The evolving role of healthcare professionals in the regulatory field

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Disclaimer

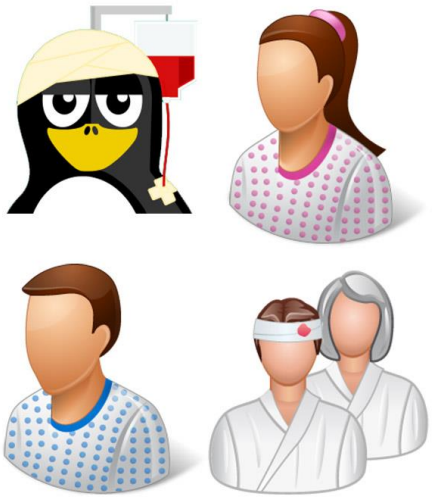
- The content of this presentation reflects my personal opinions and does not imply the agreement of my previous or current employers.

CVBF

Healthcare professionals: who are they?

Clinicians & Practitioners

Healthcare professionals: who are they?



- Clinicians & practitioners

Healthcare professionals: who are they?

- Clinicians & Practitioners

- Funders & reimbursement authorities
- Regulators
- Industry

- Academics
- Patients' associations

What's new?

- Intensive development by academia of first-in-class drugs.
- Progressive shifting from basic science to clinical proof of concepts.
- Especially active in marginal markets
  - Rare diseases
  - Paediatric diseases of genetic origin

Stakeholders in academic drug research

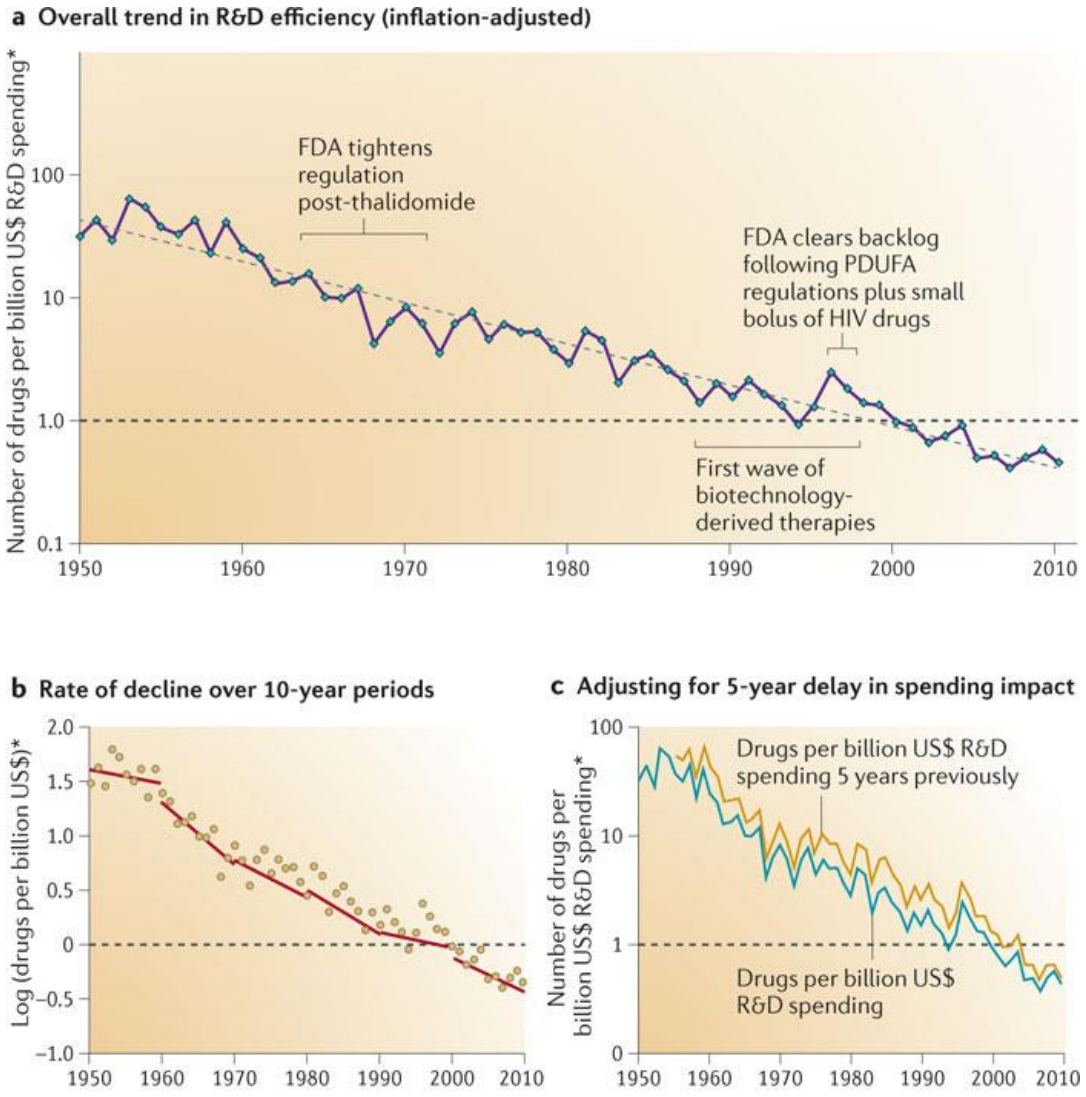
- The academic researcher
- The institution holding the researcher position and funding
- The funders of the project
- The future users of the project results (patients, physicians, industry, citizens, humans)

