



Innovative Medicines Initiative



WELCOME

Investing in excellence

Tuesday 18 February 2014

Le Meridien Hotel, Brussels



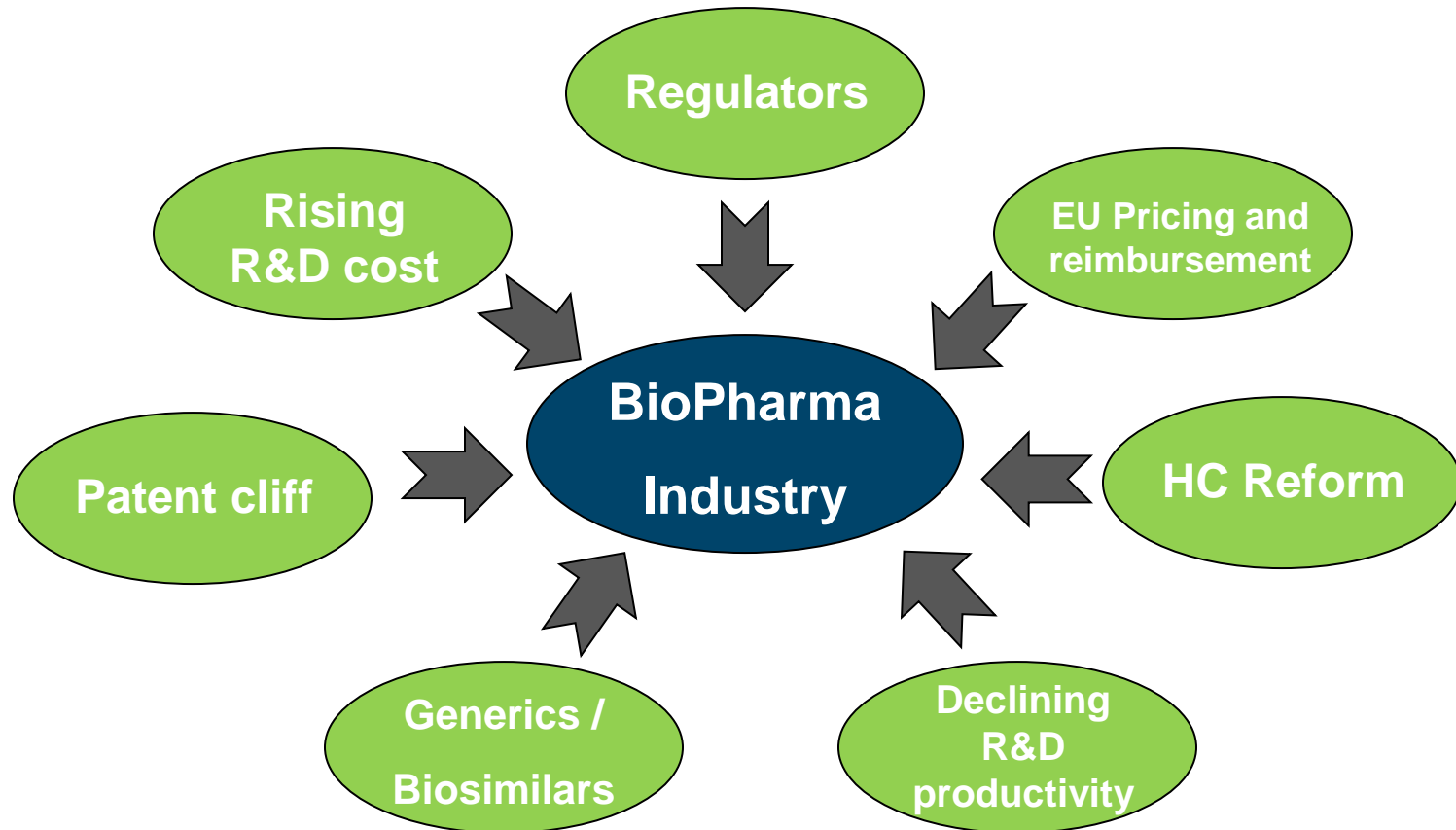
Innovative Medicines Initiative

IMI: towards a new ecosystem in healthcare

Carlo Incerti, M.D.

