



Innovative Medicines Initiative

Research and Development of Breakthrough Therapies: Opportunities for Reduction and Refinement

Michel Goldman, MD, PhD
Executive Director

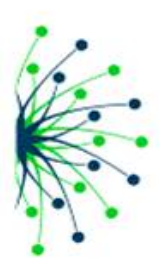
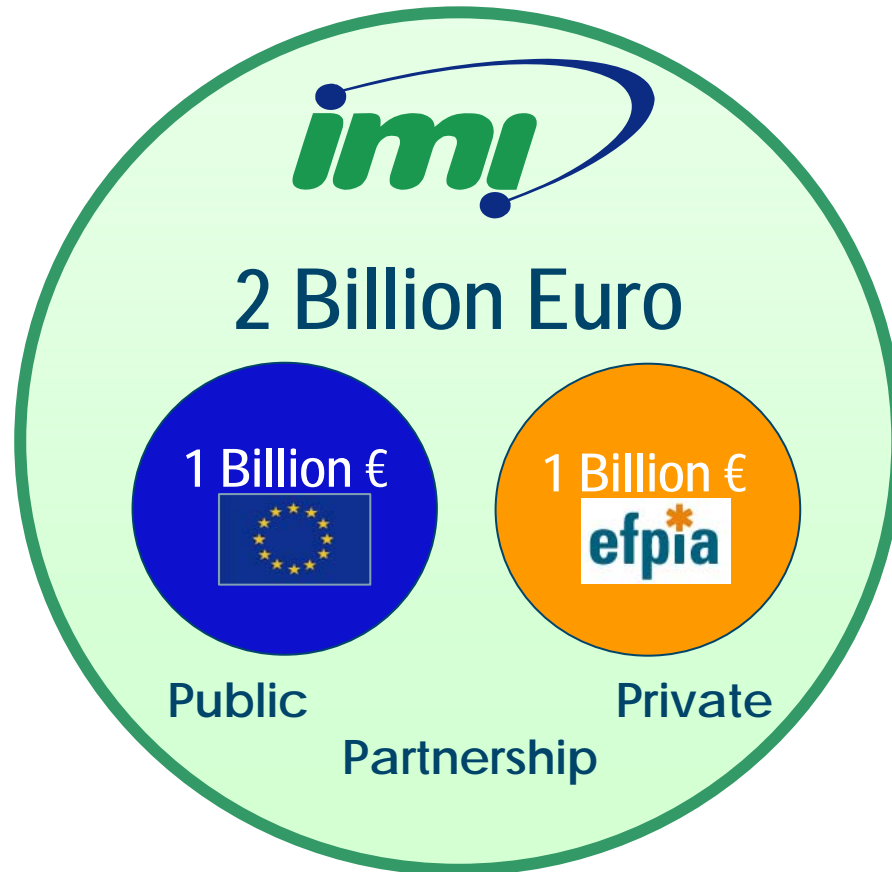


