from pathway analysis to be validated via RNAi.</li> <li>• <b>Make the data available in a knowledge portal</b> <ul style="list-style-type: none"> <li>- Informative cohorts include: Early RA, Established RA (responder/non-responder), Lupus Nephritis, Skin Lupus</li> </ul> </li> </ul>

# AMP-AD



## Project A: Biomarkers Project

- **Alzheimer's Disease Cooperative Study (ADCS) Anti-Amyloid Treatment in Asymptomatic Alzheimer's Disease Trial (A4 Trial) (U19AG010483)** Reisa Sperling, Harvard Medical School; Paul Aisen, University of California, San Diego
- **Dominantly Inherited Alzheimer Network (DIAN) Trial (U01AG042791)** Randall Bateman, Washington University
- **Alzheimer's Prevention Initiative APOE4 Trial (API APOE) (UF1AG046150-01)** Eric Reiman, Banner Alzheimer's Institute; Pierre Tariot, Banner Alzheimer's Institute

Baseline data from the trials will be made broadly available through the Alzheimer Association's **GAAIN** collaborative platform



# AMP-AD



**Project B: Target Discovery and Preclinical Validation Project**— The cooperative agreement grants that constitute the consortium were developed in response to the NIA funding opportunity RFA AG13-013:

- **Pathway Discovery, Validation and Compound Identification for Alzheimer's Disease** (U01AG046152) Philip L. De Jager, Brigham and Women's Hospital and the Broad Institute, Inc. David A. Bennett, Rush University
- **Integrative Biology Approach to Complexity of Alzheimer's Disease** (U01AG046170) Eric Schadt (Contact PI), Icahn School of Medicine at Mount Sinai
- **A System Approach to Targeting Innate Immunity in Alzheimer's Disease** (U01AG046139) Todd Golde (Contact PI), University of Florida
- **Discovery of Novel Proteomic Targets for Treatment of Alzheimer's Disease** (U01AG046161) Allan Levey, Emory University David A. Bennett, Rush University

**Sage Bionetworks** facilitates data sharing and data integration activities within the Target Discovery and Preclinical Validation AMP-AD project. – **AMP\_AD Knowledge Portal**

# The Biomarkers Consortium



*Fosters the exchange of knowledge and expertise among industry, academic, and government leaders*