Innovation Crisis

Industry productivity has been declining steadily for decades.

Known as:

Eroom's law

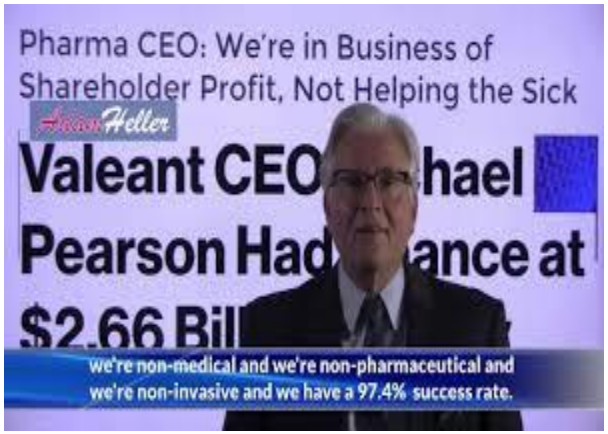


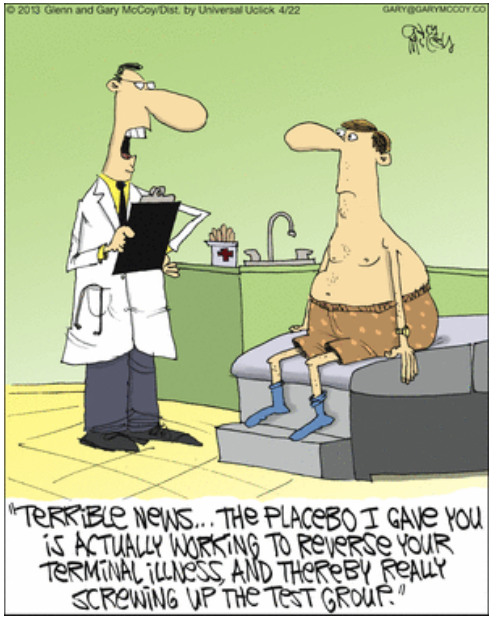
Nature Reviews | Drug Discovery

Why are we failing in producing new therapies?

- Increasing regulatory requirements
- Poorly predictive non-clinical models
- Increasingly expensive therapeutic modalities