EPAA Annual Conference, Brussels, 30 November 2010



efpia

Innovative Medicines Initiative: the Largest PPP in Life Sciences R&D

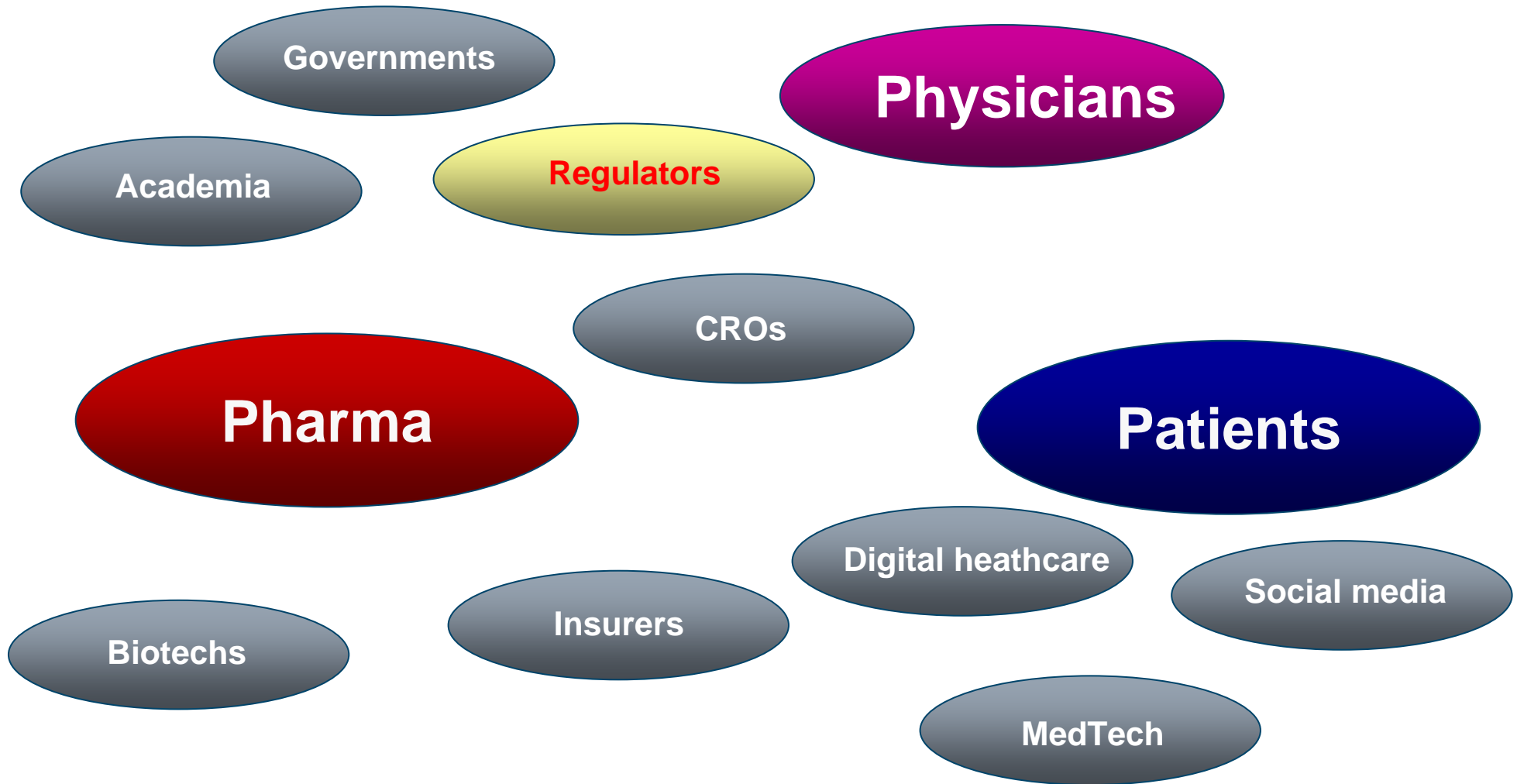


Building the Future of Medicine

- More personalised
- More predictive
- More preventive
- More participative



Towards a new Ecosystem for Drug Development



Governments

Physicians

Academia

Regulators

CROs

Pharma

Patients

Digital healthcare

