High Hurdles

Productivity Decline

- Costs rising faster than launches; cost/time
- Significant patent expirations
- Innovation has been incremental; defined by payors
- Pricing pressures based on generic and other considerations; SOC; CER; HTA
- Regulatory expectations are increasing; higher bars for efficacy and safety
- Personalized medicine
Problem

The promise of genomics. Not yet fulfilled.

Complexity of disease:

Target/pathway biology is complex.
Disease definition is imprecise.
A disease often has multiple causes.
Drugs work with multiple mechanisms.
Patients with the same disease are different.

Patients and clinical samples define relevance.

Translation science and medicine: key roles.
Shifting Knowledge Curves

(after Pisano)
Solution

Create an environment that fosters innovation.

• Steven Johnson: “Reef, city and Web.”
• Ecosystem of academia, industry, government, NGO.
• Private-public partnerships.
• Need for “honest broker” or third party convener.
“I see by the current issue of ‘Lab News,’ Ridgeway, that you’ve been working for the last twenty years on the same problem I’ve been working on for the last twenty years.”
Key challenges faced in public-private partnerships

◆ **Education/Culture**; lack of expertise and experience; applied science as a pejorative in academia [not ACTS].
◆ **Alignment of goals** among academia, industry government, and non-governmental organizations; need for academia to publish and communicate freely.
◆ **Reward and recognition**; not optimal to promote team efforts.
◆ **Intellectual property**; assiduous and poorly prioritized efforts to protect IP may serve to limit collaborative opportunities; tech transfer office—friend or foe?
◆ **Conflicts of interest concerns**; may limit some participation.
COMMENTARY

DRUG DISCOVERY

A Call for Sharing: Adapting Pharmaceutical Research to New Realities

Bernard H. Munos* and William W. Chin

Published 2 December 2009; Volume 1 Issue 9

From the dawn of time, the sharing of knowledge has been one of the main forces driving science and innovation. Yet in recent decades, a proprietary culture, which wrongly posits that all intellectual property must be restricted, has spread across the pharmaceutical industry and threatens to stall the engine that has given us so many valuable treatments. This paper argues that pharmaceutical companies, together with universities and government agencies, stand to gain much from reversing that trend and engaging in widespread collaboration early in the research process to expand foundational knowledge and create a shared infrastructure to tap it.
Sustainable Innovation in Challenging Times

◆ Expansion of the knowledge market is required due to novelty and complexity of diseases.
◆ Collaborative consortia are necessary to improve flow of information and consequent innovation. Open innovation models.

Being competitive.

✓ Understand need for incremental innovation.
✓ Expand access to information.
✓ Improve utilization of knowledge markets.
✓ Match work with current budget realities.
✓ Marketplace rewards are needed.
✓ Influence changes in policies that limit innovation.
What is a precompetitive consortium?

A collaborative partnership that focuses on work that increases the overall knowledge or technology base in a field, shares risks and costs, reduces duplication of effort and resource expenditure in that work, and does not ordinarily provide a competitive advantage to a specific company. The output of the consortium may be restricted or public. Unlike the competitive space, such a consortium engages in areas that are shared challenges important for progress usually involving novel and/or highly complex problems, requires a longer time-frame for resolution, and generally cannot be performed by one company.
Overlap of pre-/procompetitive and competitive spaces
## Eight models of precompetitive collaboration

### Collaboration goals:

<table>
<thead>
<tr>
<th>Build enabling platforms</th>
<th>Generate/aggregate data</th>
<th>Conduct research</th>
<th>Develop a product</th>
</tr>
</thead>
<tbody>
<tr>
<td>Develop standards/tools</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Linux</td>
<td>Crystallography OD</td>
<td></td>
<td>Pink Army Coop</td>
</tr>
<tr>
<td>Wikipedia</td>
<td>PatientsLikeMe</td>
<td></td>
<td>India OSDD</td>
</tr>
<tr>
<td>Synaptic Leap</td>
<td>Sage</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Open Health NLP</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

#### 1. Open source initiatives

<table>
<thead>
<tr>
<th>CDISC</th>
<th>HGP</th>
<th>Biomarkers Consort</th>
</tr>
</thead>
<tbody>
<tr>
<td>Pistoia</td>
<td>Alliance for Cell Sig</td>
<td>Diabetes Genetics Init</td>
</tr>
<tr>
<td>C-Path</td>
<td>SNP Consortium</td>
<td>Innovative Meds Init</td>
</tr>
<tr>
<td></td>
<td>HapMap</td>
<td>CCMX</td>
</tr>
<tr>
<td></td>
<td>RNAi</td>
<td>SAEC</td>
</tr>
<tr>
<td></td>
<td>Signaling Gateway</td>
<td></td>
</tr>
</tbody>
</table>