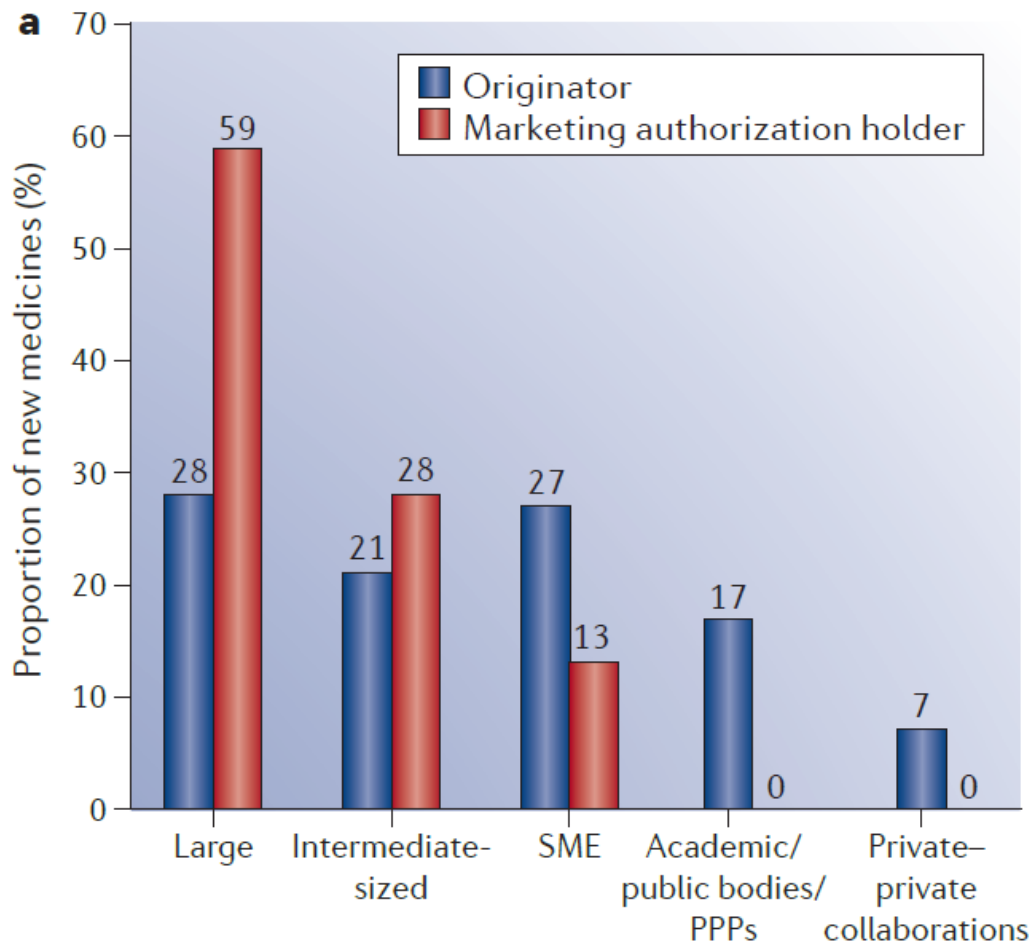
**Head of Global Medical Affairs, Chief Medical Officer
Genzyme Corporation**