Social media

Biotechs

Insurers

MedTech

Key Concepts

- Open Innovation
- Pre-competitive research



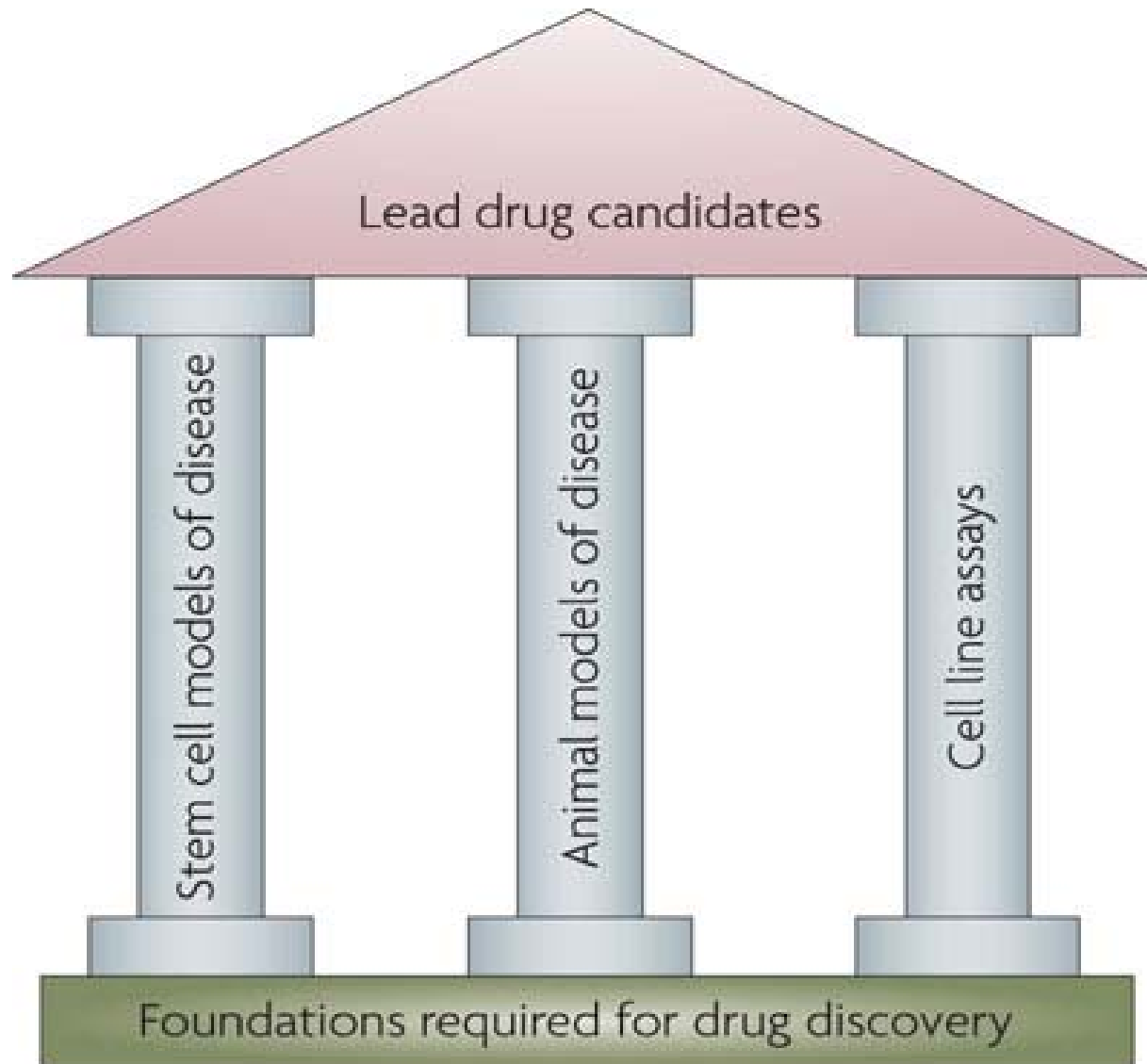
nature medicine

Mechanism matters

The path of drug development is fraught with hurdles. Gaining a clear understanding of how a drug works before it enters clinical trials is the intelligent route to drug discovery and could increase the likelihood for drug success.