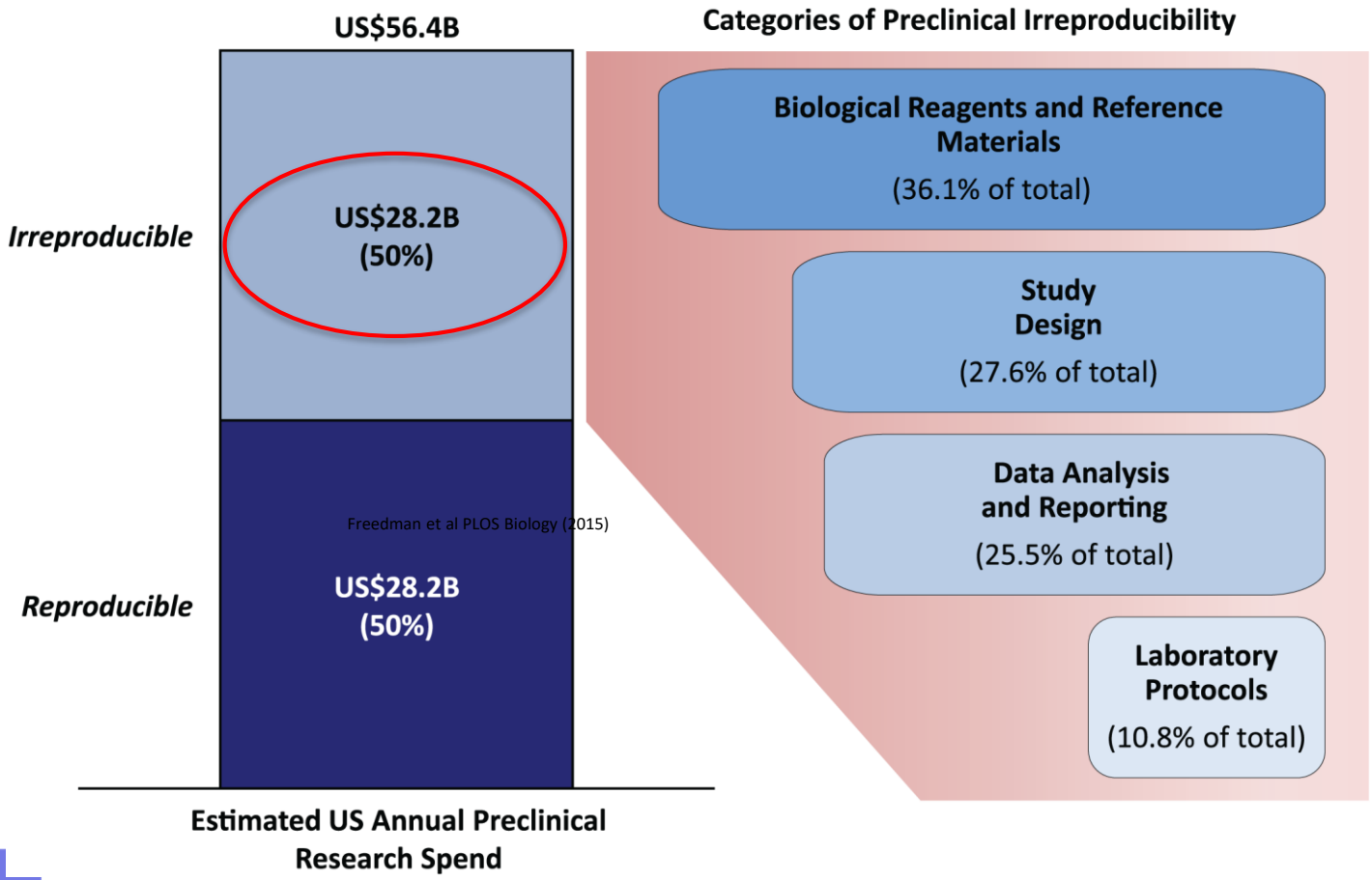
Who is responsible? Blame game



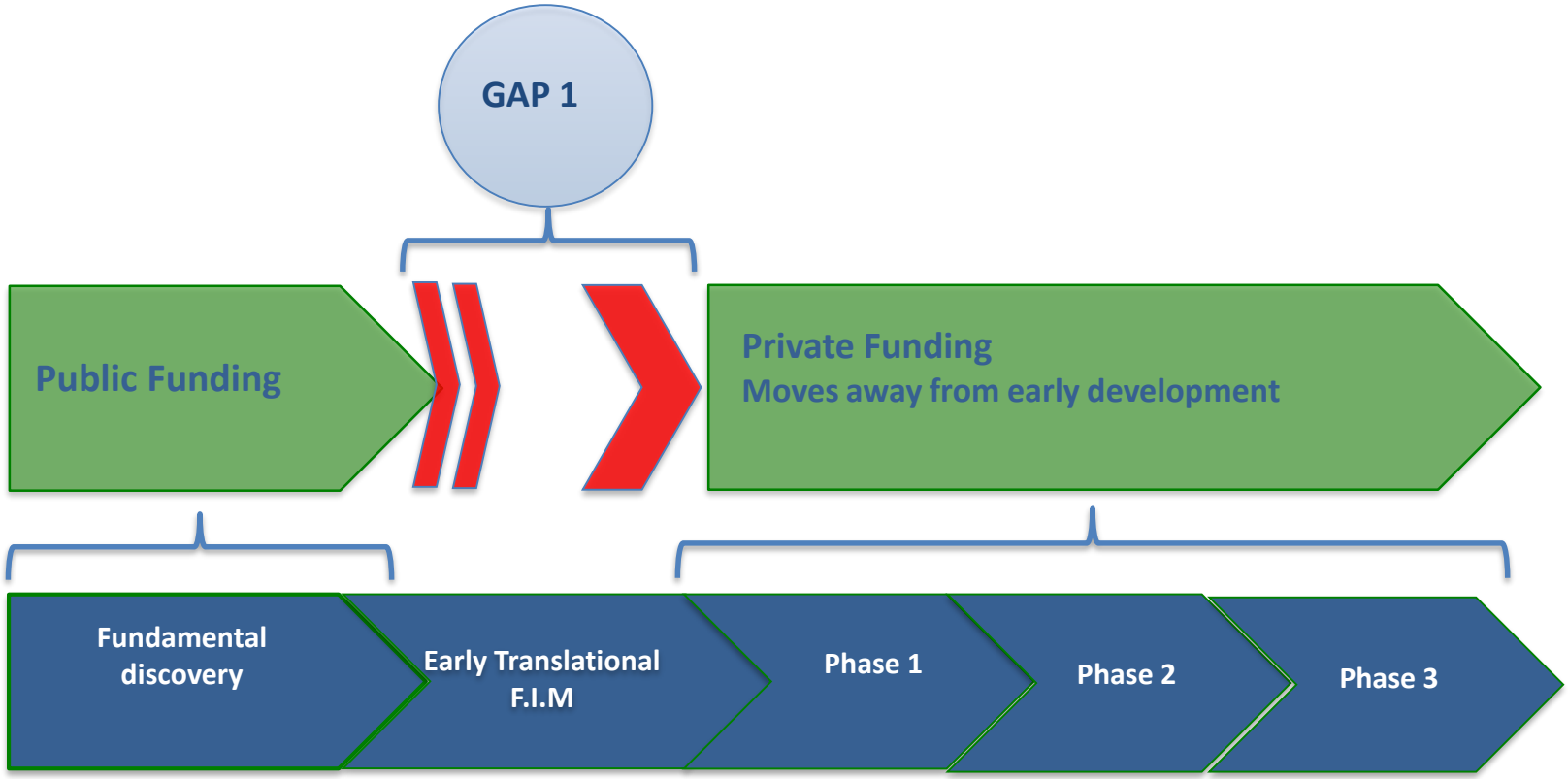


Industry Position

Spending on non-reproducible research



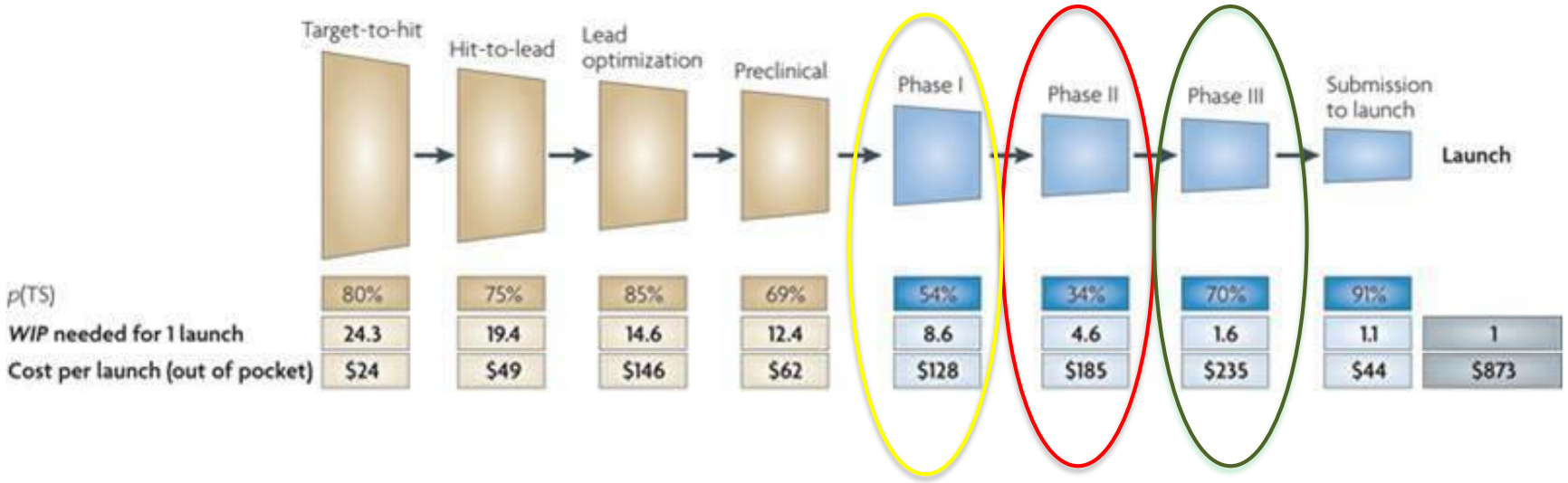
Current trends Biopharma Development Pipeline



GAP 1
Public increasingly funding early development, funding gap remains

Failing too late in development

Ca. 65% fail at phase II



Adapted from Nature Reviews Drug Discovery

Why are we failing in producing new drugs?