#### 4. Public-private consortia for knowledge creation

#### 5. Prizes

<table>
<thead>
<tr>
<th>Prize4Life</th>
<th>X Prize Genomics</th>
<th>InnoCentive</th>
<th>Netflix Prize</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>P&amp;G Connect/Develop</td>
<td>Biogen b²</td>
<td>Siemens Tech to Bus</td>
</tr>
<tr>
<td></td>
<td>Merck-AZ</td>
<td>Pfizer-GSK</td>
<td>MMRF</td>
</tr>
<tr>
<td></td>
<td>CHDI</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

#### 2. Industry consortia for process innovation

<table>
<thead>
<tr>
<th>Sematech</th>
<th>CERN</th>
<th>Fermilab</th>
<th>SLAC</th>
</tr>
</thead>
</table>

#### 3. Discovery-enabling consortia

<table>
<thead>
<tr>
<th>AltschulerGray</th>
</tr>
</thead>
</table>

#### 6. Innovation incubators

#### 7. Industry complementors

#### 8. Virtual pharma companies

<table>
<thead>
<tr>
<th>Academic / public only</th>
<th>Academic / industry</th>
<th>Industry only</th>
<th>Foundation</th>
</tr>
</thead>
</table>

- Participant/beneficiaries:
  - Open participation
  - Restricted participation
  - Open output
  - Restricted output
Many examples of precompetitive or open-source consortia in pharma/academia.

Innovative Medicines Initiative
Biomarkers Consortium; HGP
Predictive Safety Testing Consortium; C-Path
Alzheimer’s Disease NeurolImaging (ADNI)
SNP/HapMap Consortia; 1000 Genomes Project
Health and Environmental Sciences Institute
Serious Adverse Events Consortium
Drug Safety Executive Council
Dundee Signaling Consortium
Animal Models Consortium
Health Commons; Enlight; Structural Genomics
Genetic Association Information Network
Alzheimer’s Disease Neuroimaging Initiative (ADNI)

Source: [http://adni.loni.usc.edu/](http://adni.loni.usc.edu/)
Partnership formation has accelerated over the past decade

New partnerships formed over time

Number of partnerships (n=62)$^1$

<table>
<thead>
<tr>
<th>Year Range</th>
<th>IMI</th>
<th>NCATS</th>
<th>CPI</th>
<th>FNIH</th>
</tr>
</thead>
<tbody>
<tr>
<td>1996-1999</td>
<td>4</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>2000-2003</td>
<td>3</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>2004-2007</td>
<td>7</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>2008-2011</td>
<td>42</td>
<td>17</td>
<td>1</td>
<td>1</td>
</tr>
</tbody>
</table>

$^1$ Excludes 2 partnerships that were formed before 1996, 6 partnerships formed in 2012, and 6 partnerships where founding year unknown

$^2$ Includes 2 partnerships in Asia and 1 partnership in Canada

~50% of partnerships founded after 2007 were supported by umbrella organizations

United States
24 partnerships

Europe
31 partnerships

Global/Other
21 partnerships$^2$

SOURCE: Partnership websites; literature search; press search
Public-private partnerships have become an increasingly popular topic in the academic literature.

Mentions of public-private partnerships in PubMed

<table>
<thead>
<tr>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>Total¹</td>
<td>23</td>
<td>60</td>
<td>88</td>
<td>139</td>
</tr>
<tr>
<td>R&amp;D²</td>
<td>1</td>
<td>7</td>
<td>17</td>
<td>23</td>
</tr>
</tbody>
</table>

Public-private partnerships in pharma R&D are part of a broader cross-industry trend toward greater collaboration.