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



94 MAAs 2010 to 2012

21 2010

37 2011

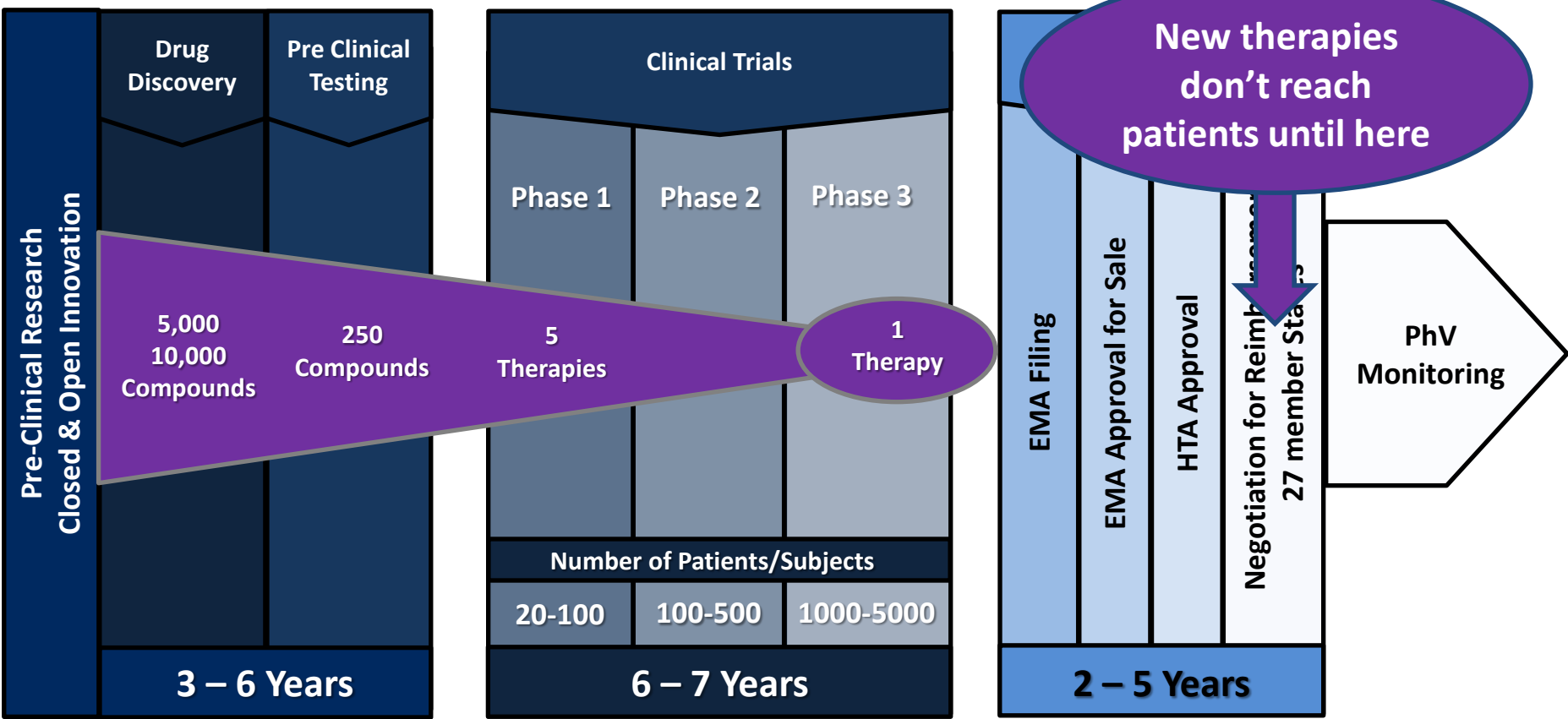
36 2012

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

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

Current EU “Patient Journey” is expensive and slow



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

Sources: Drug Discovery and Development: Understanding the R&D Process, www.innovation.org;
 CBO, *Research and Development in the Pharmaceutical Industry*, 2006;
 Forbes, [Matthew Herper](#), “The Truly Staggering Cost Of Inventing New Drugs”, February 10, 2012

A new approach needed

Sci Transl Med 29 January 2014:
Vol. 6, Issue 221, p. 221ed2
Sci. Transl. Med. DOI: 10.1126/scitranslmed.3008294

