New opportunities offered by Public-Private Partnerships: The IMI model

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Executive Director
Major Challenges for Pharmaceutical Companies

- Patents expiration and fights
- Generics and « Biosimilars »
- Unpredicted failures at late stage of drug development
- Regulatory rules
- Decline in R&D productivity
Key Hurdles in Pharma R&D

- Disease heterogeneity
- Lack of predictive biomarkers for drug efficacy/safety
- Insufficient pharmacovigilance tools
- Unadapted clinical designs
- Lack of incentive for industry
Innovative Medicines Initiative: 
Joining Forces in the Healthcare Sector

2 Billion Euro

1 Billion €
Public

1 Billion €
Private

Partnership
Key Concepts

• “Non-competitive” collaborative research for EFPIA companies

• Competitive calls to select partners of EFPIA companies (IMI beneficiaries)

• Open collaboration in public-private consortia (data sharing, wide dissemination of results)
The Precompetitive Space: Time to Move the Yardsticks

Thea Norman,¹ Aled Edwards,² Chas Bountra,³ Stephen Friend⁴

Industry, government, patient advocacy groups, public funders, and academic thought leaders met in Toronto, Canada, to set into motion an initiative that addresses some of the scientific and organizational challenges of modern therapeutics discovery. What emerged from the meeting was a public-private partnership that seeks to establish proof of clinical mechanism (POCM) for selected “pioneer” disease targets using lead compounds—all accomplished in the precompetitive space. The group will reconvene in April 2011 to create a business plan that specifies the generation of two positive POCM results per year.

2011 may become known as the year in which “out-of-the-box thinking” transformed into “out-of-the-box doing” in the realm of therapeutics discovery—that is, if the bold conclusions that emerged from the February 2011 Summit in Toronto, Canada, archipelago, of experts funded by industry, public funding agencies, and private foundations and would engage patients, clinicians, and scientists from academia, industry, and regulatory agencies as active co-participants. The name ARCH2POCM has their limits; but with a precompetitive drug discovery effort in place, it should be possible to rapidly disseminate negative POCM information in order to protect patient safety and minimize the costly redundancy of having multiple pharmaceutical companies pursuing the same disease targets in isolation of one another.

From this mutual starting point, Summit participants agreed that bold ideas, not pilot programs, are needed to meet the challenges that today’s pharmaceutical industry faces. And everyone concurred that ARCH2POCM must be structured such that all resulting data are made publicly available with no intellectual property (IP) generated through the POCM stage; such an open-access model would unleash truly translational, mechanism-based research and would foster rapid clinical validation of pioneer targets in a manner that (i) maximizes patient safety and (ii) rapidly informs the drug-development industry about those targets for which POCM has been successfully...
Opening Clinical Trial Data

Open Clinical Trial Data for All? A View from Regulators

Hans-Georg Eichler\textsuperscript{1,}*, Eric Abadie\textsuperscript{1,2}, Alasdair Breckenridge\textsuperscript{3}, Hubert Leufkens\textsuperscript{1,4}, Guido Rasi\textsuperscript{1}

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A Typical IMI Consortium

Private Investment 'n kind (Eur 1 billion)

EU Public Funding cash (Eur 1 billion)

ACADEMIA

HOSPITALS

PATIENTS’ ORGANISATIONS

SMALL AND MEDIUM-SIZED ENTERPRISES

REGULATORS

EFPIA

Pharma 1
Pharma 2
Pharma 3
Pharma 4
Pharma 5
Pharma 6
## Overview of current IMI projects

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Expected output of current IMI projects

Number of IMI projects

- Improved knowledge management
- Biomarkers (efficacy/safety)
- Early dialog with regulators
- Mechanistic understanding
- Patient stratification
- Better trained scientists
- Active involvement of patients
- Improved pharmacovigilance
- New manufacturing processes
- Innovative clinical trial design
U-BIOPRED: Towards a new taxonomy of asthma

Paves the way towards tailored treatments for asthmatic patients

38 Partners
- 9 EFPIA companies
- 23 Academic institutions
- 3 Patients’ organizations
- 3 SMEs

- Creates a biobank containing samples from more than 800 adult and children patients suffering from asthma

Diagnosis and definition of severe refractory asthma: an international consensus statement from the Innovative Medicine Initiative (IMI)

Elisabeth H Bel,1 Ana Sousa,2 Louise Fleming,3 Andrew Bush,4 K Fan Chung,5 Jennifer Versnel,6 Ariane H Wagener,7 Scott S Wagers,7 Peter J Sterk,1 Chris H Compton,8 on behalf of the members of the Unbiased Biomarkers for the Prediction of Respiratory Disease Outcome (U-BIOPRED) Consortium, Consensus Generation

Thorax, 66: 910-7 (2011)
PRO-ACTIVE: Patient-reported outcomes in COPD

Paves the way towards patient-reported outcomes as markers of drug efficacy in COPD patients

Activity Monitoring

PDA for PROs

Physical activity

Symptoms reported

Time (days)
NEWMEDS: Innovative approaches to schizophrenia and depression

Develops biomarkers to allow better treatments for schizophrenia and depression

19 Partners
- 9 EFPIA companies
- 7 Public organisations
- 3 SMEs

First achievements

- Has assembled the largest known repository of antipsychotic clinical trial data
- The database contains information on 23,401 patients from 67 industry sponsored studies
- Bringing together data from public projects and 3 companies on the genetics and clinical response in 1,800 well characterized patients with depression
EU-AIMS: Towards new treatments for autism spectrum disorders (ASD)

Develops new tools to study the pathogenesis of ASD and test the efficacy of innovative therapies

25 Partners
- 6 EFPIA companies
- 15 Academic institutions
- 3 SMEs
- 1 charity organization

- Creates an European clinical investigator network
- Establishes a biobank of DNA samples to investigate genetic predisposing factors (CNVs)
- Generates cell lines from iPS of patients
IMIDIA: New knowledge on β cells

A genetically engineered human pancreatic β cell line exhibiting glucose-inducible insulin secretion

Philippe Ravassard, Yasmine Hizhouz, Séverine Pechbert, Emilie Bricout-Neveu, Mathieu Armanet, Paul Czemichow, and Raphael Scharfmann

Finally! A human pancreatic β cell line

Gordon C. Weir and Susan Bonner-Weir

Section on Islet Cell Biology and Regenerative Medicine, Research Division, Joslin Diabetes Center, and Department of Medicine, Harvard Medical School, Boston, Massachusetts, USA.