Drug development is a risky business. According to the US Food and Drug Administration (FDA), only eight percent of drugs that enter clinical trials are eventually approved. For a drug to gain FDA approval, it must be safe and show some efficacy. Because the FDA does not require any understanding of the mechanism by which a drug acts, it could be tempting to move into clinical trials without this knowledge. However, this may set the stage for failure. An investigational

It is true that we use many highly prescribed drugs without a clear idea of how they work—which targets they hit, what processes they alter and which of these actions are required for therapeutic efficacy. For instance, lithium, used to treat bipolar disorder, modulates many molecular targets, but which—or how many—of these are required for its beneficial effects is uncertain. Nevertheless, understanding a drug's mechanism could guide drug development and help to prevent late-stage failures such as Dimebon's.



Ebert AD, Svendsen CN. *Nature Reviews Drug Discovery*, 9: 367, 2010

Rare Diseases as Surrogates in Drug Development



Disease	Surrogate for	Molecular targets	Drugs
Familial hypercholesterolemia	Common forms of hypercholesterolemia	HMG CoA PCSK9	Statins
Cryopyrin-associated periodic syndromes	Rheumatoid arthritis	IL-1β	Riloncept Canakinumab
Idiopathic Hypereosinophilia	Allergic asthma	IL-5	Mepolizumab
Castleman Disease	Rheumatoid arthritis	IL-6	Tocilizumab
Tuberous sclerosis	Various cancers	mTor	Temsirolimus

Drug Safety: The Need for Novel Approaches



- Individual susceptibility
- Combination therapies
- Unwanted reactions to targeted therapies
- Late effects



The NEW ENGLAND JOURNAL of MEDICINE



Perspective

MARCH 11, 2010

The Missing Voice of Patients in Drug-Safety Reporting

Ethan Basch, M.D.

A patient wants to know about symptoms she may have from a prescription drug she is taking. Consulting the label's "Adverse Reactions" section, she finds a wealth of data. Little does she realize that

drug-development cycle if reporting by patients were standard practice.

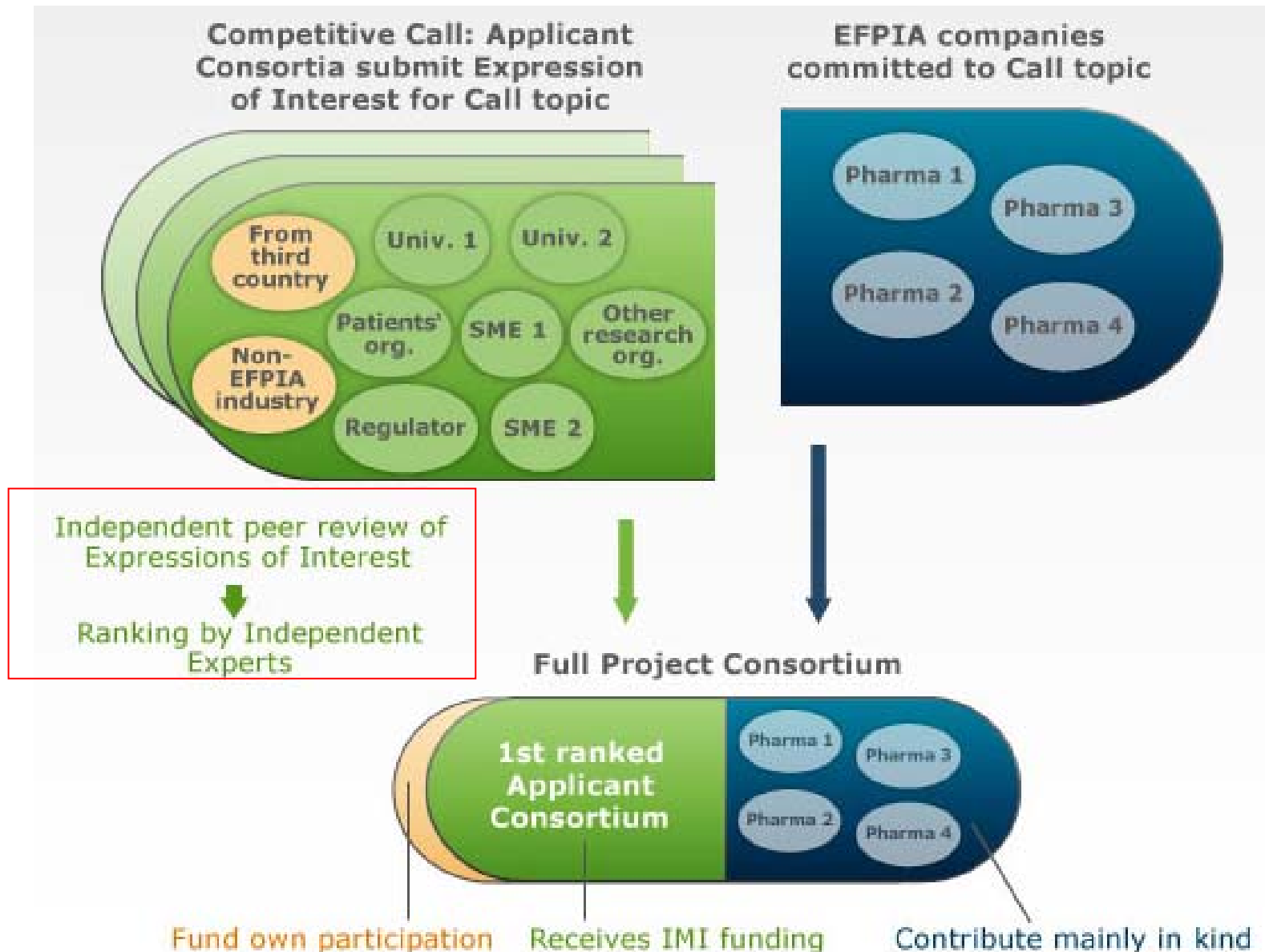
Before a drug has received marketing approval from the Food and Drug Administration (FDA),

