CRITICAL PATH INSTITUTE (C-PATH) & INNOVATIVE MEDICINES INITIATIVE (IMI) 2ND ANNUAL MEETING

ACCELERATING THE DEVELOPMENT OF DRUGS, DIAGNOSTICS, AND DEVICES: PARTNERSHIPS TO EXPAND THE PRECOMPETITIVE SPACE

December 3, 2014

Perspectives on Partnering for Global Health

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nature REVIEWS DRUG DISCOVERY

- The Driving Role of Consortia on the Critical Path to Innovative Therapies
  J. Woodcock, M. Brumfield, D. Gill, and E. Zerhouni, NRDD 13, 781 (2014)

- Paving the Critical Path of Development: The CDER Perspective
  J. Woodcock, NRDD 13, 783-784 (2014)
- The Critical Path Institute: Transforming Competitors into Collaborators *M. Brumfield, NRDD 13, 785-786 (2014)*
- Re-inventing Clinical Trials through TransCelerate *D. Gill, NRDD 13, 787-788 (2014)*
- The Biomarkers Consortium *D. Wholley, NRDD 13, 791-792 (2014)*
- The Predictive Safety Testing Consortium & the Coalition Against Major Diseases *D. Stephenson and J-M. Sauer, NRDD 13, 793-794 (2014)*

Accelerating the Development of Drugs, Diagnostics, and Devices: Partnerships to Expand the Precompetitive Space

AGENDA OVERVIEW

8:45 – 9:00	**Welcome: Martha Brumfield and Michel Goldman**
9:00 – 11:00	**Session 1: Partnerships to Advance Regulatory Science and Leverage Global Biopharmaceutical Development**
11:00 – 11:30	**Break**
11:30 – 1:00	**Session 2: Safety Biomarkers: The PSTC and SAFE-T Collaboration**
1:00 – 2:00	**Lunch on the Concours Terrace**
2:00 – 4:00	**Session 3: Maximizing the Value of Data Shared by Multiple Organizations**
4:00 – 4:30	**Closing Remarks: Key Messages and Identification of Key Next Steps**
4:30 – 7:00	**Reception (on Concours Terrace located on the Lobby Floor)**

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Session 1:

Partnerships to Advance Regulatory Science and Leverage Global Biopharmaceutical Development

A Vision for Future Collaborative Efforts

Collaboration across Consortia

- Benefits of a non-competitive workspace
- Combining forces across collaborative efforts

Global View

- Non US or non EU centric approach as drug development is global

Importance of FDA/EMA Participation

- Connect needs of development and regulatory science

Proactive Approach to Data Sharing

- Up front collaboration on how data are collected, generated, shared

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Session 1: Partnerships to Advance Regulatory Science and Leverage Global Biopharmaceutical Development (9:00 am – 11:00 am)

Co-chairs/Moderators:	Martha Brumfield (C-Path) and Michel Goldman (IMI)
Panelists:	**William Chin (PhRMA)** **Dalvir Gill (TransCelerate BioPharma Inc)** **David Wholley (FNIH)** **Janet Woodcock (FDA)**
Panel Discussion Topics:	‣ What have partnerships produced that could not have been accomplished by a single organization? ‣ What metrics should be applied to evaluating partnerships? ‣ What are the key challenges facing todays' partnerships, and how can those challenges be optimally addressed? ‣ What factors should be considered when partners prioritize projects? ‣ How can newly formed partnerships leverage ongoing efforts of established partnerships? ‣ How to ensure coordination?

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An Overview of TransCelerate Biopharma Inc.

Dalvir Gill, PhD - Chief Executive Officer

03 December, 2014

TransCelerate BIOPHARMA INC.

ACCELERATING THE DEVELOPMENT OF NEW MEDICINES

Our Purpose

TransCelerate is a Not For Profit Entity Created To Drive Collaboration

History

Launched in 2012, TransCelerate evolved from discussions at various forums for executive R&D leadership to debate issues facing the industry, and examine solutions for addressing agreed-upon common challenges.

Vision

To improve the health of people around the world by accelerating and simplifying the research and development of innovative new therapies.

Mission

To collaborate across the global research and development community to identify, prioritize, design and facilitate implementation of solutions designed to drive the efficient, effective and high quality delivery of new medicines.