EDITORIAL

DRUG DISCOVERY

Turning the Titanic

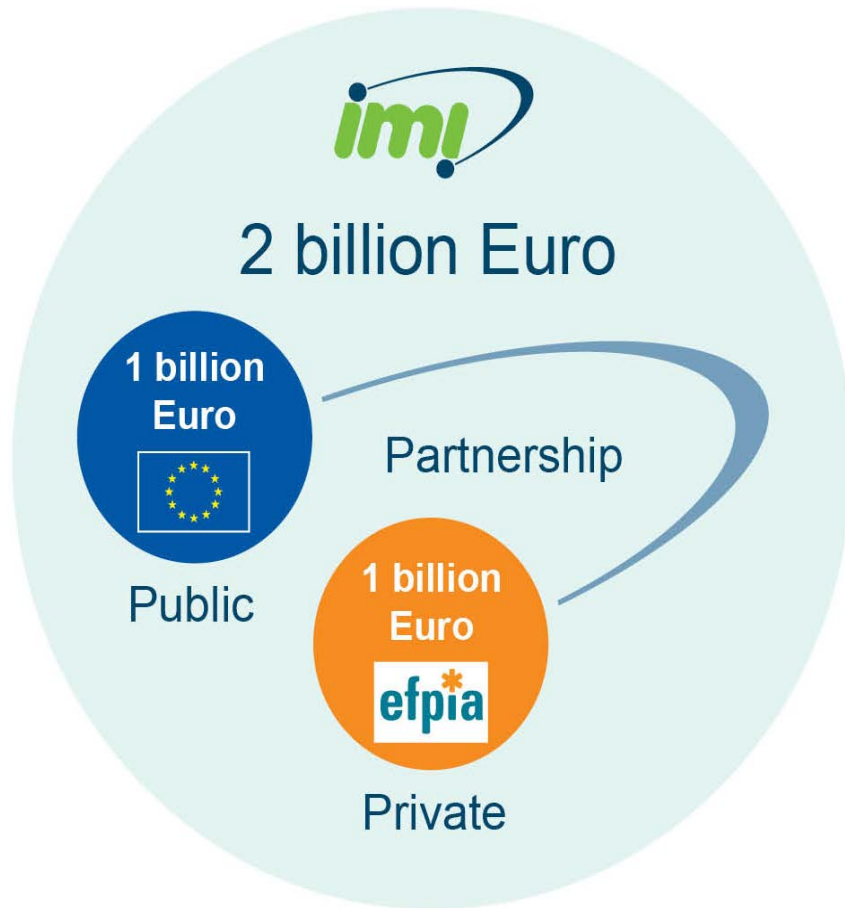
Elias A. Zerhouni



“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*



