



FOUNDATION
FOR THE
National Institutes of Health

Knowledge Markets in Biomedical Research Public-Private Partnerships: GAIN and The Biomarkers Consortium

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Foundation for NIH Overview

Mission

- Sole entity authorized by the U.S. Congress to raise private funds in support of NIH's mission of improving health through scientific discovery and translational research
- Creates innovative public-private biomedical partnerships with NIH and other federal partners, industry, academia, the philanthropic community

Organization

- Independent nonprofit 501(c)(3) public charity founded in 1990; ; independent Board of Directors with public and private sector representation

Track Record

- Proven expertise in public-private partnership development and management
- Currently supporting 50+ projects; raised over \$410M since 1996
- 96 cents of every dollar raised supports programs


Major Public-Private Partnerships

Grand Challenges in Global Health Partner: Bill & Melinda Gates Foundation	\$200M
Collaboration for AIDS Vaccine Discovery (CAVD) Partners: VRC/NIAID, Bill & Melinda Gates Foundation	\$33M
Alzheimer's Disease Neuroimaging Initiative (ADNI) Partners: NIA & AstraZeneca, Bristol-Myers Squibb, Eisai, Elan, GE Healthcare, GlaxoSmithKline, Innogenetics, Eli Lilly, Merck, Novartis, Pfizer, Schering-Plough, Synarc, Wyeth Alzheimer's Association, Institute for the Study of Aging	\$27M
Genetic Association Information Network (GAIN) Partners: NHGRI, NLM & Pfizer, Affymetrix, Broad Institute, Abbott Laboratories	\$26M
Osteoarthritis Initiative (OAI) Partners: NIAMS & Pfizer, Novartis, Merck, GlaxoSmithKline	\$18.5M
Avon-NCI Progress for Patients Award Program Partners: NIAMS & Pfizer, Novartis, Merck, GlaxoSmithKline	\$12M
The Biomarkers Consortium Partners: NIH, FDA, CMS, PhRMA, BIO, pharmaceutical/biotech companies, non-profits	\$10.4M*
Schizophrenia Metabolic Initiative Partners: NIMH & Bristol-Myers Squibb	\$8M

* to date

PPPs: The Key Reagents

- True agreement by all parties to a common set of goals
- Clear public benefit
- High-level leadership from all sectors
- Public and private sector balance
- Appropriate governance structure and partnership → using a trusted third party intermediary if possible
- Policies
- Adequate financial and project management resources
- Transparent communication between partners
- Clear recognition of goals and distinct roles and limitations of each partner
- Commitment to move fast



PPP-based knowledge markets present special challenges

- Antitrust
- Government regulations/policies
- Governance
- Privacy/Confidentiality
- Human Subjects Protections
- Intellectual Property
- Data Access:
 - Who gets access, for what purposes, and under what conditions?
- Publication and Communication
- Ownership of Data and Infrastructure
- Data Quality



The Genetic Association Information Network (GAIN)

- Partnership with FNIH, NHGRI, NLM, Pfizer, Abbott, Affymetrix, Perlegen Sciences, The Broad Institute
- Genotyped 18,000 samples from existing case-control studies in 6 common diseases
- Studies chosen via peer review, technical assessment group (TAG), and 21-member Steering Committee final selection
- Resulting genotypes and phenotypes made broadly available to qualified researchers via large-scale database (dbGaP) built at NLM (NCBI)
- Developed extensive set of policies governing use (human subjects, privacy, intellectual property, data access, publication)—basis for policies now used broadly for GWAS studies across NIH

The Guiding Principle of GAIN:

The greatest public benefit will be achieved if results of whole genome association studies are made immediately and broadly available

Key GAIN Information Policies

- **Data Quality:** Extensive analysis beyond peer review
- **Privacy/Confidentiality:** Home institutions responsible for compliance with HIPAA/regulations; phenotypic data is double-coded
- **Human subjects:** Oversight for initial participation resides at local level; data use restrictions are enforced via Data Use Certification and oversight by an NIH-based Data Use Review Board
- **Intellectual property:** GAIN data is pre-competitive; pre-computed associations and Data Use Certification discourage pre-emptive claims
- **Data access:** No “early look” at data; controlled access to individual genotype and phenotype data via online application process & NIH-based Data Access Committee review
- **Publications:** Contributing investigators get 9-month ‘head start’ on submitting analyses on their data for publication