- Increasing regulatory requirements
- Poorly predictive non-clinical models
- Increasingly expensive therapeutic modalities

- Higher biological complexity of remaining unmet clinical needs

"I guess we should have tried it on the rats first."

Funders and regulators roles?

- Funders support basic science and early translational medicine
- Payers cover the cost of new therapies
- Regulators regulate the flow from basic to applied to market

Funder dilemma

- Institutional funders are moving slowly from a "sky is the limit" research model to a mix of basic science and Innovation.
- The move depends from macroeconomic factors as much as public pressure.
- The definition and evaluation system for INNOVATION is not harmonized between Funders, ranging from hard to soft approaches.

Impact assessment

- Stakeholders have various codified systems to assess the impact based on Key Indicators.
- The codified system might be quantitative or qualitative and have different indicators depending on the Stakeholder
- Institution holding position for research usually focus on Funding success and Publication Indicators (Impact Factors, H Index etc.) to assess the impact of single individual or departments and calibrate the rewards.

Regulators dilemma

- No limit to the safety assessment
- Separation between risk/benefit and cost/benefit assessment
- Same standards required for profit and non profit drug development
- Conflict of interest rules limit their involvement in academic enterprises

21st Century Cures Act -- FDA position

The 21st Century Cures Act (Cures Act), signed into law on December 13, 2016, is designed to help accelerate medical product development and bring new innovations and advances to patients who need them faster and more efficiently. […] It also provides new authority to help FDA improve our ability to recruit and retain scientific, technical, and professional experts and it establishes new expedited product development programs, including:

The Regenerative Medicine Advanced Therapy, or RMAT, that offers a new expedited option for certain eligible biologics products.

The Breakthrough Devices program, designed to speed the review of certain innovative medical devices.

In addition, the Cures Act directs FDA to create one or more intercenter institutes to help coordinate activities in major disease areas between the drug, biologics and device centers and improves the regulation of combination products.

PRIME Initiative

- PRIME is a scheme launched by the European Medicines Agency (EMA) to enhance support for the development of medicines that target an unmet medical need. This voluntary scheme is based on enhanced interaction and early dialogue with developers of promising medicines, to optimise development plans and speed up evaluation so these medicines can reach patients earlier.
- **Accelerated assessment**
- PRIME builds on the existing regulatory framework and tools already available such as scientific advice and accelerated assessment. This means that developers of a medicine that benefitted from PRIME can expect to be eligible for accelerated assessment at the time of application for a marketing authorisation.

Conditional marketing authorisation

- The European Medicines Agency (EMA) supports the development of medicines that address unmet medical needs of patients.
- In the interest of public health, applicants may be granted a conditional marketing authorisation for such medicines where the benefit of immediate availability outweighs the risk of less comprehensive data than normally required, based on the scope and criteria defined in legislation and guidelines.

Academic-Regulators interaction

- **"Framework for reinforced collaboration with academia adopted**

- The Board adopted a framework of collaboration between EMA and academia. The framework aims to reinforce and further develop the collaboration between the Agency and academia by clarifying the scope, and by formalising and structuring interactions in the wider context of the European medicines regulatory network."