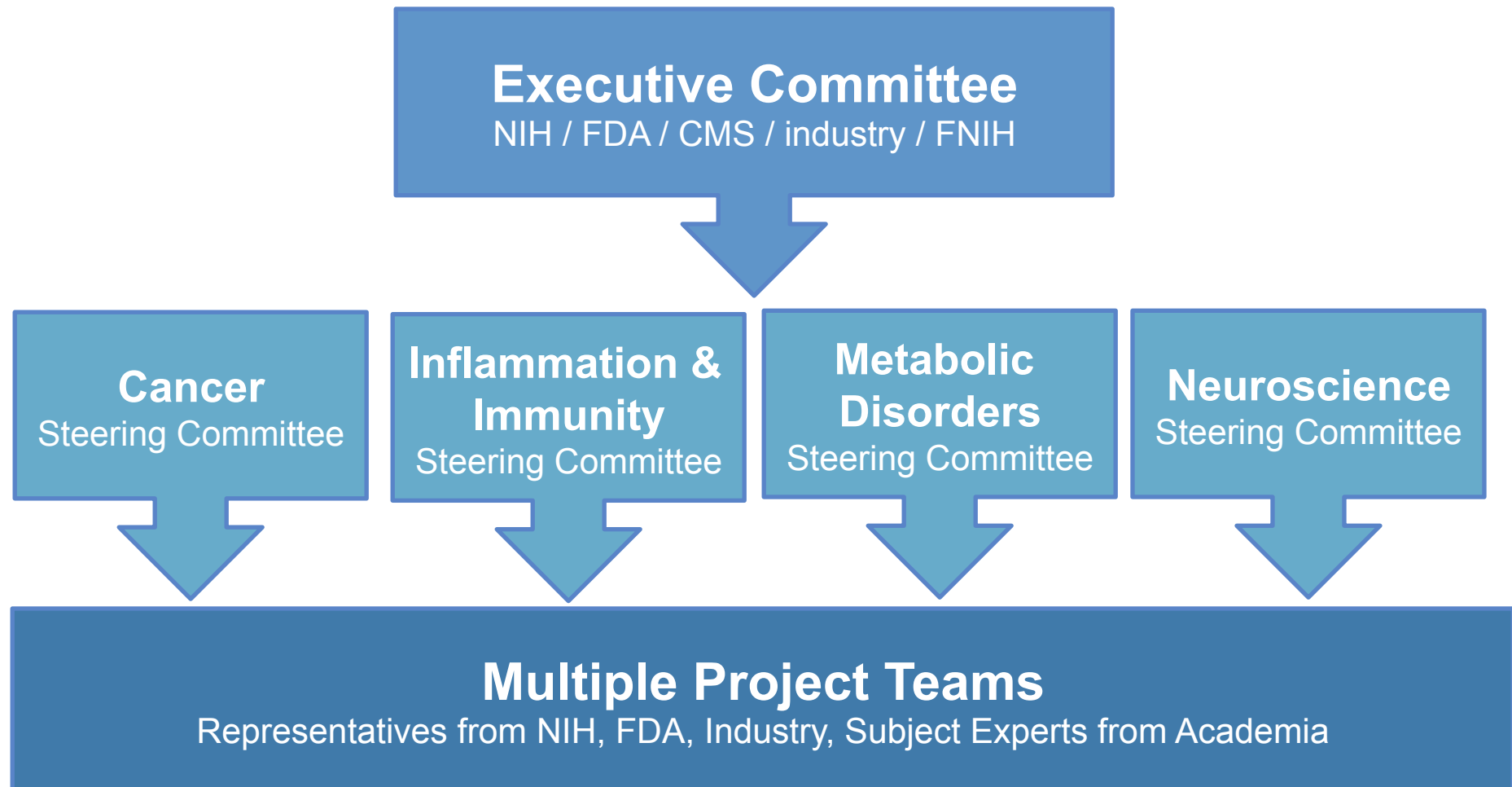
- Develops biomarkers for specific applications in diagnosing disease, predicting therapeutic response, and improving clinical practice
- Generates information useful to inform **regulatory decision-making** in the qualification process
- Employs rigorous, inclusive governance and project management with clearly defined goals and milestones
- Addresses a broad range of disease / therapeutic areas
- Pre-competitive; makes consortium project results broadly available to the entire scientific community

**Our Founding Partners: FDA, NIH, FNIH, PhRMA, BIO, CMS**



# Biomarkers Consortium

## governance selects and plans projects



# Biomarkers Consortium: 19 Launched Projects to Date



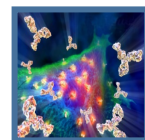
## Executive Committee

- Kidney Safety Biomarkers
- Skin Infection Pneumonia (CABP/ABSSSI)
- Hospital-Acquired & Ventilator Acquired Bacterial Pneumonia



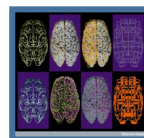
## Cancer

- FDG-PET in Lung Cancer
- FDG-PET in Lymphoma
- I-SPY 2 Trial\*\*
- Minimal Residual Disease in ALL
- Volumetric CT for Clinical Trials



## Inflammation and Immunity

- Osteoarthritis Biomarkers



## Neuroscience

- PET Radioligand in Neuroinflammation
- Alzheimer's Plasma Proteomics
- Alzheimer's CSF Proteomics\*
- Alzheimer's / MCI Placebo Data Analysis\*

