Partnerships: AMP and PACT
Francis S. Collins, M.D., Ph.D.
Director, National Institutes of Health
Advisory Committee to the Director
June 9, 2017
Drug Companies Join NIH in Study of Alzheimer's, Diabetes, Rheumatoid Arthritis, Lupus

Ten Drug Companies Form Pact With NIH to Find Paths to New Medicines

By MONICA LANGLEY and JONATHAN D. ROCKOFF
Feb. 3, 2014 11:00 p.m. ET

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.

In an unprecedented move designed to jump-start medical science, 10 rival drug companies that normally compete fiercely against each other will now cooperate, not just with government researchers and nonprofits, 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 and diabetes. This new public-private partnership – the Accelerating Medicines Partnership – innovation of our private sector complements our health challenges.

The Washington Post

Health & Science

NIH announces novel venture with drug companies to fight major diseases

By Ariana Sunjung 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 find new treatments to change the world.

Science Insider

NIH, 10 Drug Companies Partner to Study Four Diseases

By: Jennifer Nakash
Tuesday, February 4, 2014 - 3:46pm | 3 Comments

Ramping up its efforts in drug discovery, the National Institutes of Health (NIH) today unveiled what it called an unprecedented $200 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
AMP: Overview

- NIH partnered with FNIH, FDA, 10 biopharmaceutical firms, multiple non-profits (including patient advocacy groups), to:
  - Increase the number of new diagnostics, therapies
  - Reduce time, cost of developing them

- Investing $230M over five years on three pilot projects:
  - Alzheimer’s disease
  - Type 2 diabetes
  - Autoimmune disorders (rheumatoid arthritis and systemic lupus erythematosus)

- Project management provided by FNIH
AMP: Alzheimer’s Disease (AD)

$129.5M program to shorten time between discovery of potential drug targets and development of new AD drugs

- Biomarkers Into Clinical Trials

Distribution of tau across brain with AD
AMP: Alzheimer’s Disease (AD)

$129.5M program to shorten time between discovery of potential drug targets and development of new AD drugs

- Biomarkers Into Clinical Trials
- Target Discovery and Preclinical Validation
  - Human omics datasets from >2000 brains

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www.synapse.org.ampad

© AMP AD Knowledge Portal

ACCELERATING MEDICINES PARTNERSHIP (AMP)
AMP: Alzheimer’s Disease (AD)

$129.5M program to shorten time between discovery of potential drug targets and development of new AD drugs

- Biomarkers Into Clinical Trials
- Target Discovery and Preclinical Validation
  - Human omics datasets from >2000 brains
  - Network models of candidate targets generated

www.synapse.org.ampad
AMP: Type 2 Diabetes (T2D)

$59M program to link human genetic data on risk, protection for T2D & its complications with phenotypic data – to identify novel drug targets

- T2D Knowledge Portal
  - Tools allow easy, integrated interrogation across datasets

www.type2diabetesgenetics.org
AMP: Rheumatoid Arthritis and Systemic Lupus Erythematosus (RA/SLE)

$40M+ program to define pathways that identify drug targets for treatment of RA, SLE, related autoimmune diseases

- Example of results from SLE Phase I
  - Kidney biopsy single-cell RNAseq reveals immune cell clusters
AMP: Parkinson’s Disease (PD)

The newest kid on the block

Partners: GSK, MJFF, NINDS, Pfizer, Sanofi, Verily

- Goal: identify and validate diagnostic, prognostic, progression biomarkers
  - Will improve clinical trial design, contribute to identification of new pathways for therapeutic developments

- $20M Project aims to:
  - Standardize data collection for biomarkers in multiple cohorts
  - Conduct standardized assays on thousands of existing biosamples, incorporating existing clinical, imaging, genetic data
  - Pursue additional large-scale biomarker discovery with transcriptomics, epigenomics, whole genome sequencing, metabolomics, proteomics
  - Dissect new targets and disease subtypes; track, predict disease progression
Cancer Moonshot: Partnership for Accelerating Cancer Therapies (PACT)