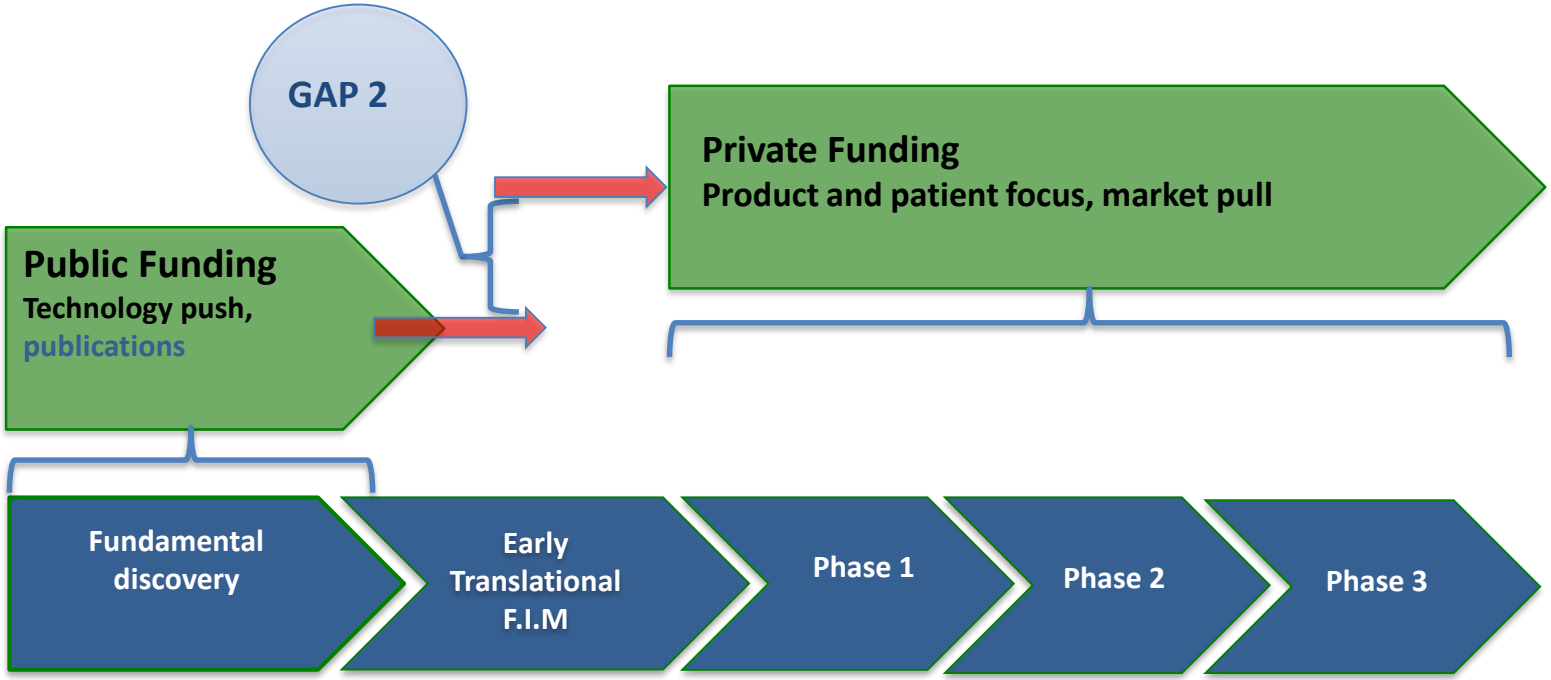
EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

17 March 2017
EMA/184615/2017
Media and Public Relations

Press release

EMA Management Board: highlights of March 2017 meeting

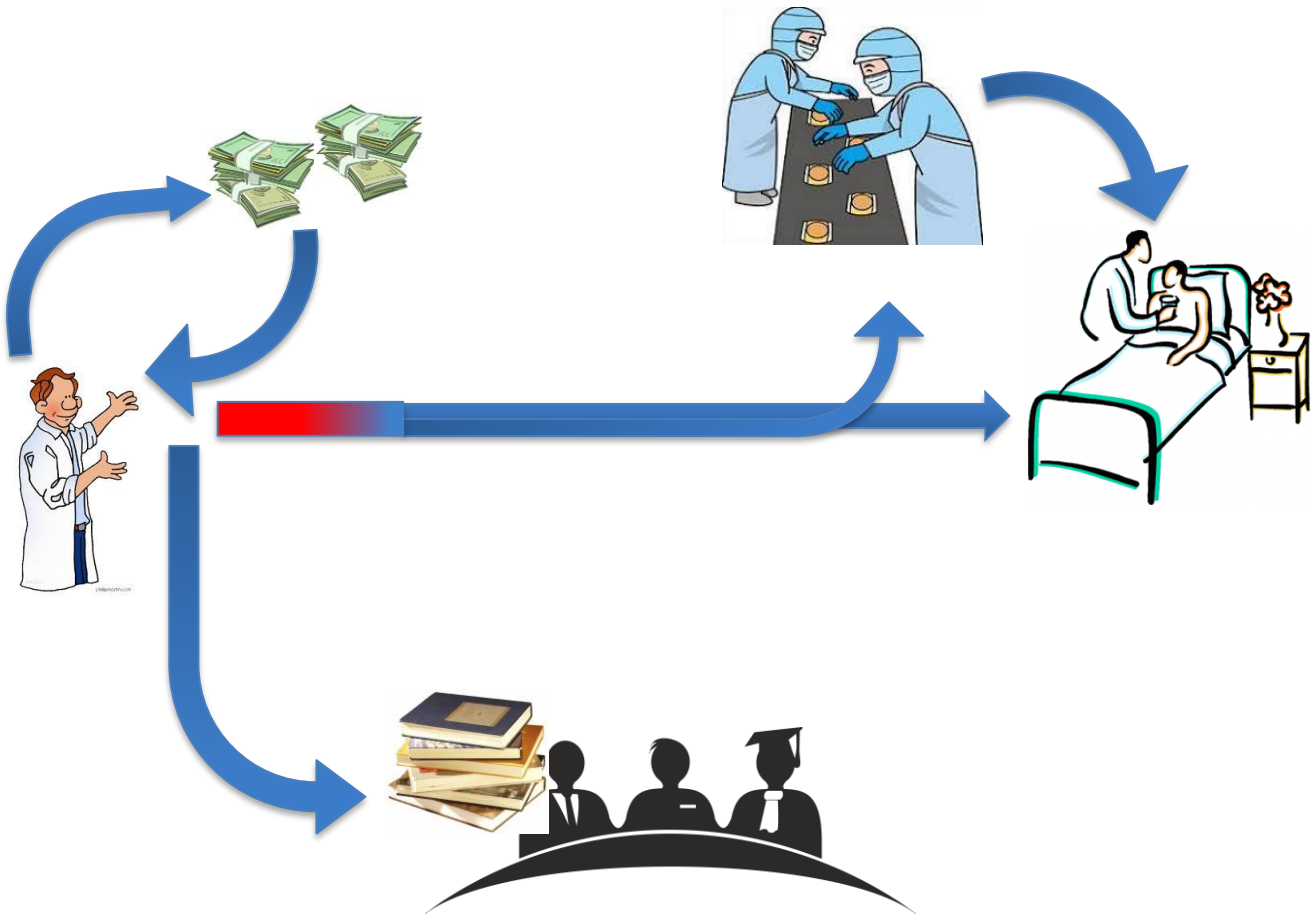
Current trends Biopharma Development Pipeline