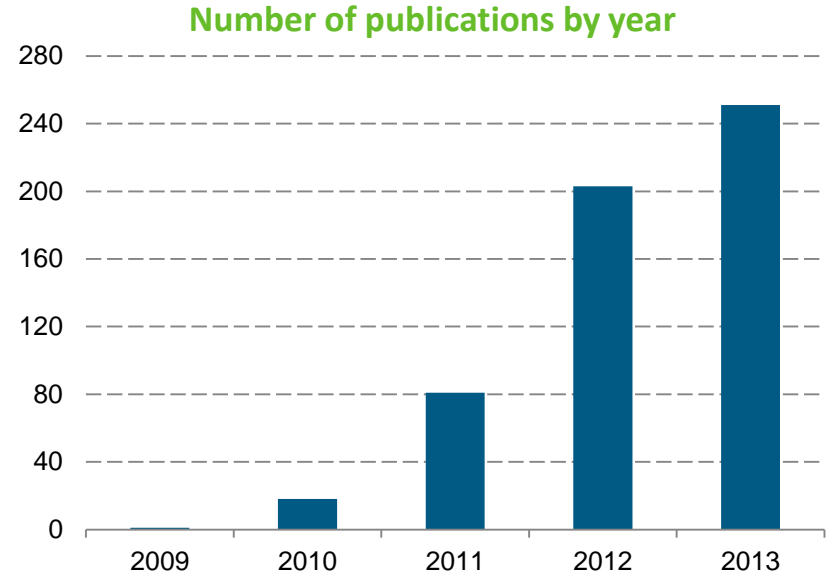
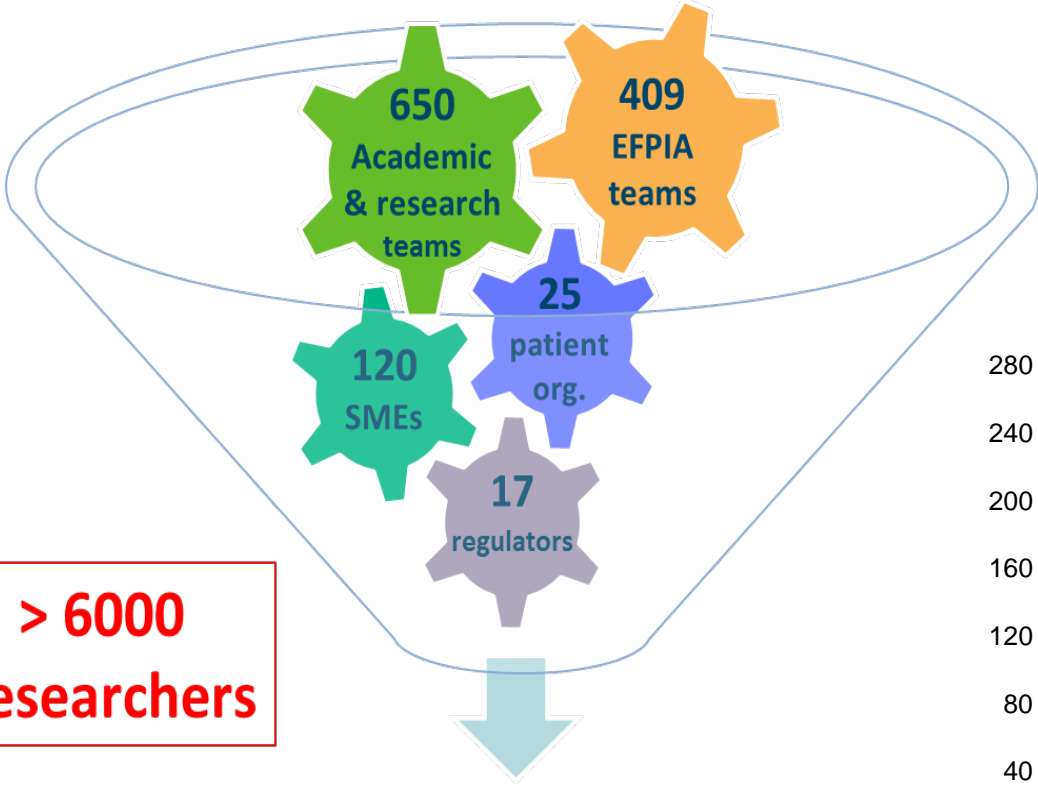
The biggest public/private partnership in Life Science aiming to:

- 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



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

How IMI addresses Anti-Microbial Resistance: the ND4BB programme

Antimicrobial resistance – a growing threat

25 000

Europeans killed / year

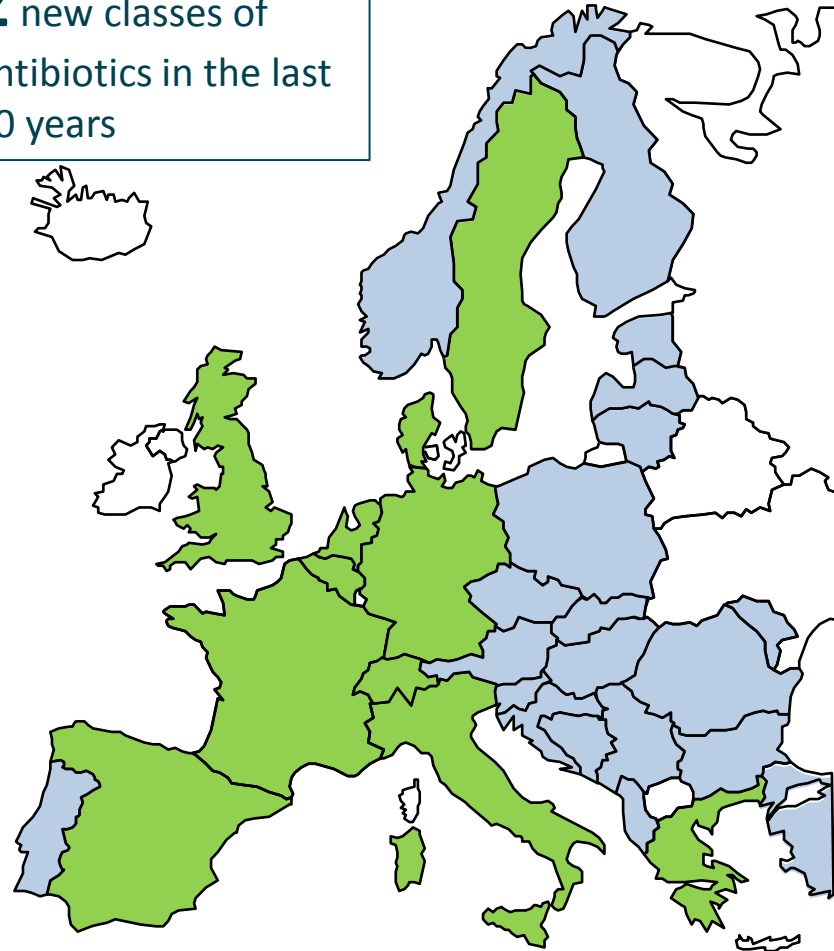
€1.5 bn

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

Major Public Health Need

- **10m** Europeans affected, will reach **14m by 2040**
- Annual cost in EU: **€180b**, will reach **250b by 2030**

Recent failures

- Inconclusive results of 3 large clinical trials:**
- solanezumab
 - bapineuzumab
 - human immunoglobulins

Hurdles to drug development

- Complexity of brain pathology**
- Patients' heterogeneity**
- Lack of validated markers for disease activity**

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

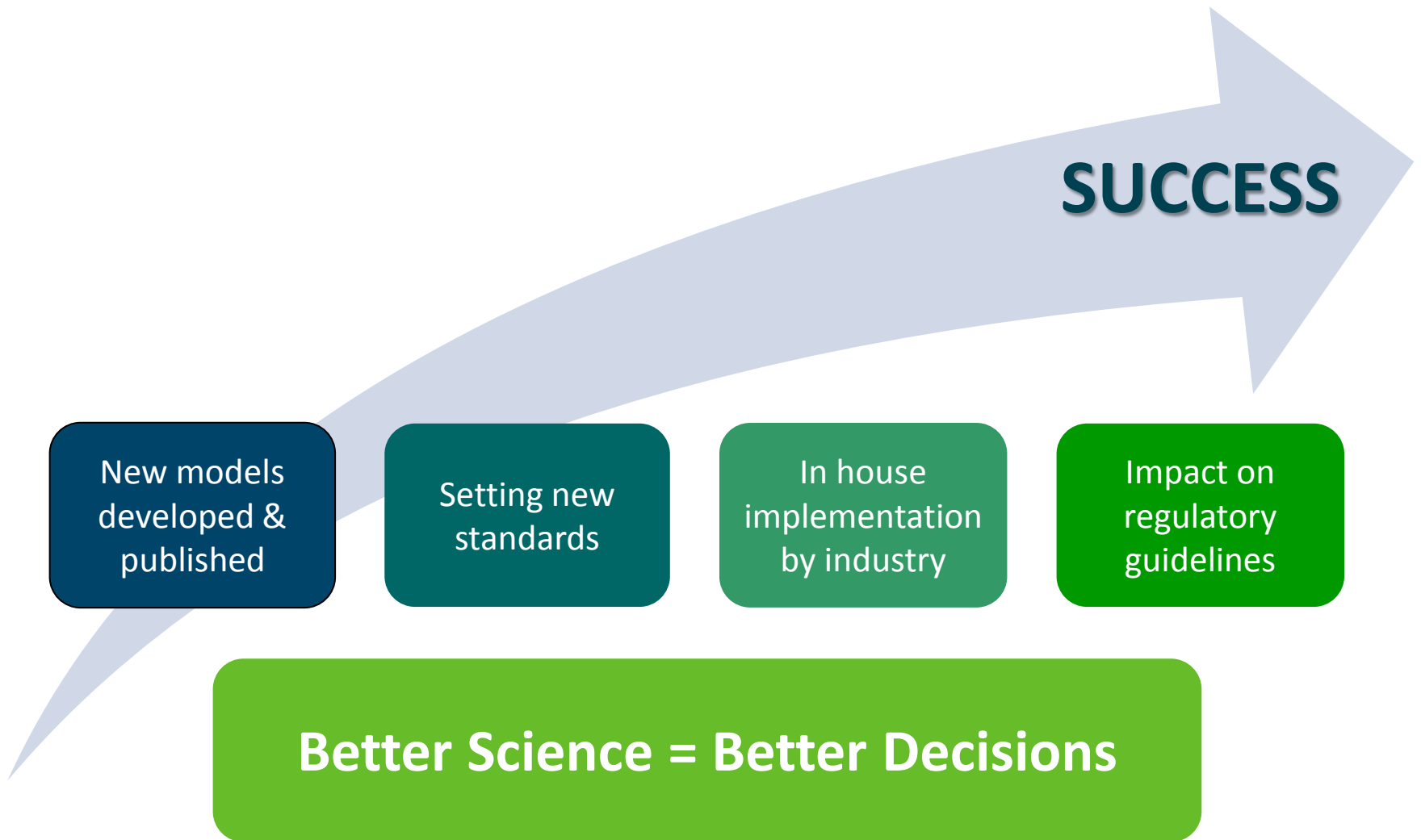
**Lack of reliable markers
for disease activity and
complications**