N ENGL J MED 362: 865-869, March 11, 2010

The Four Pillars of the Innovative Medicines Initiative



Building a IMI Consortium



IMI Executive Office as a Neutral Third-Party



- To implement programmes and activities in the common interest of all stakeholders
- To monitor the combined use of public funds and industry investment
- To guarantee fair and reasonable conditions for optimal knowledge exploitation and dissemination



Ongoing Projects (1st Call)



15 Projects
395 Teams
Total budget:
281 M€

<i>Acronym</i>	<i>EFPIA Coordinator</i>	<i>Budget (M€)</i>
SAFE-T	Novartis Pharma	35.9
PROTECT	European Medicines Agency	29.8
SUMMIT	Boehringer Ingelheim	28.4
PHARMA-COG	GSK	27.7
IMIDIA	Sanofi-Aventis	25.4
NEWMEDS	Lundbeck	24.0
U-BIOPRED	Novartis Pharma	20.6
EUROPAIN	AstraZeneca	18.2
PROactive	Chiesi Farmaceutici	16.7
MARCAR	Novartis Pharma	13.3
E-TOX	Novartis Pharma	12.9
EMTRAIN	AstraZeneca	7.7
EU2P	F. Hoffman-La Roche	7.2
Pharma Train	Eur. Federation of Courses	6.6
SafeSciMET	F. Hoffman-La Roche	6.3