The Biomarkers Consortium

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PhRMA
Pharmaceutical Research and Manufacturers of America

Bio
BIOTECHNOLOGY
INDUSTRY ORGANIZATION

The Biomarkers Consortium seeks to address key challenges in biomarker development

- There are 1,000s of candidate biomarkers in the literature
- Biomarkers require extensive testing and qualification for practical use
- Biomarkers Qualification:
 - Challenging, expensive, and time-consuming
 - Requires consensus among the scientific community
 - Can be a pre-competitive activity
 - Regulatory pathways still being refined
- Difficult to accomplish these activities through a single entity/sector

Requires partnerships and a strategic approach

The Biomarkers Consortium

Goals:

- Advance the discovery, development, qualification and regulatory acceptance of biomarkers
- Conduct joint research in “pre-competitive” areas with partners that share common interest in advancing human health and improving patient care
- Speed the development of medicines and therapies for detection, prevention, diagnosis and treatment of disease
- Make Consortium project results broadly available to the entire research community

Contributing Members (55)

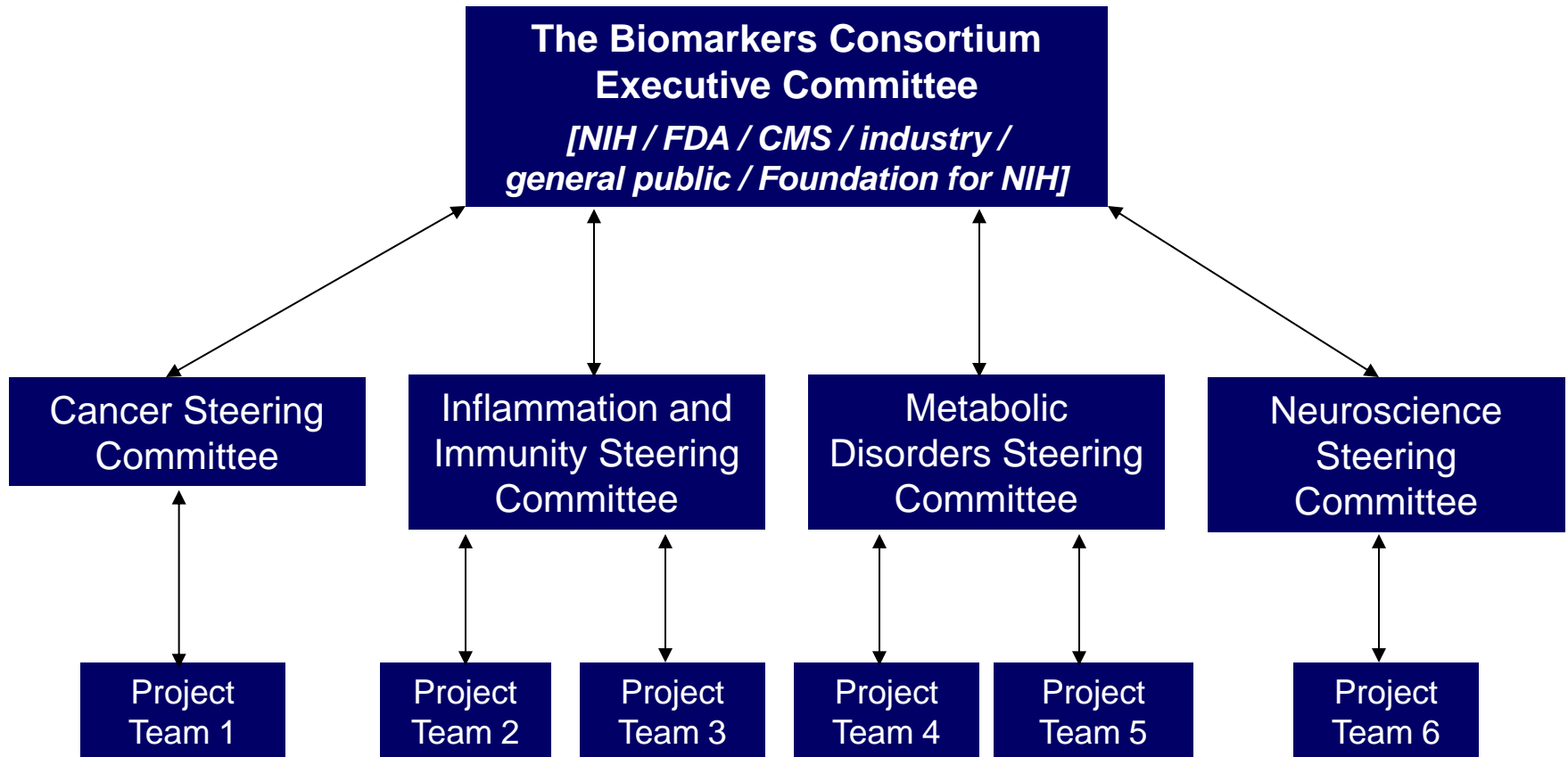
For-Profit Companies (24)