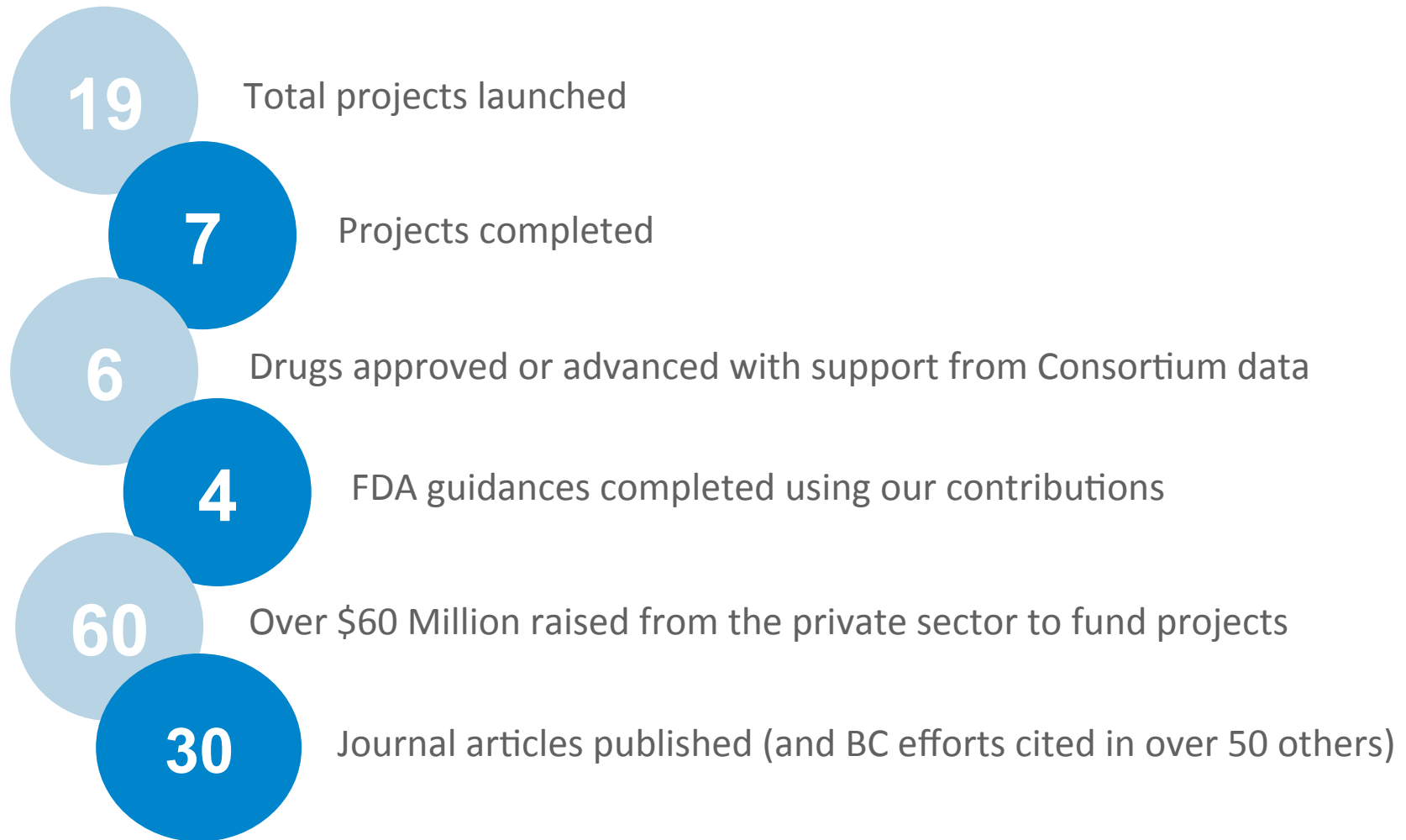


## Metabolic Disorders

- Adiponectin
- Carotid MRI Reproducibility
- Sarcopenia Consensus Definition
- Atherosclerosis In-Silico Modeling
- Beta Cell Clinical Studies
- Bone Quality

➤ Completed Projects   ■ Ongoing Projects   \* Final publication pending   \*\* Transitioned to standing trial