The Journal of Clinical Investigation  http://www.jci.org  Volume 121  Number 9  September 2011
DIRECT: Towards personalised therapy of diabetes

**Aim:** identification of biomarkers to predict evolution of diabetes and responses to drugs

**25 Partners**
- 4 EFPIA companies
- 21 Academic institutions

- Creates a large European diabetes repository of blood, urine and DNA samples: **5,000** patients with type 2 diabetes enrolled
SUMMIT: Predicting complications of type 2 diabetes

Develops surrogate markers to predict micro- and macro-vascular complications in diabetes

23 Partners
- 18 EFPIA companies
- 4 Public organisations
- 1 SME

- Two multi-centre studies (surrogate markers, imaging):
  - ‘Macrovascular study’ has completed 38% recruitment (564 patients)
  - ‘Retinopathy study’ has completed 54% recruitment (650 patients)

- The consortium has access to samples from 17,867 subjects

- Data repository will contain information on complication profiles from 23,888 diabetic individuals
eTOX: In silico toxicology

- Builds a large searchable database containing drug toxicity-related data extracted from relevant pharmaceutical pre-clinical legacy reports
- Develops innovative methodological strategies and novel software tools to predict toxicological profiles in silico

25 Partners
- 13 EFPIA companies
- 8 Public organisations
- 4 SMEs

First achievement
An innovative multi-scale modelling strategy for the prediction of cardiotoxicity has been developed, successfully tested and published

Generates Comprehensive high-quality **Joint European Compound Collection**:  
- integrating industry’s medicinal chemistry know-how  
- focusing on value (IP) generation => unique, commercially non-available compounds  
- complementing “Pharma Collection” by novel **Public Compound Collection**

Establishes **European Screening Centre** as ‘Centre of Excellence’ for  
- development of target or pathway-specific bioassays  
- performing High Throughput Screening  
- driving experimental hit characterization (selection) process  
- managing data flow and analysis (‘honest broker concept’) and project execution
Coming soon….

6th Call for Proposals 2012
“Combating Antibiotic Resistance”
NEWDRUGS4BADBUGS (ND4BB)
The ND4BB programme

A unique collaborative endeavour to fight antimicrobial resistance:

• Clinical trials to accelerate access to novel antibiotics
• Drug discovery projects
• Extensive data sharing and results dissemination
Public-private partnerships need honest brokering

Michel Goldman

Given the current challenges in research and development, it’s increasingly apparent that collaboration between large pharmaceutical companies, academic teams and biotechnology enterprises is essential for converting basic biomedical discoveries into lifesaving medicines. But these partnerships work best when a neutral third party helps foster them.

A trickling pipeline of new products at many pharmaceutical companies has led to a paradigm shift in the industry’s research and development (R&D) strategy. Indeed, the integrated R&D model in which every step of drug development is conducted in-house has proved largely inefficient in delivering the novel therapies needed to address major health challenges. Therefore, this model is being progressively replaced by open innovation networks that allow the leveraging of external pools of knowledge, especially in universities and biotechnology companies.

The pharmaceutical industry realizes that the best approach is to apply an open innovation concept to pre-competitive research that encourages companies to share expertise. These principles were the cornerstones of the Critical Path Initiative launched by the US Food and Drug Administration in 2004, which led to the creation of the Critical Path Institute, an Arizona-based nonprofit dedicated to fostering collaborations between industry, academia and regulators.

Across the pond, the Innovative Medicines Initiative (IMI), a public-private partnership between the EU and the European Federation of Pharmaceutical Industries and Associations, is a prototypic example of an organization created to support open innovation and pre-competitive research in the pharmaceutical sector. It has raised awareness about the principles of open collaboration and has launched several education and training programs for scientists from industry or academia interested in drug development and transparent competition, rather than through pre-existing connections. For this reason, IMI organizes a competitive process to identify the best partners to match with the pharmaceutical companies that, for their part, invest considerable resources in the projects, propose the research topics and most often coordinate the projects.

This leading role of industry, which distinguishes IMI from most other public-private partnerships, guarantees the optimal exploitation of the knowledge created and its dissemination by the research consortia. As an example, within one of the IMI consortia for diabetes, the optimal exploitation of the first human beta cell line usable for the development of anti-diabetic drugs was made possible by the partnership between the academic team that made the basic discovery, a small enterprise that commercializes the cell product and the large pharmaceutical enterprises that will develop drug screening assays relying on this innovative tool.

Ensuring that consortia operate in a balanced manner in terms of intellectual property and allocation of resources requires a neutral party that can act as a referee whenever needed. To address this need, IMI facilitates consortium agreements by playing the role of impartial broker. A key mission of a neutral body such as IMI is, of course, to ensure the sound management and allocation of public funds in the interest of both industry and society. Here, IMI develops performance indicators suited to measure the added value of public-private partnerships. As an example, IMI is closely

“A neutral organizer is key to ensure the sustainability of public-private partnerships and to restore trust in and among the stakeholders committed to the development of innovative therapies.”
THANK YOU!

www.imi.europa.eu