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Our Members

19 Pharmaceutical Companies* providing their best talent to collaborate and develop solutions to overcome industry Inefficiencies

ALLERGAN
Actavis
astellas
abbvie
AstraZeneca
ucb
biogen idec
SHIONOGI
Boehringer Ingelheim
SANOFI
Bristol-Myers Squibb
CUBIST
Roche
gsk GlaxoSmithKline
Pfizer
Johnson & Johnson
Merck Serono
Lilly
Medgenics

** 10 Pharmaceutical Companies chartered TransCelerate and 9 additional companies joined in 2013*

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Our Industry Position

TransCelerate is often a "catalyst" to open the doors for collaboration amongst industry groups outside of TransCelerate

The intent is not to recreate, but partner whenever feasible

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Our Projects

TransCelerate currently has 11 projects which share the goals of increasing quality, patient safety & accelerating development timelines

1. **Model Approach for High-Quality, Risk-Based Monitoring***
2. **Shared Site Qualification and Training***
3. **Shared Investigator Platform***
4. **Clinical Data Standards – Efficacy** *(in partnership with CFAST)**
5. **Comparator Drugs for Clinical Trials***
6. **Common Protocol Template**
7. **Investigator Registry**
8. **Pediatric Trial Efficiencies**
9. **Clinical Trial Diversification**
10. **Clinical Data Transparency**
11. **Quality Management System**

****Original initiative which began in 2012***

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TransCelerate BIOPHARMA INC.

ACCELERATING THE DEVELOPMENT OF NEW MEDICINES

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FNIH

Foundation for the
National Institutes of Health

David Wholley
Director, Research Partnerships

Critical Path Institute-IMI Joint Meeting
December 3, 2014

FNIH

Building partnerships for discovery and innovation to improve health.

Purpose

Created by Congress:

→ To support the NIH in its mission;

→ To advance collaboration with biomedical researchers from universities, industry and not-for-profit organizations.