1 Search terms include: ("public private partnerships" OR "public-private partnerships")
2 Search terms include: ("public private partnerships" OR "public-private partnerships") AND (pharmaceutical AND (research OR development)) OR "drug discovery"

SOURCE: pubmed.gov
Umbrella organizations have supported over 20 new partnerships in the past 5 years

<table>
<thead>
<tr>
<th>NCATS</th>
<th>• Description</th>
<th>• Founded</th>
<th>• Sample partnerships</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>NIH center established to catalyze the creation of innovative methods and technologies that will enhance the development of diagnostics and therapeutics across multiple diseases</td>
<td>2011</td>
<td>Target Validation Consortium, Rescuing and Repurposing Drugs</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>REAGAN – UDALL FOUNDATION FOR THE Food and Drug Administration</th>
<th>• Description</th>
<th>• Founded</th>
<th>• Sample partnerships</th>
</tr>
</thead>
<tbody>
<tr>
<td>Not-for-profit organization created by Congress to advance the mission of the FDA by advancing regulatory science and research</td>
<td>2007</td>
<td>Systems Toxicology Project, Critical Path to Tuberculosis Drug Regimes</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>CRITICAL PATH INSTITUTE</th>
<th>• Description</th>
<th>• Founded</th>
<th>• Sample partnerships</th>
</tr>
</thead>
<tbody>
<tr>
<td>Independent nonprofit institute founded by the University of Arizona and the FDA</td>
<td>2005</td>
<td>Predictive Safety Testing Consortium (PSTC), Coalition Against Major Diseases (CAMD)</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>FNIH</th>
<th>• Description</th>
<th>• Founded</th>
<th>• Sample partnerships</th>
</tr>
</thead>
<tbody>
<tr>
<td>Nonprofit that raises private sector funds to stimulate and facilitate the formation of public-private partnerships with the NIH</td>
<td>1996</td>
<td>Biomarkers Consortium, OMOP</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>TI Pharma</th>
<th>• Description</th>
<th>• Founded</th>
<th>• Sample partnerships</th>
</tr>
</thead>
<tbody>
<tr>
<td>Dutch nonprofit formed to establish, support and manage public-private collaborations between academia and the pharmaceutical industry</td>
<td>2006</td>
<td>Medicines for Malaria Venture</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>IMI</th>
<th>• Description</th>
<th>• Founded</th>
<th>• Sample partnerships</th>
</tr>
</thead>
<tbody>
<tr>
<td>€2 billion budget funded half by the EU and half by EFPIA</td>
<td>2004</td>
<td>Preeduct, EuroPain, NewMed s, SUMMIT, SAFE-T, eTOX</td>
<td></td>
</tr>
</tbody>
</table>

SOURCE: Partnership websites; press releases
Relative impact across a subset of selected partnerships

<table>
<thead>
<tr>
<th>Types of impact</th>
<th>Data consolidation</th>
<th>Publications</th>
<th>Recommendations</th>
<th>Regulatory changes</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Low</td>
<td>Low</td>
<td>Low</td>
<td>Low</td>
</tr>
<tr>
<td></td>
<td>Low</td>
<td>High</td>
<td>Low</td>
<td>Low</td>
</tr>
<tr>
<td></td>
<td>Low</td>
<td>Low</td>
<td>High</td>
<td>Low</td>
</tr>
<tr>
<td></td>
<td>Low</td>
<td>Low</td>
<td>High</td>
<td>High</td>
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<td>Low</td>
<td>Low</td>
<td>Low</td>
<td>High</td>
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<tr>
<td></td>
<td>Low</td>
<td>Low</td>
<td>High</td>
<td>Low</td>
</tr>
</tbody>
</table>

A relative impact scale was developed to assess changes in regulatory approach and three other dimensions based on publicly available data and expert interviews.

- We mapped the impact of eight partnerships with this method, and have qualitative data behind each assigned value.

- Key takeaways from this analysis are that partnerships:
  - Have made early steps along each of these dimensions,
  - May or may not have prioritized impact on regulatory changes.

SOURCE: Partnership websites; literature search; press search
Innovative Therapeutics
Discovery and Development Ecosystem

Network Space
Interaction Space

Patient Groups
Industry
Academy
FDA, NIH
Payors
Health Care Providers

New Therapy
Accelerating Medicines Partnership (AMP)

NIH, fNIH, Industry/PhRMA, NGO
Accelerating Medicines Partnership: A public-private collaboration with industry and NIH to advance understanding of disease targets

Problem

We do not sufficiently understand the targets/pathways involved in diseases – leading to R&D investment against the wrong targets

No single group is positioned to change this efficiently and quickly

– Scale is beyond that achievable even by large academic labs
– Benefits are too diffuse for any one pharmaco to pursue
– Necessary skills span these groups

Solution

Systematic characterization of complex, heterogeneous diseases, combining clinical & molecular information to:

– Facilitate rational selection of targets
– Identify patients and specific subpopulations for trials and customized prophylaxis, diagnosis and treatment decisions