Althea Technologies
 AstraZeneca
 Avalon Pharmaceuticals
 BG Medicine
 Boehringer-Ingelheim Pharmaceuticals
 Bristol-Myers Squibb
 Digilab Biovision GmbH
 EMD Serono
 Genstruct
 GlaxoSmithKline
 GVK Biosciences
 Ingenuity Systems
 Johnson & Johnson
 Eli Lilly and Company
 Luminex Corporation
 Lundbeck
 Merck and Co., Inc.
 Novartis
 Novo Nordisk
 Pfizer Inc
 F. Hoffmann-La Roche
 Scout Diagnostics
 Wyeth

Nonprofit Organizations (31)

Academy of Molecular Imaging
 Advanced Medical Technology Association
 Alliance for Aging Research
 Alzheimer's Association
 American Association for Cancer Research
 American Cancer Society
 American College of Neuropsychopharmacology
 American Health Assistance Foundation
 American Society for Clinical Pharmacology and Therapeutics
 American Society for Therapeutic Radiology and Oncology
 American Society of Clinical Oncology
 Association of Clinical Research Organizations
 Autism Speaks
 Battelle Memorial Institute
 Biotechnology Industry Organization
 Federation of Clinical Immunology Societies
 Hamner Institutes for Health Sciences
 High Q Foundation
 Immune Tolerance Institute
 Polo Ralph Lauren Foundation
 Juvenile Diabetes Research Foundation
 Kidney Cancer Association
 The Leukemia and Lymphoma Society
 Lupus Foundation of America
 Lupus Research Institute
 Michael J. Fox Foundation for Parkinson's Research
 Pharmaceutical Research and Manufacturers of America
 Radiological Society of North America
 Ryan Licht Sang Bipolar Foundation
 Society of Nuclear Medicine
 Vanderbilt University

The Biomarkers Consortium Governance Structure



The Biomarkers Consortium Executive Committee

Chairman

Charles Sanders, *Foundation for NIH*

NIH

Thomas Insel, *NIMH*

John Niederhuber, *NCI*

Lawrence Tabak, *NIDCR*

FDA

ShaAvhree Buckman,

*Office of Translational Science, Center
for Drug Evaluation and Research*

Dan Schultz,

Center for Devices & Radiological Health

Janet Woodcock,

Center for Drug Evaluation and Research

Public Member

Mary Woolley, *Research!America*

CMS

Barry Straube

Industry

Stephen Eck, *Eli Lilly & Co.*

Garry Neil, *Johnson & Johnson*

Perry Nisen, *GlaxoSmithKline*

Sara Radcliffe, *BIO*

Foundation for NIH Board

Steve Paul, *Eli Lilly & Co.*

Ellen Sigal, *Friends of Cancer
Research*

The Biomarkers Consortium Steering Committee Co-Chairs

Cancer

- Anna Barker, National Cancer Institute
- Barbara Weber, GlaxoSmithKline

Inflammation and Immunity

- Daniel Rotrosen, National Institute of Allergy and Infectious Diseases
- Bruce Littman, Translational Medicine Associates (ex-Pfizer)

Metabolic Disorders

- Björn Carlsson, AstraZeneca
- Myrlene Staten, National Institute of Diabetes and Digestive and Kidney Diseases