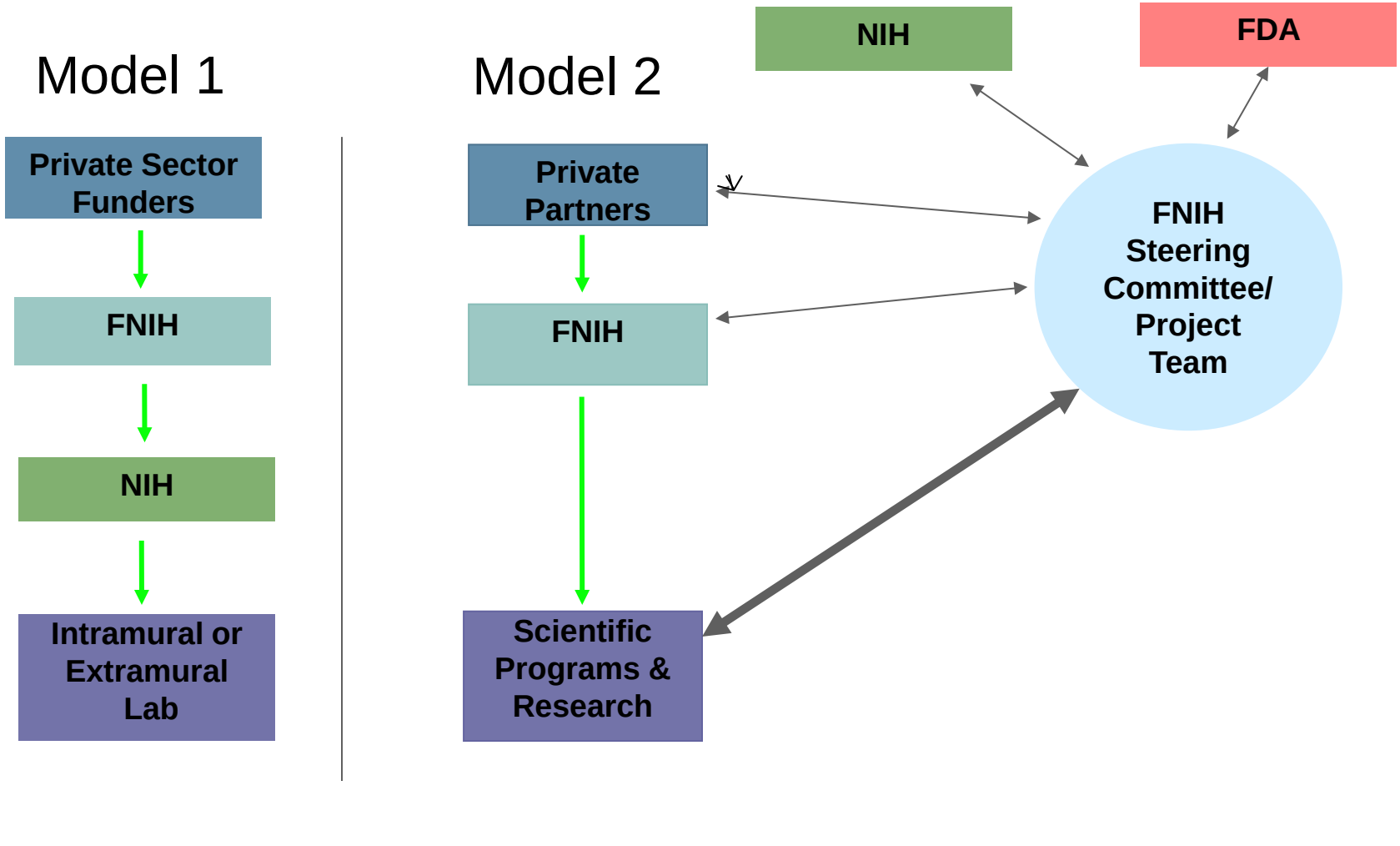
Structure

- 501(c)(3) not-for-profit organization;
- Independent Board of Directors;
- NIH Director and FDA Commissioner *ex-officio* Board Members

Highlights

- Raised over $800 million since 1996;
- Supported over 500 projects;
    - research partnerships
    - scientific education/training
    - conferences/events
    - capital programs

Funding & Partnership Models



Select Programs

FNIH
Foundation for the National Institutes of Health

VCTR
Vector-based Control of Transmission: Discovery Research
Grand Challenges in Global Health

LUNG-MAP

THE biomarkers CONSORTIUM
www.biomarkersconsortium.org

ADNI

AREDS 2
Age-Related Eye Disease Study 2
Funded by the National Institutes of Health

SPIROMICS
SUBPOPULATIONS AND INTERMEDIATE OUTCOME MEASURES IN COPD STUDY

CTVIMC

Accelerating Medicines Partnership

FNIH
Foundation for the National Institutes of Health

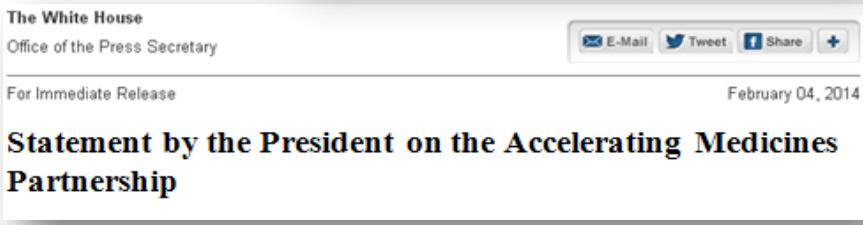
- Public-private partnership with NIH, 10 companies, non-profits
- Aims to distinguish targets of disease most likely to respond to new therapies in:
  - Alzheimer's Disease
  - Type 2 Diabetes
  - RA/SLE
- Launched in 2014, $230M investment over five years on pilot projects
- FNIH secures private sector funding, establishes governance, and provides program and project management for AMP.


The Washington Post | PostTV | Politics | Opinions | Local | Sports | National

Health & Science

NIH announces novel venture with drug companies to fight major diseases

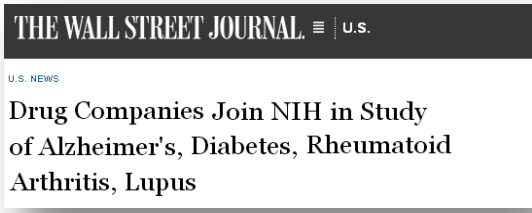
By Ariana Eunjung Cha, Published: February 4


The White House
Office of the Press Secretary

E-Mail | Tweet | Share | +

For Immediate Release February 04, 2014

Statement by the President on the Accelerating Medicines Partnership


THE WALL STREET JOURNAL. | U.S.