# Biomarkers Consortium Results



# Applying Precision Medicine to Clinical Trials

## Umbrella

Test impact of different drugs on different mutations in a single type of cancer

- BATTLE
- I-SPY2
- SWOG Squamous Lung Master



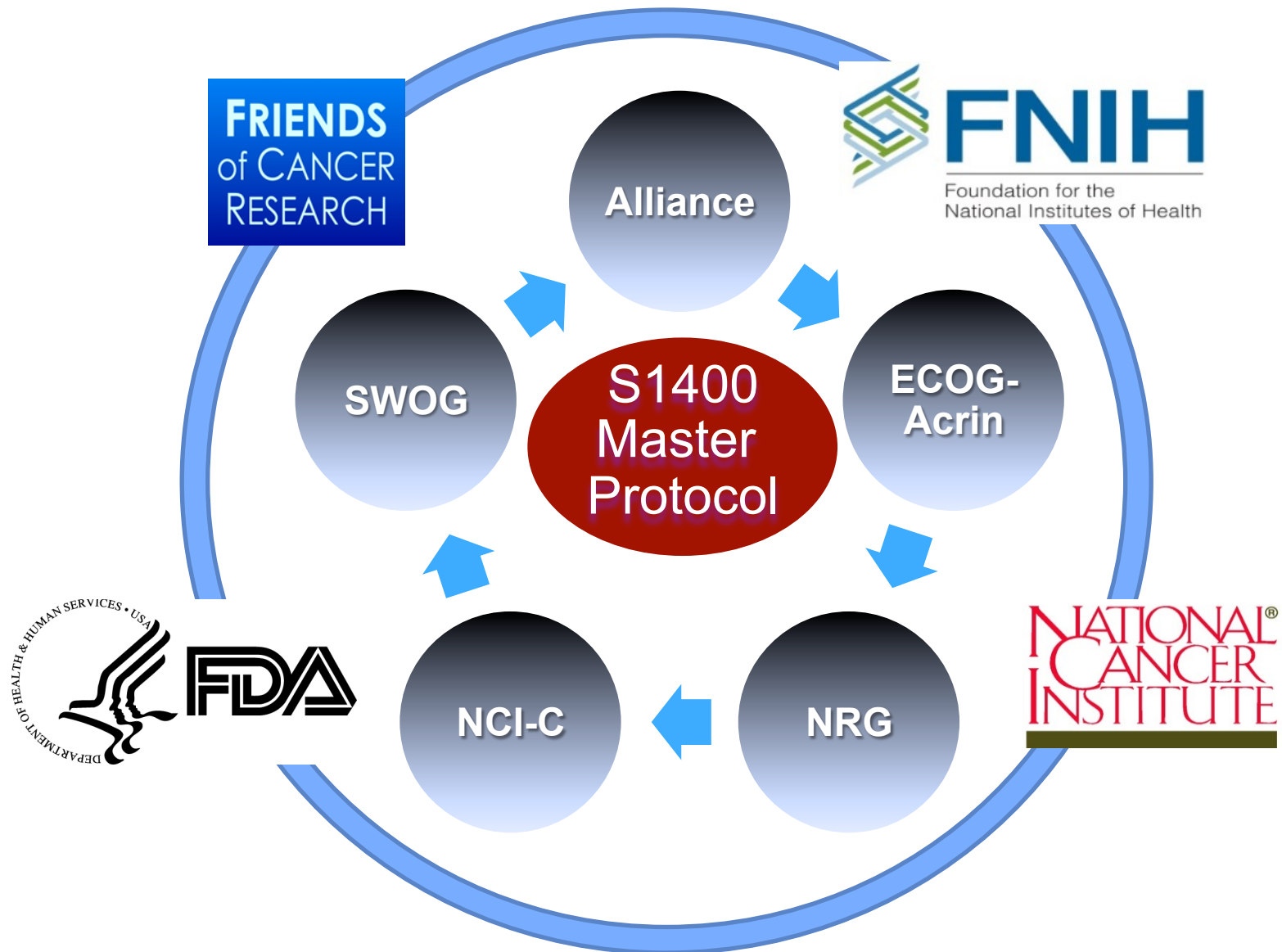
## Basket

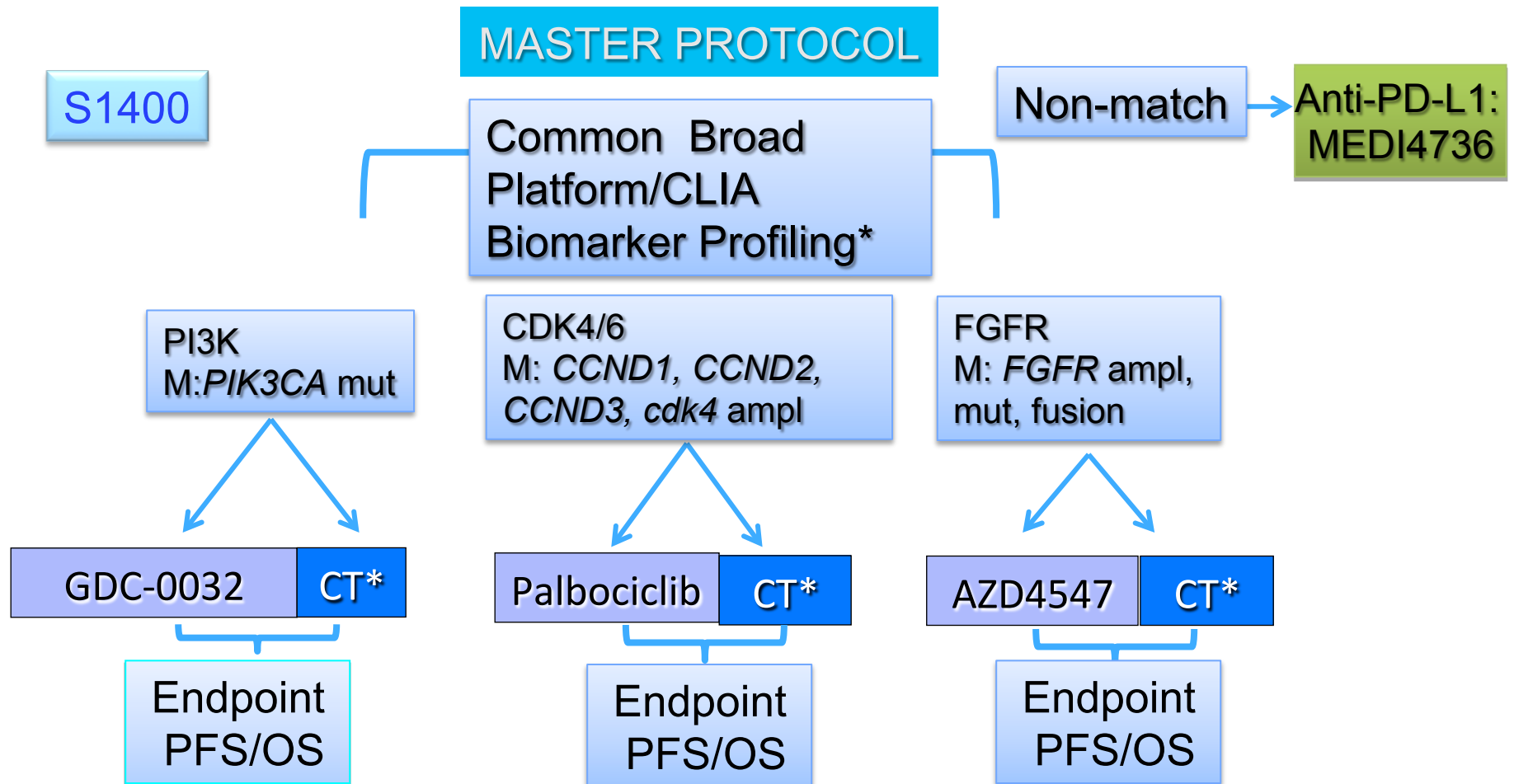
Test the effect of a drug(s) on a single mutation(s) in a variety of cancer types

- Imatinib Basket
- BRAF+
- NCI MATCH



# Applying Precision Medicine to Clinical Trials





TT=Targeted therapy, CT=chemotherapy (docetaxel or gemcitabine), E=erlotinib

\*Archival FFPE tumor, fresh CNB if needed

Target/M: Drug target and biomarker

# Partnerships – Lessons Learned

- A matrix that is greater than the sum of its parts
- Well defined objectives, budgets, milestones and deliverables
- Common governance, rules and legal framework
- Realistic funding goals, expectations and timelines
- Projects aligned with donor interest
- Appreciation of the value of gifts
- Collaboration adds complexity: must “play nice with others!”
- Nimble, transparent and accountable