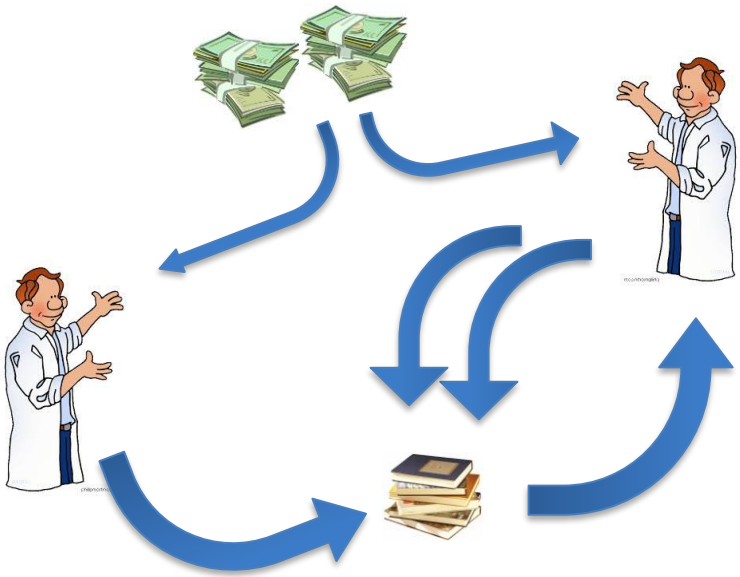
GAP 2

Outputs of publicly funded projects misaligned with privately financed requirements for further development