Work collaboratively across government, academia and industry to harness collective capabilities, scale & resources

© The Boston Consulting Group, Inc.
### AMP research topics covered by program

<table>
<thead>
<tr>
<th>Disease area</th>
<th>Research plan topics</th>
<th>Deliverables and approach</th>
</tr>
</thead>
<tbody>
<tr>
<td>Alzheimer's disease</td>
<td>Exploratory biomarker validation in clinical trials and network analysis on human tissue</td>
<td>• Embed (ie, incentivize the use of) exploratory biomarkers in upcoming clinical trials to develop biomarkers of disease progression and surrogate endpoints&lt;br&gt;• Conduct network analysis in human brain samples to identify genetic nodes &amp; networks linked to AD to support target identification &amp; validation</td>
</tr>
<tr>
<td>Type 2 Diabetes</td>
<td>Sequencing &amp; phenotyping of targets of interest and a tool to enable easy interrogation of all available data</td>
<td>• Create a knowledge portal containing comprehensive T2DM (&amp; diabetic complications) genotype/phenotype data sets – apply informatics to identify predictors of risk and potential drug targets&lt;br&gt;• Conduct targeted sequencing/genotyping of high priority targets of interest (as defined by industry)</td>
</tr>
<tr>
<td>RA, SLE &amp; related autoimmune diseases</td>
<td>Immune module deconstruction with blood/tissue and cross-disease comparisons</td>
<td>• Conduct extensive profiling of key immune modules 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. The data will be made available in a knowledge portal&lt;br&gt;- Informative cohorts include: Early RA, Established RA (responder/non-responder), Lupus Nephritis, Skin Lupus</td>
</tr>
</tbody>
</table>

**Funding for AMP projects will be split 50/50 between industry participants and NIH**
AMP Participation by Disease Area

**Alzheimer’s disease**
- AbbVie
- Biogen Idec
- GlaxoSmithKline
- Lilly

**Type 2 Diabetes**
- Johnson & Johnson
- Lilly
- Merck
- Pfizer
- Sanofi

**RA, SLE & related**
- AbbVie
- Bristol-Myers Squibb
- MERCK
- Pfizer
- Sanofi
- Takeda

**Industry members**

**Government members**
- NIH National Institute on Aging
- NIH National Institute of Neurological Disorders and Stroke
- NIH National Institute of Diabetes and Digestive and Kidney Diseases

**Non-profit members**
- Alzheimer’s Association
- Geoffrey Beene
- LUPUS Foundation of America
- Rheumatology Research Foundation
- Alliance for Lupus Research
- BioMarkers Consortium
- FNIH
THE PLATFORM
An environment where the research community can broadly share and analyze historical, patient-level, cancer phase III comparator arm data

THE PURPOSE
To advance future research in an effort to improve the lives of patients and their families around the world

www.projectdatasphere.org

[Industry]
Patient Powered Drug Development

Genetic Alliance [NGO],
in partnership with PhRMA [Industry]
The Problem

From social networks to online banking, the Internet has made it easy to share and connect with the people we care about, and to keep track of the information that helps our lives run smoothly.

Why isn’t there a safe place to keep track of our health information online?

And why can’t we safely share it with the medical and research community - the people who can advance health for all of us?

Learn More
Virtually every article on Big Data, every policy body, every medical research project is looking for a solution to the question: “How can we share the clinical and genetic data of millions of individuals and still respect their diverse wishes?”

**Conditions Under Which the Public is Willing to Have their Data Used for Health Research**

Dr. Alan F. Westin, Institute of Medicine (2009)

- **48%**: Want each study seeking to use my data to contact me in advance and to get my specific consent each time...
- **16.5%**: Would not want researchers to contact me or to use my data under any circumstance...
- **24%**: Consent is not needed if my identity will never be revealed and the study is IRB supervised...
- **10%**: Willing to give general consent in advance for use of my data without being contacted...
- **1.5%**: Okay for researchers to use my data without my consent at all...

*Percentages shown reflect the views of those persons expressing an opinion. An additional 20% of the persons surveyed indicated that they were “Not sure.”*
Participants use privacy settings to specify who can, and cannot, access or use their data, and for what purpose.

“Discover” Each participant controls who can locate his or her de-identified data...

“Contact” Who can have access to the user’s contact information

“Export” Who can export the user’s de-identified data

Participants may choose to Permit, Decline, or wait for more information before deciding...

So long as the data are held by PEER or a system that adheres to the person’s Private Access settings.