U.S. NEWS

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


CNN Health

February 4th, 2014
02:41 PM ET

NIH, drug companies team up to target diseases

The National Institutes of Health is partnering with researchers from 10 rival drug companies and several nonprofit organizations to develop new and earlier treatments for diseases including diabetes, Alzheimer's and lupus.

The partnership, announced Tuesday by NIH director Dr. Francis Collins, "could change the way scientific research is conducted."

AMP T2D Information Portal

Datasets of interest

Project	Description	Ethnicity	Sample Size	Status
CHARGE	Cohorts for Heart and Aging Research in Genomic Epidemiology	multi-ethnic	38,000	Aspirational
DIAGRAM	Diabetes Genetics ; T2D, QTs	European	34,840 cases 114,981 controls	Discussions initiated
FIND	Nephropathy and Diabetes; familial	Mexican/African/ Native American	2,622 genotype/ phenotype	Discussions initiated
GoT2D	T2D Genetics; T2D, QTs	European	3,000 whole genome/ exomes/SNP arrays	Some in portal
IMIDIA	IMI for Diabetes; Human islet cells	European	465; 93 T2D	Discussions initiated
MAGIC	Glucose and Insulin-related traits ; glycemic/ metabolic traits	European	133,010 GWAS	Discussions initiated
NHLBI ESP	Exome Sequencing; T2D, QTs	multi-ethnic	6,800 exomes; ~1,000 T2D	In portal
SIGMA	Genomic Medicine for the Americas; T2D, QTs	Mexican/Latin American	4,200 exomes	Permission granted; data being collected
SUMMIT	IMI Surrogate markers for Micro- and Macro-vascular endpoints; diabetic complications	European	10,000 GWAS/exomes	Discussions initiated
T2D GENES	T2D Genetics Next-generation sequencing ; T2D, some QTs	multi-ethnic	100,000: ~250 genes, 9,000 SNPs; 600 WGS; 11,000 exomes	In portal
TODAY	Treatment Options for T2D in Adolescents and Youth; T2D 10 -17 year olds with treatment	Caucasian/African/Hispanic American	~ 700	Some in portal

Biomarkers Consortium

FNIH
Foundation for the National Institutes of Health

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

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

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

Biomarkers Consortium Kidney Safety Project

THE biomarkers CONSORTIUM
www.biomarkersconsortium.org

$4M; 4-year project : Amgen, AZ, FDA, FNIH, J&J, Lilly, Merck, NIDDK, Pfizer, PSTC

Healthy Volunteer Biomarker Analysis

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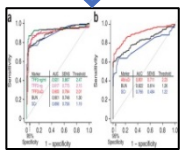
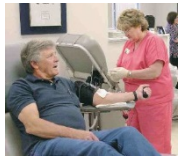


Retrospective Analysis

MERCK & FNIH Foundation for the National Institutes of Health



Prospective Analysis (2 Clinical Studies)

FNIH Foundation for the National Institutes of Health

Sample Bank & Database

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FDA

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Publications, Regulatory Submissions

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&
FNIH Foundation for the National Institutes of Health

Funds raised: 80% FNIH; 20% PSTC

Lead PI: Frank Sistare, Merck

Developing a Common Regulatory Strategy with the IMI SAFE-T Consortium

FNIH
Foundation for the National Institutes of Health

Timeline and List of Joint Activities

- Q2 2012 – Joint F2F Meeting between the IMI SAFE-T Team leaders and select Biomarkers Consortium Kidney Safety/PSTC Project members in Paris during the European Renal Annual Meeting
- Q4 2013 – Joint CDA executed
- Regular calls in 2014 to develop a joint regulatory strategy
- IMI SAFE-T representative invited to attend FNIH/PSTC FDA/EMA meeting discussions
- Shared documents:
  - List of biomarkers considered for submission for qualification
  - All regulatory feedback received
  - Context of Use Statement
  - Statistical analysis plans