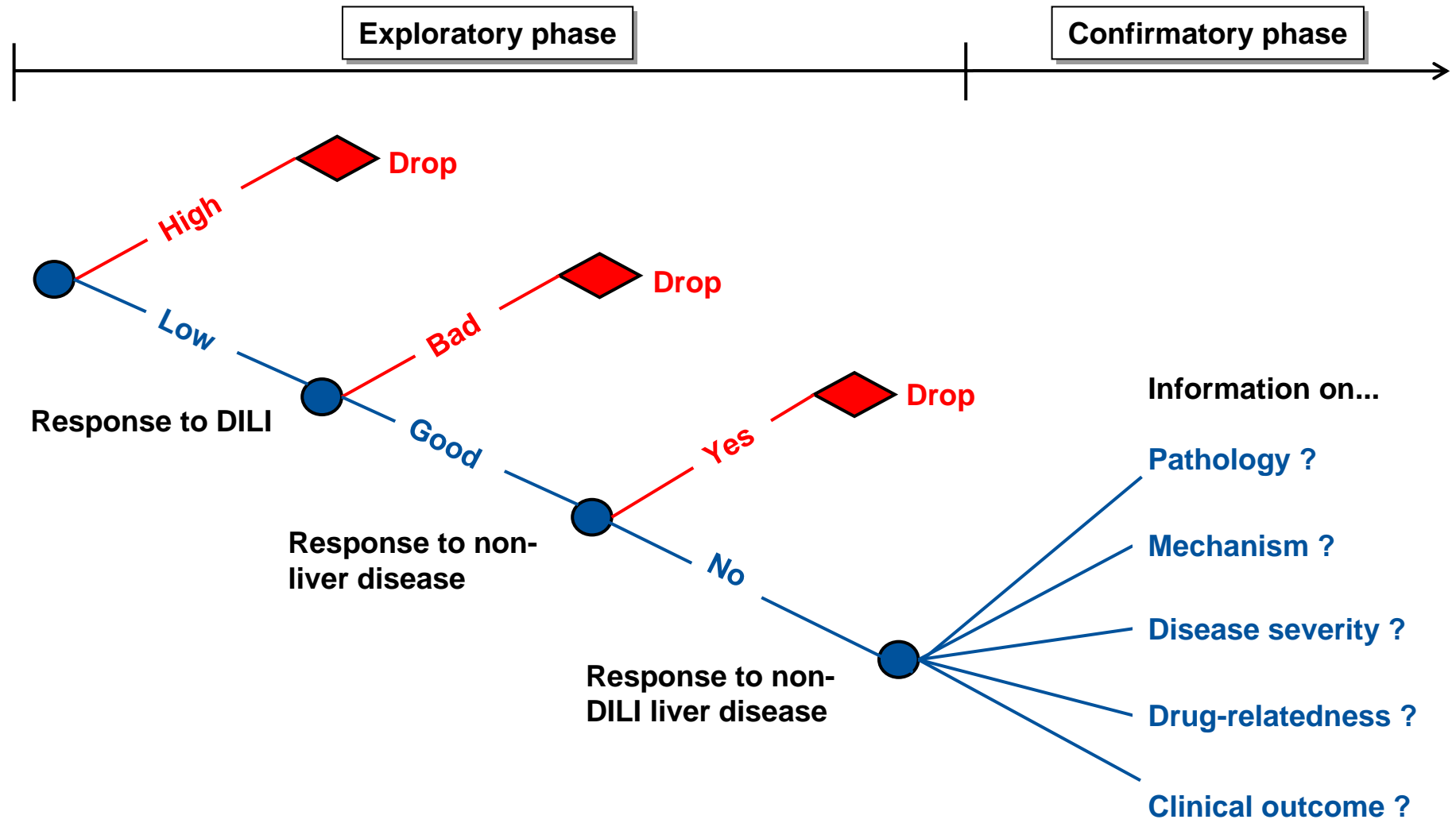
IMI SAFE-T Consortium

Safer and Faster Evidence-based Translation

- *Three organs needing better clinical monitoring of drug-induced injuries:*
 - **Kidney**: current standards increase only once 50-60% of kidney function is lost
 - **Liver**: current standards are not sufficiently sensitive and specific
 - **Vascular System**: no biomarkers currently available

- *Consortium objectives:*
 - To evaluate **utility** of BMs for monitoring DIKI, DILI and DIVI **in humans**
 - To develop **assays and devices** for **clinical application** of safety BMs
 - To **qualify safety BMs for regulatory decision**