*“When you’ve seen  
one partnership,  
you’ve seen one  
partnership.”*

# Contacts



## Research Partnerships

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## Fundraising

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## Inflammation & Immunity

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## Metabolic Disorders

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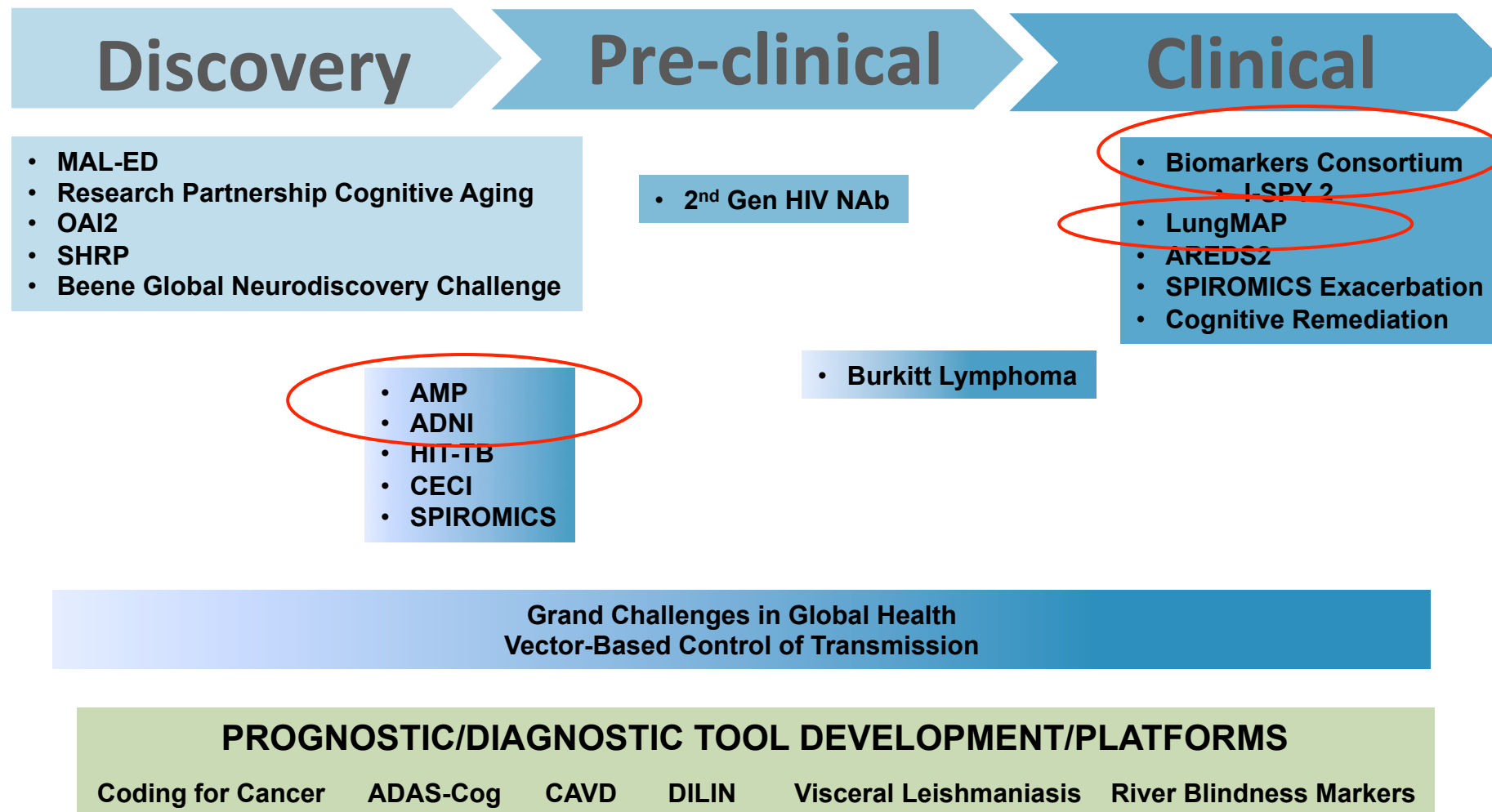
## Neuroscience

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# Alternate Slides

# FNIH Research Projects in the R&D context



# Alzheimer's Disease Neuroimaging Initiative (ADNI)



- Launched in 2004 by the National Institute on Aging (NIA) as an innovative \$ 60M collaborative effort supported with funding from both the federal government and the private sector; coordinated by the Foundation for the NIH.
- Designed to be a multi-site longitudinal study of normal cognitive aging, mild cognitive impairment (MCI) and early Alzheimer's disease (AD).
- Plan to validate, standardize and optimize:
  - neuroimaging and other biomarkers for use in clinical trials in Alzheimer's Disease
  - biomarker methods for early detection and disease progressionvia a collaborative network of clinical and imaging sites.
- Aims to help create a world-wide network to improve AD studies and clinical trials for disease-modifying treatments.
- In 2011 ADNI2 launched with an additional \$70M. Builds up on the successes of earlier ADNI phases and seeks to identify the earliest changes in brain structure and function as people transition from normal cognitive aging to mild cognitive impairment (MCI) to AD.
- ADNI 3 planning is now underway.