And may change these preferences over time.

Participants use privacy settings to specify who can, and cannot, access or use their data, and for what purpose.

For multiple categories of uses, and specified usage rights.
Sickle Cell PFDD Portal Example

The PEER portal can be placed in an iFrame directly onto the group’s website. The group’s selected logo art and call-to-action headlines and text appear fonts, background colors and links that express its theme. The opportunity to select from existing personalities or to substitute the group’s leadership and key trusted members of its community. Flexibility to present the steps in the way the group feels will have the greatest appeal to its users. PEER network’s unique participant-centric Privacy Policy and Terms of Use automatically extend to use on the site and are easily accessible. PEER provides a complete stats package to see what outreach strategies are working. Useful on any website or blog.
<table>
<thead>
<tr>
<th></th>
<th>FDA Docket</th>
<th>PEER Sickle Cell Communities</th>
<th>% Increase</th>
</tr>
</thead>
<tbody>
<tr>
<td># of Respondents</td>
<td>31</td>
<td>129</td>
<td>516%</td>
</tr>
<tr>
<td># of Questions Answered</td>
<td>Unknown</td>
<td>20,438</td>
<td>N/A</td>
</tr>
<tr>
<td>Race</td>
<td>Unknown</td>
<td>90% African American 10% Caucasian</td>
<td>N/A</td>
</tr>
<tr>
<td>Age</td>
<td>Unknown</td>
<td>14-19: 14% 20-24: 12% 25-29: 12% 30-34: 12% 35-39: 12% 40-44: 6% 45-49: 8% 50-54: 2% 55-59: 14% 60-64: 8%</td>
<td>N/A</td>
</tr>
<tr>
<td>Genotype</td>
<td>Unknown</td>
<td>HbAS: 37% HbSS: 43% HbS/B Th: 6%</td>
<td>N/A</td>
</tr>
</tbody>
</table>

- HbSC: 11% HbEE: 1% HbSF: 1%
Collaboration to Enhance Efficiency of Clinical Trials

Transcelerate [Industry]
Pharmaceutical R&D leaders identified collaboration as a key opportunity for generating industry-wide efficiencies.

Conducted an industry survey on areas amenable to collaboration.
An Entity that Engages with the Wider Clinical Ecosystem Globally

Strategically focusing engagement efforts with selected key stakeholder groups

The intent is not to recreate, but partner whenever feasible
Five opportunities were chosen for action based on industry readiness and ability to execute in 2013

### Prioritized Near Term Opportunities

<table>
<thead>
<tr>
<th>#</th>
<th>Project Name</th>
<th>Objective</th>
<th>Benefits</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>Model Approach for High-Quality, Risk-Based Monitoring</td>
<td>Develop Guidelines for targeted, risk based clinical trial monitoring</td>
<td>Improvement in data quality and patient safety for clinical trials; reduction in effort expended on low-value activities</td>
</tr>
<tr>
<td>2</td>
<td>Shared Site Qualification and Training</td>
<td>Program established for mutual recognition of GCP training and site qualification credentials</td>
<td>Realization of improved quality of clinical sites and accelerated study start-up times</td>
</tr>
<tr>
<td>3</td>
<td>Common Investigator Site Portal</td>
<td>Establish a single, intuitive interface for investigators use across the industry</td>
<td>Ease of use and harmonized delivery of content and services for investigators</td>
</tr>
<tr>
<td>4</td>
<td>Clinical Data Standards – Efficacy <em>(in partnership with CDISC)</em></td>
<td>Accelerate current efforts underway through CDISC to establish efficacy data standards</td>
<td>Increased quality of clinical data and enablement of industry end-to-end data flow</td>
</tr>
<tr>
<td>5</td>
<td>Comparator Drugs for Clinical Trials</td>
<td>Establish a supply network to source comparator drugs between companies for use in clinical trials</td>
<td>Enhanced patient safety due to known product source and acceleration of study timelines</td>
</tr>
</tbody>
</table>

Projects have the shared goals of increased quality, patient safety & accelerated development timelines
Diversity in Clinical Trial Advocacy

Launch of “I’m in” Campaign and Clinical Trial Engagement Network

Collaborative Partnership between NMQF and PhRMA
Participation and Retention in Clinical Trials is Particularly Challenging among “Minorities”

- African Americans represent 12% of the U.S. population but only 5% of clinical trial participants.
- Hispanics make up 16% of the population but only 1% of clinical trial participants.
- By 2020, “minorities” are projected to account for over 40% of the nation’s population.
- By 2050, Hispanics will make up to 29% of the U.S. population (emerging “majority”).
A partnership to increase diversity in clinical trials by raising awareness and connectivity among patients, sponsors, investigators, healthcare providers, their institutions and advocacy organizations across the nation.
The Campaign

EDUCATE audiences about the importance of participation and diversity in clinical trials

ENGAGE traditional and non-traditional allies, patient and provider organizations, research institutions to share the story

ENHANCE participation by underrepresented populations in clinical trials.
Are you in?
Without the patients who volunteer to participate in clinical trials, the development of new treatments would not be possible.