Biomarker selection process



IMI SAFE-T Consortium

Partners



- **Pharma:**

- Novartis
- Almirall
- Amgen
- Pfizer
- Hoffmann-La Roche
- AstraZeneca
- Bayer Schering Pharma AG
- Boehringer Ingelheim
- Eli Lilly
- GlaxoSmithKline
- Sanofi Aventis

- **Collaborators:**

- University of Malaga/ Spanish DILI Registry
- University of Liverpool/Centre for Drug Safety Sciences

- **Academic:**

- Barcelona Cardiovascular Research Center
- Charité Hospital
- Groupe d'Etudes et de Recherches en Médecine Interne et Maladies Infectieuses - APHP
- Groupe Hospitalier Pitié Salpêtrière - APHP
- Natural and Medical Sciences Institute
- Tel-Aviv (Souraski) Medical Center

- **SMEs:**

- Argutus Medical Limited
- Experimental & Diagnostic Immunology GmbH
- Firalis SAS
- Interface Europe

- **External Advisors:**

- European Medicines Agency
- FDA

U-BIOPRED:

Biomarkers for Stratification of Patients with Severe Asthma



Consortium:

- **10 EFPIA Pharma Companies**
- **7 Global networks for research on asthma**
- **20 Academic Institutions**
- **3 SMEs**

Aim: To develop a “handprint” of the different types of severe asthma based on genetic data, blood measurements, analysis of tissue sample analyses, clinical findings, patient reported outcomes.....

Deliverable:

- New classification of severe asthma , a key step for the development of novel therapies tailored to patients’ individual needs

PROTECT :

Pharmaco-epidemiological Research on Outcomes of Therapeutics

Consortium:

- **International Alliance of Patients' Organisations**
- **6 Regulatory Bodies** including **EMA** (coordinator)
- **11 EFPIA Pharma Companies**
- **10 Academic Institutions**
- **1 SME**

Aim: To strengthen the monitoring of the **benefit-risk** of medicines

Deliverables:

- New methods for data collection from consumers
- New methods of communicating benefit-risk decisions to all stakeholders

NEWMEDS:

at the Forefront of Research on Schizophrenia



The shock wave hit when they broke the code. It was January 2005, nearly four years since the start of a clinical trial to definitively compare schizophrenia therapies. The US\$43-million trial, involving nearly 1,500

BY ALISON ABBOTT

pressure to rein in costs, several large companies, including London-headquartered AstraZeneca and GlaxoSmithKline, chose to pull out of

of pragmatism thrown in.

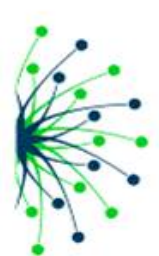
No one questions the transformational impact of the first antipsychotic drugs when they were introduced in the 1950s. Psychiatric hospitals could, for the first time, release

Nature, 11 November 2010

Lessons from Ongoing Projects



- **IMI is more than an industry-academia PPP:**
successful involvement of regulatory agencies, patients' organisations and SMEs
- **First successes and enthusiasm** provide « **proof-of-concept** »
evidence for IMI-type PPPs
- **Pooling of existing databases and patient-reported outcomes** at
the core of many IMI projects



Proposals under Finalisation (< 2nd Call)



Topics

- Knowledge Management
- Cancer
- Rapid diagnosis for infections
- Inflammatory disorders



3rd Call: Indicative Topics



- Early prediction of drug-induced liver injury
- Risk minimisation of antibodies to biopharmaceuticals
- Immunosafety of vaccines
- Translational research on autism spectrum disorders
- Personalised medicine in Type II Diabetes
- New strategies to treat tuberculosis
- Patient awareness on pharmaceutical innovation



Key Challenges

- Boundaries of precompetitive research
- Intellectual property management
- Incentives/rewards for collaboration

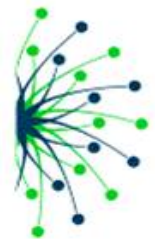




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www.imi.europa.eu

THANK YOU !



efpia