WELCOME

Investing in excellence
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IMI: towards a new ecosystem in healthcare

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The way in which new medicines are developed is changing
BioPharma companies are collaborating more with external partners

Origin of new medicines in the European Union 2010-2012

- **In the past 3 years 35-40% of top pharma company pipelines have been sourced externally.**

- **94 MAAs 2010 to 2012**
  - 21 2010
  - 37 2011
  - 36 2012

Data Source: ‘Where do new medicines originate from in the EU?’ – Lincker et al., Nature Drug Discovery, 2014
SMEs – An important source of new medicines, especially for orphan drugs and Specialty Therapeutics

- Data shows the important role of SMEs in the upstream phase of pharmaceutical innovation especially for orphan drugs
  - 61% of orphan drugs originated in SMEs
  - 22% originate in pharma
  - 11% originate in academic/public bodies/PPPs

Data Source: ‘Where do new medicines originate from in the EU?’ - Lincker et al., Nature Drug Discovery, 2014
Current EU “Patient Journey” is expensive and slow

![Diagram showing the process of drug discovery and development]

- **Pre-Clinical Research**
  - Closed & Open Innovation
  - Drug Discovery
  - Pre Clinical Testing

- **Clinical Trials**
  - Phase 1
  - Phase 2
  - Phase 3

- **Phase 1**: 5,000 - 10,000 Compounds
- **Phase 2**: 250 Compounds
- **Phase 3**: 5 Therapies

- **Number of Patients/Subjects**
  - 20-100
  - 100-500
  - 1000-5000

- **2 – 5 Years**
- **6 – 7 Years**
- **3 – 6 Years**

**Total Cost:** $2 - $4 Billion USD

**Sources:**
A new approach needed

“Deciphering the complexity of human diseases and finding safe, cost-effective solutions that help people live healthier lives requires collaboration across scientific and medical communities throughout the health care ecosystem.

Indeed, we must acknowledge that no single institution, company, university, country, or government has a monopoly on innovation.”
Innovative Medicines Initiative: Joining Forces in the Healthcare Sector

The biggest public/private partnership in Life Science aiming to:

- Make drug R&D processes in Europe more **innovative** and **efficient**
- Enhance Europe’s **competitiveness**
- Address key **societal challenges**

**Features:**

- 1:1 funding, joint decision making
- All EU funds go to SMEs, academia, patient organisations and regulatory agencies
- Large pharmaceutical industry, represented by EFPIA, contributes in-kind
Key Figures IMI Projects up to call 9

- **650** Academic & research teams
- **409** EFPIA teams
- **120** SMEs
- **25** patient org.
- **17** regulators

> **6000** researchers

Collective intelligence networks
Improved R&D productivity of pharma industries
Innovative approaches for unmet public health needs

Number of publications by year:
- 2009: 0
- 2010: 10
- 2011: 40
- 2012: 200
- 2013: 280
How IMI addresses Anti-Microbial Resistance: the ND4BB programme

Antimicrobial resistance – a growing threat

25 000 Europeans killed / year
€1.5 bn costs to economy / year
2 new classes of antibiotics in the last 30 years

IMI already invested €655 million for:
- Solving scientific challenges
- Fostering new models of industrial collaborations
- Developing clinical networks
- Revisiting regulatory rules
- Providing incentives to industry
ALZHEIMER’S DISEASE: An urgent need for new therapeutic strategies

<table>
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<tr>
<th>Major Public Health Need</th>
<th>Recent failures</th>
<th>Hurdles to drug development</th>
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<td>- 10m Europeans affected, will reach 14m by 2040</td>
<td>Inconclusive results of 3 large clinical trials:  - solanezumab  - bapineuzumab  - human immunoglobulins</td>
<td>Complexity of brain pathology  Patients’ heterogeneity  Lack of validated markers for disease activity</td>
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<td>- Annual cost in EU: €180b, will reach 250b by 2030</td>
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How IMI addresses Alzheimer’s disease

IMI invested €114 million in 3 projects aiming at:

- Developing models to predict the efficacy of drug candidates in patients

- Connecting data on 40 millions of individuals to decipher links between genetic background, biological abnormalities, brain imaging changes, mental symptoms and disease progression

- Identifying subgroups of the disease allowing to tailor therapies according to the different causal factors involved
DIABETES: Fighting the epidemic through Public-Private Partnership

Major Public Health Need

Diabetes will affect 43 million Europeans in 2030

€89 million spent on 2011 on treating diabetes and its complications

Distrust in past-research

Cardiovascular complications of rosiglitazone and benfluorex

Hurdles to drug development

Patients’ heterogeneity

Lack of reliable markers for disease activity and complications
How IMI facilitates the development of new diabetes therapies

IMI already invested €117 million in 3 projects aiming at:

- Solving scientific challenges
- Developing reliable measures of diabetes activity and complications
- Developing treatments tailored to the different needs of individual patients
The measures of success

- New models developed & published
- Setting new standards
- In house implementation by industry
- Impact on regulatory guidelines

Better Science = Better Decisions
Science is leading to innovation in targeted, personalised therapies

• **Diseases are becoming more discrete entities**
  • Every disease will be a molecular ‘orphan disease’
  • Diseases with the same molecular ‘faults’ will have common therapies

• **Therapies will target a smaller and focused group of individuals**
  • New therapies will fit the ‘right’ patients
  • Diagnostic tests will determine who is best to benefit from the new treatments
  • Groups will be stratified into smaller subsets based on outcomes

• **Testing will require better use of data**
  • Better simulations, family history, capturing data from the ‘real world’
Legislative and regulatory pathways have not kept pace with scientific innovation

- **Science has evolved beyond the current trial system**
  - Trials slow, expensive, highly regulated, inflexible

- **Development pathways require large trials to target small populations**
  - Small studies may miss subsets of patients who respond
  - Large trials may be impossible as treatments become more personalised and science continues to improve our knowledge

- **Reimbursement needs to reflect the reality of new therapies**
  - They use medical resources more efficiently
  - They create value for high efficacy populations
  - They will have better outcomes as we remove non-responders through stratification
Important unmet medical needs still exist

• Burden of disease on patient and society = total cost of disease for healthcare and social security

• Unmet need:
  – No treatment
  – Inadequate treatment (resistance or treating symptoms, not cause)
  – Inadequate formulation for specific population (geriatric, pediatric, etc)

• Barriers and incentives
The Evolution of IMI: From bottlenecks in industry – to bottlenecks in Industry and 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

Daily Medical practice

Primary focus of early IMI calls
2007 SRA

Shift to also addressing challenges in
in society and healthcare
2011 SRA

IMI 2 includes real life medical practice
2013 SRA
Major Axes of Research

1. Biomarker identification/validation (precision medicine)
   - Innovative methodologies to evaluate treatment effect
   - Adoption of innovative clinical trial designs
   - Benefit/Risk Assessment

2. Target Identification and Validation (human biology)
   - Determinants of drug/vaccine Safety and efficacy
   - Innovative drug delivery methodologies
   - Manufacturing for personalised medicines

3. Innovative Medicines
   - Discovery and Development of novel preventative and therapeutic agents
   - Innovative adherence programmes

4. Innovative clinical trial paradigms
   - Healthcare delivery: focus on the treatment programmes not just the medicine

5. Patient tailored adherence programmes

European Health Priorities

Drive change in delivery of medical practice
Conclusions - barriers need to be removed, collaboration among stakeholders must be fostered:

• More stimulus to basic research and enhanced academia/industry collaboration

• Investment in e-health records, biobanks, genetic databases and linking these up... ‘real world’ data should be harnessed to improve patient outcomes

• Innovative evaluation systems and coherent HTA processes and flexible pricing are essential to better address the needs of patients and support access to personalised medicines

• IMI2 offers a neutral platform to bring stakeholders together and enable collaboration and the practical application of revised research, regulatory and reimbursement pathways