JOIN THE CAMPAIGN

I'm a Participant...
Each day, new treatments are being developed to address the health challenges facing the African American, Asian American, and Hispanic communities. Unfortunately, these communities are underrepresented in the clinical trials that test the effectiveness of treatments. We can change that. Are you in?
Clinical Trial Engagement Network (CTEN)

A community-centered physical and virtual network connecting patients and their advocacy organizations, research sponsors and investigators, healthcare providers and points of medical care to facilitate and accelerate diversity in participation to clinical trials:

- Built around data warehouse, analytic and communications systems
- Helps engage diverse populations who share a common disease experience
- Facilitates communications between researchers and community providers who serve those populations
The Clinical Trial Engagement Centers (CTECs)

Points of care located in diverse communities with access to large populations underrepresented in clinical trials:

• Capacity to conduct clinical trials or commitment to build capacity
• History of active engagement with their surrounding community
• Commitment to engage in campaign and contribute data (as appropriate) to the CTEN
Advocacy
Local Engagement

The I’m In campaign is a national effort, with targeted regional outreach in pilot locations with a large number of key demographic groups which are currently underrepresented in clinical trials.

Pilot markets include:

**African American**
- Atlanta, GA
- Charleston, SC

**Asian American**
- San Francisco, CA
- Seattle, WA

**Hispanic**
- Edinburgh/Houston, TX
- Miami, FL
Responsible Clinical Trial
Data Sharing

PhRMA/EPFIA
Sources of Clinical Trial Information

• Medical literature / meetings
• Approved labeling
• Clinical Trials.gov
  ✓ Registry (upon recruitment)
  ✓ Results summaries
• Industry guidelines
  ✓ PhRMA Clinical Trial Principles (2002, 2009)
  ✓ PhRMA-EFPIA Data Sharing Principles (2013)
The PhRMA-EFPIA Principles supplement existing industry commitments to share clinical trial information as well as government requirements to register and post results.

Benefits of industry-wide data sharing commitments tailored to researchers, physicians, and patients

Safeguards to protect patient privacy, integrity of regulatory system, and incentives for investment in biomedical research
Principles for Responsible Clinical Data Sharing
Our Commitment to Patients and Researchers

Biopharmaceutical companies are committed to enhancing public health through responsible sharing of clinical trial data in a manner that is consistent with the following Principles:

- Safeguarding the privacy of patients
- Respecting the integrity of national regulatory systems
- Maintaining incentives for investment in biomedical research
PhRMA-EFPIA Principles: A Tailored Approach to Transparency

• **Commitment 1:** Patient-level data, study-level data, and complete CSRs to qualified researchers

• **Commitment 2:** CSR synopses to general public

• **Commitment 3:** Results to clinical trial participants

• **Commitment 4:** Compliance certification

• **Commitment 5:** Publish significant research in the medical literature
PhRMA-EFPIA Commitments Offer Responsible Alternative

• Protect Patient Privacy
  – Protection against disclosure of patient health data when it is reasonably likely that such personal information could be re-identified and publicly disclosed

• Maintain Integrity of Regulatory System
  – Disclosure of patient-level data only to legitimate researchers after submission of a research proposal
  – Avoid diversion of regulatory resources

• Encourage and Protect Investments in Medical Research
  – Disclosure of patient-level information and full CSRs only to researchers with data sharing agreement
  – Disclosure to competitors not required
Additional potential areas of focus for collaborative partnerships/precompetitive consortia in pharma

• Biomarkers: efficacy and safety.
• Target identification and validation (genetics and translational science).
• Reagents, assays.
• Predictive animal and human disease models.
• Human PoP, PoC.
Innovative Therapeutics
Discovery and Development Ecosystem

- Interaction Space
- Network Space
- Patient Groups
- Industry
- Payors
- Academy
- Health Care Providers
- FDA, NIH
- New Therapy