# AMP Participants by Disease Area



## Alzheimer's Disease

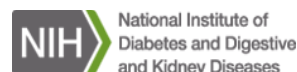
## Type 2 Diabetes

## RA, SLE & related

### Industry members



### Government members



### Non-profit members



# Current AMP Funding Commitments (total: 5 years)



Disease area	Total project funding (\$M)	Total NIH funding (\$M)	Total industry funding (\$M)	Total non-profit funding (\$M)
AD	92.5	69.6	21.9*	1.0
T2D	52.8	31 + **	21.5*	.3
RA/SLE	41.9	20.9	20.7	.3
Total	187.2	121.5	64.1	1.6

\* Does not include in-kind contributions of \$40M to AD and \$6.5M to T2D

\*\* Additional funding anticipated

# Biomarkers Consortium

## Contributing Partners



### **For-Profit Companies**

Actelion  
Amgen  
AstraZeneca  
Crescendo Bioscience  
Daiichi Sankyo, Inc  
Eisai, Inc  
Johnson & Johnson  
Eli Lilly & Company  
Lundbeck  
Merck Sharpe & Dohme Corp.  
Metabolon  
Mitsubishi Tanabe Pharma America, Inc  
Myriad RBM  
Pfizer, Inc  
Regeneron Pharmaceuticals, Inc.  
Sanofi  
Takeda Pharmaceuticals USA, Inc

### **Non-Profit Organizations**

Alzheimer's Association  
American Diabetes Association  
American Orthopaedic Society for Sports Medicine  
Arthritis Foundation  
Autism Speaks  
Biotechnology Industry Organization  
California Dairy Research Foundation (CDRF)  
Centre for Proteomic and Genomic Research  
CHDI Foundation  
Dairy Research Institute  
Foundation for Health Improvement and Technology  
Juvenile Diabetes Research Foundation  
Pharmaceutical Research and Manufacturers of America  
PROOF Centre of Excellence  
Radiological Society of North America  
US Pharmacopeia

# Alzheimer's Disease Neuroimaging Initiative (ADNI)



- Industry leaders work together in a proven, pre-competitive environment
  - Cost sharing and cost savings
  - High level interactions with top clinical/biomarker AD investigators
  - Idea and data sharing not possible in competitive environment
  - Interaction with FDA – ongoing guidance on endpoints and validation
- Study design and objectives address industry needs, i.e.:
  - Standardization of all methods for regulatory approval
  - Identification of patients at risk in the pre-dementia stage
  - Validation of biomarkers to measure change and treatment effects
  - Validation of biomarkers to identify early AD pathology
  - Longitudinal data with biomarkers for design and powering of trials
- ADNI plays a major role in:
  - Providing new information concerning the pathophysiology of AD
  - Developing early detection methods and improved treatment trials
  - Determining diagnostic requirements
  - Providing tools for evaluating milder patients
  - Understanding disease progression/rate of change during different stages of disease

# AMP – IP & Data Sharing

- Research supported by AMP will be precompetitive
- All data will be shared broadly
- AMP supported research will not be patented





# Lung-MAP Objectives and Rationale

- *Multi-arm Master Protocol*
  - Homogeneous patient populations & consistent eligibility from arm to arm;
  - Each arm independent of the others;
  - Infrastructure facilitates opening new arms faster;
  - *Phase II-III design* allows rapid drug/biomarker testing for detection of “large effects.”
- *Screening large numbers of patients* for multiple targets by a broad-based NGS platform reduces the screen failure rate.
- Provides a *sufficient “hit rate”* to engage patients & physicians.
- *Bring safe & effective drugs to patients faster.*
- Designed to facilitate *FDA approval* of new drugs.