Introduction:

**IMI moves personalized medicine forward**

Michel Goldman, MD, PhD
Executive Director
Innovative Medicines Initiative:
Joining forces in the healthcare sector

2 Billion €

1 Billion €
Public

1 Billion €
Private

Partnership
Key challenges addressed

- Disease heterogeneity
- Lack of **predictive** biomarkers
- Outdated clinical designs and regulatory processes
- Insufficient incentives for pharma industry and biotech companies
- Need for mindset shift in stakeholder communities
Key concepts underlying IMI

“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 Evolution of IMI
From bottlenecks in industry to bottlenecks in society

Make Drug R&D processes in Europe more efficient and effective and enhance Europe’s competitiveness in the Pharma sector

Idea generation
Basic research and non-clinical testing
Human testing
Regulatory Approval
HTA and Pharmacovigilance

Primary focus of early IMI calls
2007 SRA

Shift to addressing challenges in society and healthcare
2011 SRA

SRA – Strategic Research Agenda
Private Investment

in kind

(€ 1 billion)

EU Public Funding
cash

(€ 1 billion)

A Typical IMI Consortium

EFPIA

Pharma 1
Pharma 2
Pharma 3
Pharma 4
Pharma 5
Pharma 6

ACADEMIA

HOSPITALS

PATIENTS’ ORGANISATIONS

SMALL AND MEDIUM-SIZED ENTERPRISES

REGULATORS
IMI as a neutral platform

Fosters large scale industry collaboration and engagement with scientific community

Catalyses open innovation

Facilitates Intellectual Property agreements

Ensures excellence of partnerships and projects

Promotes active involvement of patients, regulators and payers
Ongoing IMI Projects
Key figures of ongoing IMI Projects

- **594 Academic & research teams**
- **363 EFPIA teams**
- **109 SMEs**
- **9 regulators**
- **18 patient org**

- **€580.7 mln IMI JU cash contribution**
- **€587.5 mln EFPIA 'n kind contribution**

~ 4500 researchers

**Increased probability of success**

**Earlier patient access**
Mapping Collaborative Networks

Collaborative publications among IMI researchers

Thomson Reuters Custom Analytics & IP Solutions, 2013
Collaborative activities in IMI Projects

N Collaborations

- Cross-Sector
- Within-Sector

Data & analysis: Thomson Reuters Custom Analytics & IP Solutions, 2013
Advances in autism research

Based on sequencing 78 Icelandic parent–offspring trios, a total of 219 distinct individuals (44 autistic, 21 schizophrenic offspring) the consortium has identified 4933 de novo mutations.

The consortium has found that as a man ages, the number of de novo mutations increases in his sperm, increasing the chance for his child to carry a deleterious mutation that could lead to autism or schizophrenia.

A new animal model that replicates a nonsyndromic autism was developed. A demonstration of the reversal of the condition with specific therapy in mice presents an opportunity clinical development of new treatments for autism.

“Synapse dysfunction in autism: a molecular medicine approach to drug discovery in neurodevelopmental disorders” – a review of the opportunities and challenges in drug development for autism and the insight into the neurobiology of ASDs.
A unique partnership
Proposed ways to reduce required numbers of patients needed for antipsychotic trials while preserving 90% power (p<.05)

Based on resampling of data from 34 such trials (n=11,670 patients) data from Astra Zeneca, Janssen, Lilly, Lundbeck, Pfizer

Samples can be reduced from 79 to 46 patients per arm by targeting trials

The trial duration can be reduced from 6 to 4 weeks

Current mix =70% female; 20% early episode; 40% enriched

Enriched=prominent positive and negative symptoms

Early episode=under 3 with 4 or more years of illness

Note: Per patient cost 6wk study $70,000-$100,000
‘Think Big’
Research on human diseases at an unprecedented scale

- Access to information on **40 million patients** through EHR
- **Alzheimer’** disease: research on **10-times more subjects than ADNI** (Alzheimer's Disease Neuroimaging Initiative)
- Metabolics research on **> 20,000 obese & Type 2 diabetes subjects**
- Linkage of clinical and “omics” data
- Development of a secure (privacy, legal) modular platform
- Continue to build a network of data sources and relevant research

**58 partners** (3 consortia + Efpiia)

- **>200 scientists** involved
- **14 European countries** represented
- **Total budget €56.4m**
- “3 projects in one”
Exploiting Electronic Health Records

**Academic perspective**
- Provide tools and services to better plan and conduct academic trials
- Facilitate comparative effectiveness research

**Pharmaceutical perspective**
- Improve patient recruitment process and study design
- Better understanding of real patient populations
- Support observational and outcomes research studies in real-world settings
- Enable more cost effective research and clinical trials

**General Healthcare perspective**
- Facilitate the re-use of EHR data to more efficiently manage public health issues
- Enabling safer and more evidence-based diagnosis and treatment
A new of taxonomy diseases to foster personalized medicine

- Cancer
- Asthma
- Diabetes
- Rheumatic diseases
- Alzheimer
- Parkinson
- Chronic obstructive pulmonary disease
The IMI Education and Training projects
A patients’ academy on therapeutic innovation

- **develop and disseminate** accessible, well-structured and user-friendly information and education on medicines R&D
- **build competencies** among well informed patients and the public about pharmaceutical R&D
- **build expert capacity** in patient advocates
- **create the leading public library** on patient information in six most common languages under public licensing
- **establish a widely used, sustainable infrastructure** for objective, credible, correct and up-to-date knowledge
- **facilitate patient involvement in R&D** to support industry, academia, authorities and ethics committees
Objective of the workshop

To demonstrate that public-private partnerships move personalized medicine forward by:

• addressing key scientific challenges
• developing tools to translate scientific advances into regulatory guidelines
• considering new pathways to accelerate patient access to innovative therapies
• providing a neutral platform that fosters collaboration between stakeholders
THANK YOU!

www.imi.europa.eu