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



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

Priority Medicines for Europe and the World 2013 Update

Warren Kaplan, Veronika J. Wirtz,
Aukje Mantel-Teeuwisse, Pieter Stolk,
Béatrice Duthey, Richard Laing

9 July 2013



Universiteit Utrecht



WHO Collaborating Centre for
Pharmaceutical Policy and Regulation



WHO Collaborating Center
in Pharmaceutical Policy

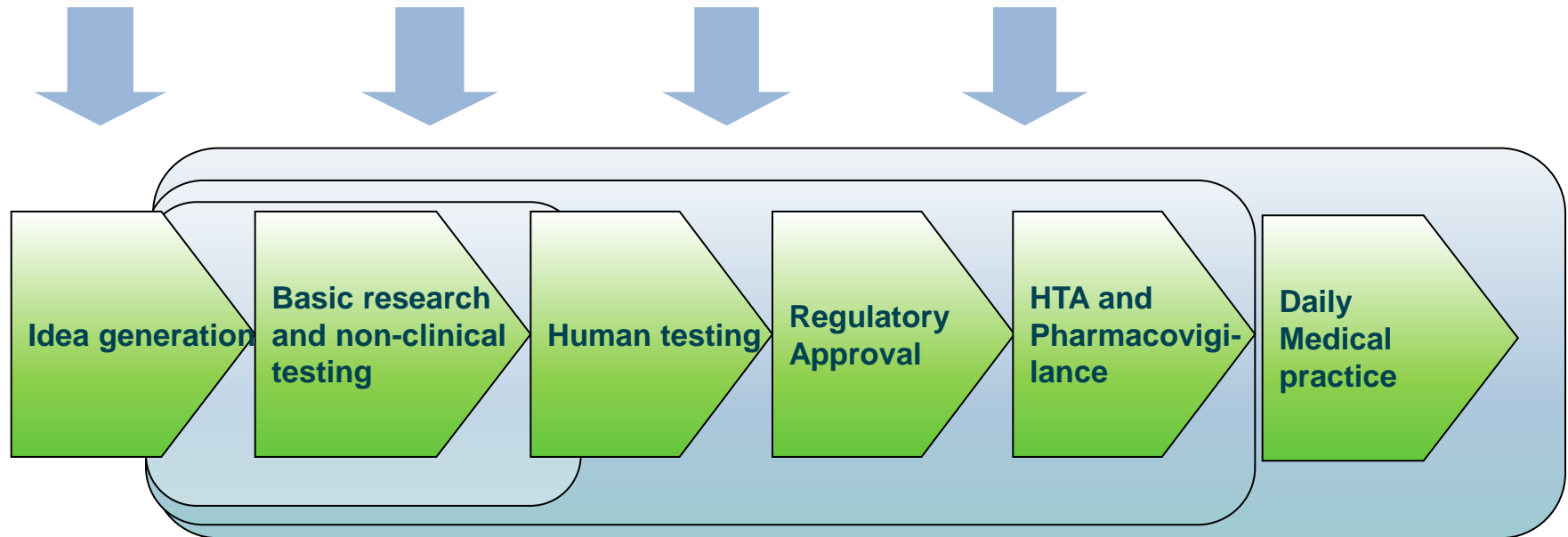


World Health
Organization

- 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

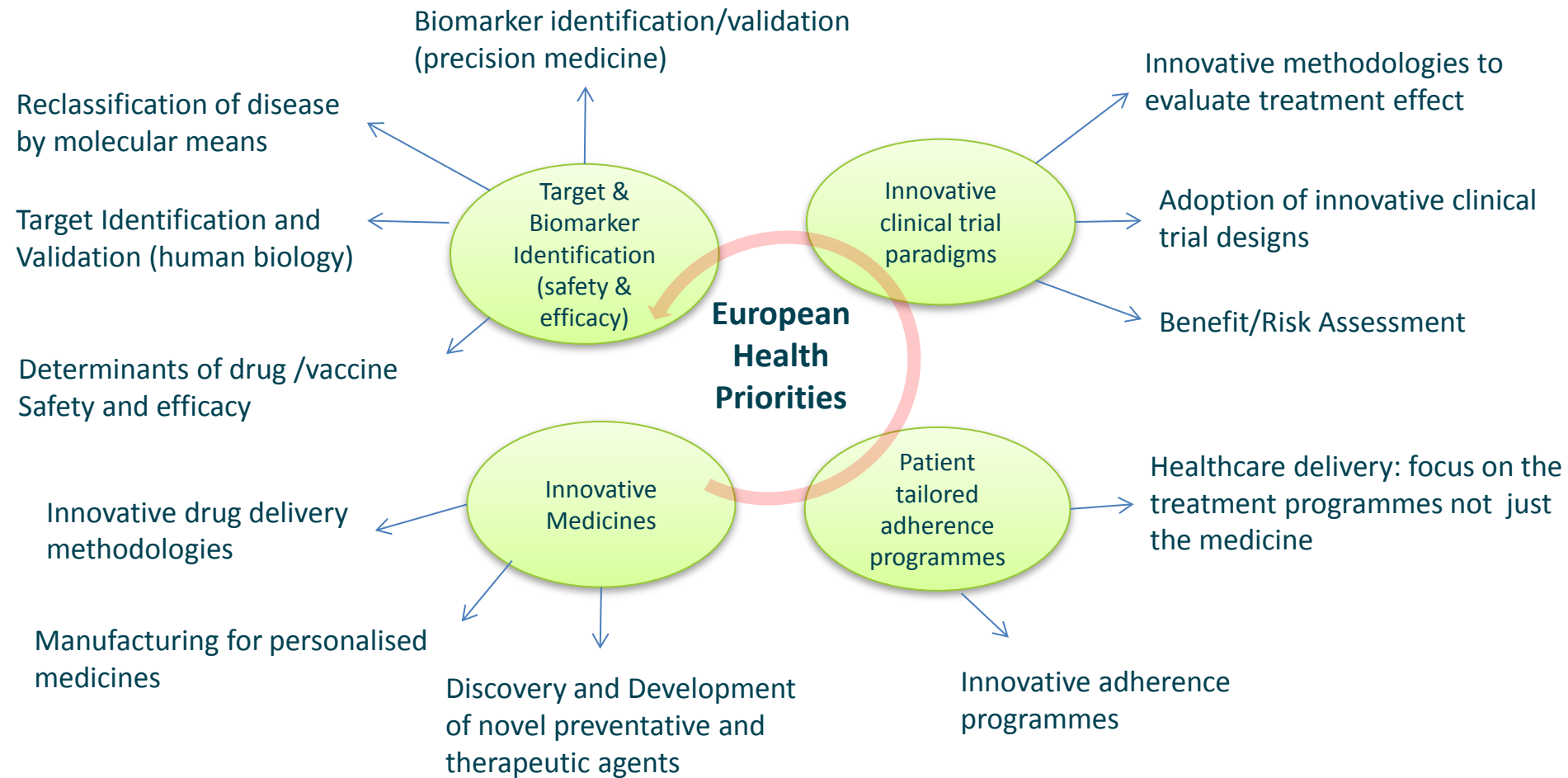


Primary focus of
early IMI calls
2007 SRA

Shift to also addressing challenges in
society and healthcare
2011 SRA

IMI 2
includes real life
medical practice
2013 SRA

Major Axes of Research



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