Neuroscience

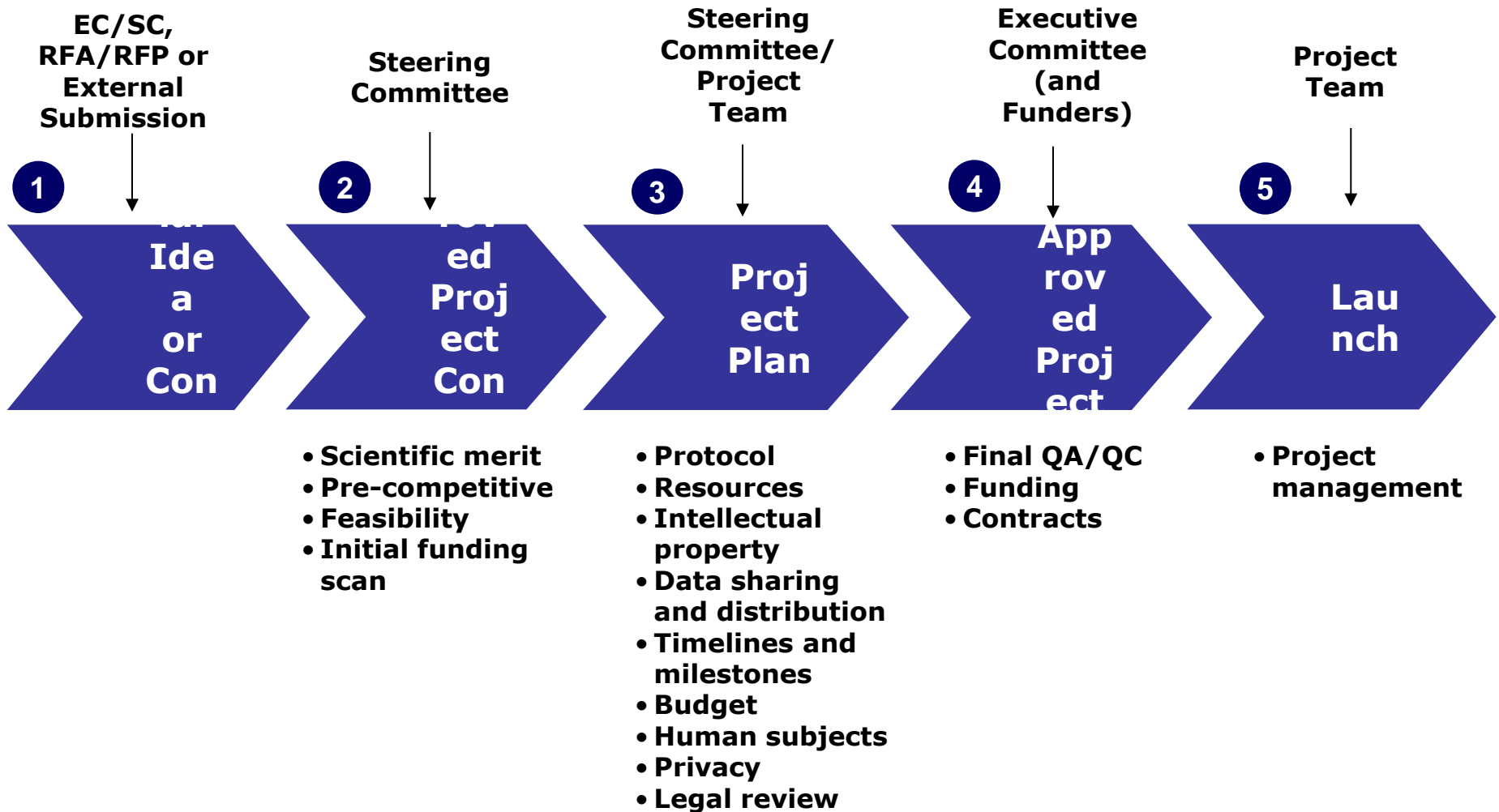
- Huda Akil, University of Michigan
- William Potter, Merck and Co., Inc.