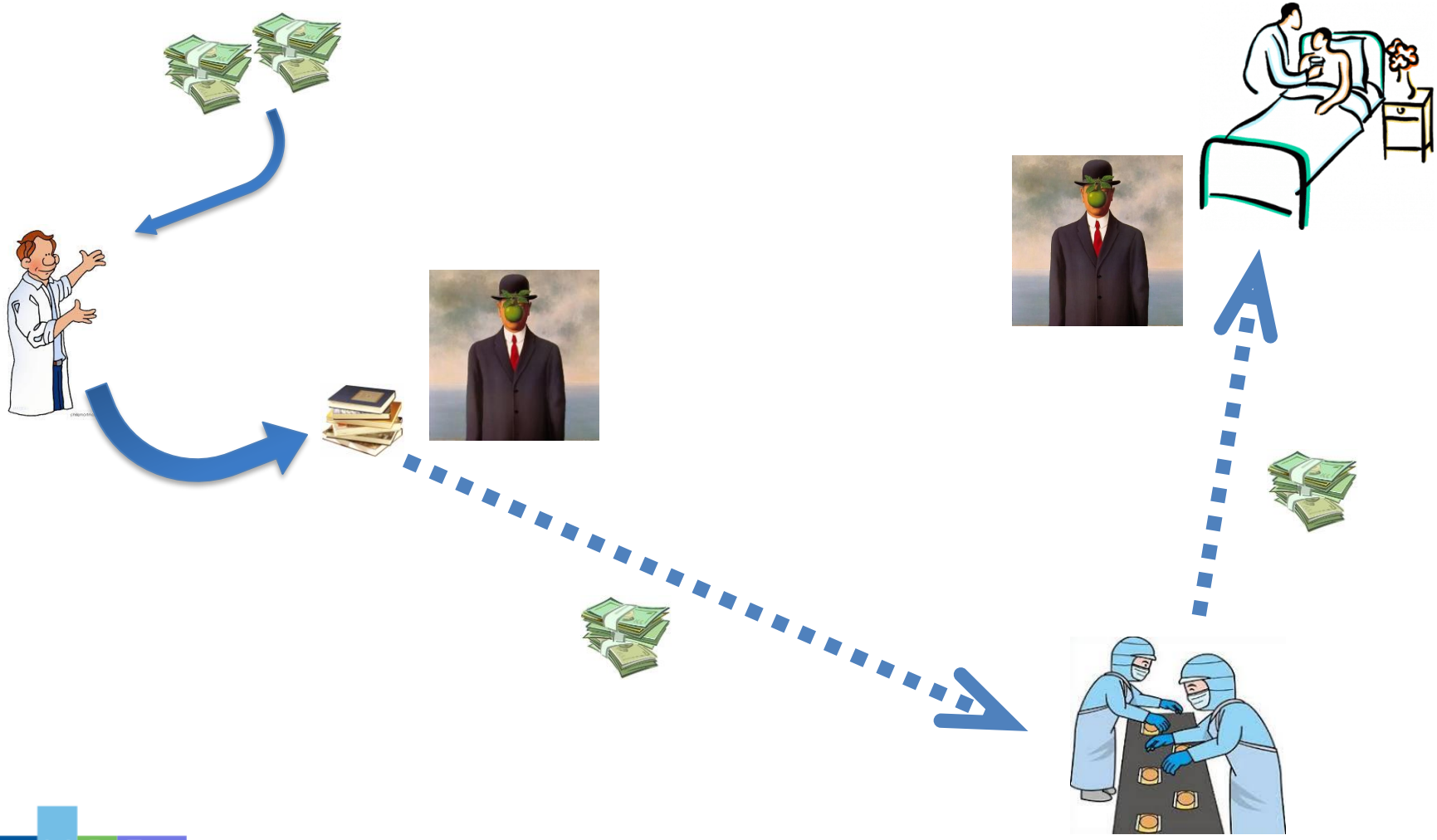
Where is the risk of late failure?



What is the validation process for academic discoveries?



What is the validation process for regulatory use?



External Providers for regulatory science?

- The validation of analytical methods or target leads need a robust data management under a quality assurance system.

- Academic institution are not funded to implement such a system and object that will limit innovation.

ESFRI program and the Research Infrastructures

- The European Strategy Forum on Research Infrastructures (ESFRI) was set-up in 2002.

- The ESFRI has a key role in policy-making on research infrastructures in Europe. In particular the ESFRI contributes to the development of a strategic roadmap that identifies vital new European RIs for the next 10-20 year

https://ec.europa.eu/research/infrastructures/index_en.cfm?pg=esfri

Integration of the services and research offered by 13 RI

Quality assured services offered to external stakeholder in various field of Bio-medical Research :

BBMRI-ERIC
EATRIS-ERIC
ECRIN-ERIC
ELIXIR
EMBRC
EMPHASIS
ERINHA
EU-OPENSCREEN
Euro-BioImaging
INFRAFRONTIER
Instruct-ERIC
ISBE
MIRRI

http://www.corbel-project.eu/home.html

Change the rules of the game……

- **Funders** – increase the surveillance on the quality assurance systems and on impact of *INNOVATION* studies

- **Institutions** – career reward criteria modified according to the *Impact criteria* of funders

- **Industry** – contribute in a PPP approach to non scientific management (i.e. quality assurance and market analysis)

Thank You!

Extra slides

Virtual Development Team

- VDT
  - Regulatory support – How to design a research so that will be compliant with regulatory requirements
  - Health Technology Assessment – Criteria and methodology for an early stage assessment
  - ELSI – Ethical and legal support