- Planned public-private partnership between NIH, FDA, private sector, foundations, advocacy organizations
  - 14 companies have expressed interest in participation

- Potential focus areas
  - Identify, validate effective biomarkers for response and resistance to cancer therapies – especially immunotherapies
  - Establish uniform platform for selecting, testing combination therapies

- Proposed investment: ~ $250M over five years, shared between NIH, industry, philanthropy
Challenges in Developing Immunotherapy and Combination Therapies for Cancer

- Hundreds of existing trials:
  - Biomarkers to predict and understand patient outcomes needed
  - Robust, standardized assays required
  - Inadequate reproducibility of data across trials
  - Large number of potential combinations to be tested

- **Problem:** Knowledge gaps; need for efficient use of research resources

- **Solution:** A systematic effort to develop and share biomarker and related clinical data to support clinical testing of combination therapies = PACT
Partnership for Accelerating Cancer Therapies (PACT): Initial Design Effort

(Additional support provided by PhRMA)
PACT: Initial Focus on Two Programs

Program Area 1: Robust, systematic testing of biomarkers to better understand the response and resistance (especially to immunotherapy), and guide treatment strategies

- Establish network of 3-5 core laboratories to perform biomarker assays
- Fund exploratory biomarker and assay development
- Incorporate biomarkers into prioritized trials
- Create comprehensive database integrating biomarker and clinical data
PACT: Initial Focus on Two Programs

Program Area 2: Identify and co-fund key clinical combination therapy trials with partners

- Create, maintain “landscape analysis” of combination therapy trials, biomarkers
- Select, co-fund high relevance combination trials not already being performed by other entities
- Facilitate information sharing by all stakeholders, including outreach to outside research efforts to:
  - Align investigative approaches
  - Enable the most relevant trials to be conducted
PACT: 2017 Next Steps

Fundraising outreach

Jan | Feb | Mar | Apr | May | Jun | Jul | Aug | Sept | Oct | Nov | Dec

- Final whitepaper and budget distributed to design phase participants and key stakeholders
- Initiate Fundraising
- Potential public announcement of PACT?
- Estimated date for announcement of NCI PACT related awards
- Potential PACT launch?
What’s Next?
Case for a Public-Private Partnership for Pain and Opioid Use Disorders

- Urgent public health crisis
- Need for better alternatives for treatment of addiction
- Need for more potent treatments for overdose
- Absence of highly potent alternatives to opioids
- Emergence of numerous potential drug targets
- Possibility of development/validation of biomarkers for pain relief
- Strong support at highest level of U.S. Government
  - FDA highly motivated
  - Potential for additional industry incentives
Planning for Public-Private Partnership for Pain and Opioid Use Disorders

- Case made to heads of R&D of big pharma on April 8-9
  - Industry agreed to pursuit of partnership
- NIH convening series of cutting-edge science meetings
  - Experts from academia, industry, government
  - Goal: Identify new approaches/recruit additional expertise to develop safe, effective therapeutics for opioid abuse and pain in half the time it currently takes

- **June 5:** Medications Development for Opioid Use Disorders and for Overdose Prevention and Reversal
- **June 16:** Development of Safe, Effective, Non-Addictive Pain Treatments
- **July 7:** Understanding the Neurobiological Mechanisms of Pain
- Results will be used to design a possible partnership
Public-Private Partnerships: 
*Lessons Learned*

- Start by identifying a need that can’t be met readily by partners working alone
- Convene scientific experts from all relevant sectors to design a possible workplan
  - Requires leadership from relevant NIH Institute
  - Identify a few champions from industry
  - Include advocates, FDA
- Then call the question: who’s in?
- Then refine the workplan, develop milestones, deliverables, Go-NoGo decision points, detailed budget
- Needs an honest broker for both design and implementation phase: FNIH is highly effective in this role
- Needs engagement throughout the process from senior leadership
- Beware partnership fatigue!
NIH...  Turning Discovery Into Health

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