2008 Approach: “High-Impact Biomarkers Opportunities”

Strategic focus on high impact areas of biomarker development and validation:

- ***Important:*** addresses a significant unmet/scientific need
- ***Translational:*** will result in significant improvement in the development, approval or delivery of care to patients
- ***Transformational:*** addresses critical gaps
- ***Feasible:*** end goals can be likely achieved in a specific timeframe
- ***Practical:*** leverages pre-existing resources wherever possible
- ***Fundable:*** is capable of generating the required funding/stakeholder support needed
- ***Unique:*** not already substantially being done elsewhere
- ***Collaborative:*** would uniquely benefit from the multi-stakeholder composition and approach of The Biomarkers Consortium

Project Development Process



Projects Launched To Date

FDG-PET Imaging in Non-Hodgkin's Lymphoma to Predict Tumor Response to Treatment (Cancer Steering Committee)

Phase II Study of FDG-PET/CT as a Predictive Marker of Tumor Response and Patient Outcome: Prospective Validation in Non-Small Cell Lung Cancer (Cancer Steering Committee)

The Utility of Adiponectin as a Biomarker of Glycemic Efficiency (Metabolic Disorders Steering Committee)

Carotid MRI Reproducibility Study (Metabolic Disorders Steering Committee)

Launched Projects Example: ***FDG-PET Lung and Lymphoma Projects***

Project Goals:

- Determine the linkage of FDG-PET to the effect of conventional cytotoxic drugs in clinical outcome and survival in lymphoma and lung cancer
- Determine the linkage of FDG-PET to the effect of drugs on clinical response and survival in lymphoma and lung cancer
- Develop standard protocols for acquiring and evaluating FDG-PET data
- Evaluate robustness and clinical feasibility of protocols

Timeline/Cost: 5 years / \$10.18M (launched in 2007)

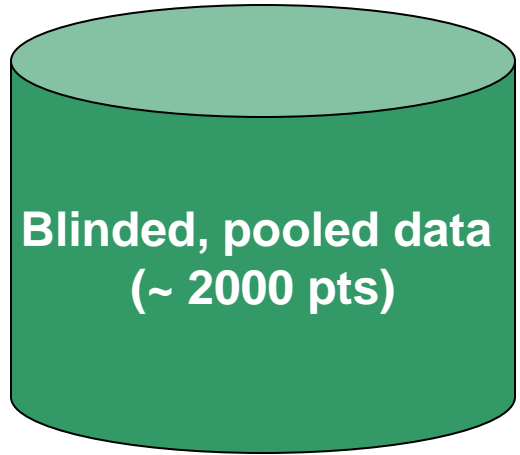
- Private sector: \$6.43M
 - Amgen, AstraZeneca, BMS, Genentech, GSK, J&J, Merck, Pfizer, Wyeth
- Government: \$3.75M
 - National Cancer Institute



Launched Project Example: *The Utility of Adiponectin as a Biomarker for Predicting Glycemic Efficacy*

De-identified clinical trials data

- GSK
- Lilly
- Merck
- Roche



Blinded, pooled data (~ 2000 pts)

[Quintiles]
[NIDDK]



Analysis

NIDDK



Analysis

Quintiles

Is there relationship between adiponectin and:
•glucose
•HbA1C levels?



Results Review

The Biomarkers Consortium Project Team



Results made public

Key Principles of The Biomarkers Consortium

- **The Biomarkers Consortium supports pre-competitive research**
- **Intellectual Property:**
 - **Contributed IP may be protected, remain property of contributing entity**
 - Project participants grant the BC licenses for research use within each project
 - IP developed by the Consortium should be placed in public domain as much as possible, with consideration for encouraging downstream development
- **Data Sharing:**
 - Source/raw data may be protected/kept confidential
 - Results data should be made broadly available as soon as possible after appropriate review
- **IP and Data Sharing are specified as part of each BC Project Plan**

Key Principles of The Biomarkers Consortium

- **Antitrust:** Competitively sensitive or commercial information should not be shared; FNIH retains antitrust counsel
- **Grants/contracts:** Selection and award of grants/contracts will be conducted to ensure fairness, impartiality and inclusiveness, as well as conformity with all BC policies.
- **Confidentiality:** Consortium participants certify that they will maintain the confidentiality of all confidential information and only use such confidential information in connection with the Consortium
- **Conflicts of Interest:** Funding members and participants must promptly disclose any current or potential conflict of interest; EC or FNIH board decides action
- **Data Quality, Privacy, Human Subjects:** are addressed within each Project Plan

The Biomarkers Consortium Policies

Key governing policies pre-negotiated prior to Consortium launch with principals/legal counsel representing the Foundation for NIH, NIH, FDA, PhRMA and BIO:

- Intellectual property and data sharing
- Antitrust
- Selection and award of grants/contracts
- Confidentiality
- Conflict of interest

All policies and other information available at

www.biomarkersconsortium.org

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