Regulator-Academia Relationships

Presented by Sergio Bonini

Professor of Medicine, Second University of Naples
Expert-on-Secondment, European Medicine Agency

University of Southern California, Los Angeles June 19, 2014
Disclaimer

The views expressed in this presentation are the personal views of the speaker and may not be understood or quoted as being made on behalf of or reflecting the position of the EMA or one of its committees or working parties.

© European Medicines Agency, 2013. Reproduction is authorised provided the source is acknowledged.
ACADEMIA

INDUSTRY
Where innovation comes from

Figure 1 | Origin of new medicines in the European Union

Current EU pathways are expensive and slow in getting new therapies to patients

Pre-Clinical Research

Drug Discovery

Pre Clinical Testing

Pre-Clinical Research & Open Innovation

5,000
10,000
Compounds

250
Compounds

5
Therapies

1
Therapy

3 – 6 Years

Clinical Trials

Phase 1

Phase 2

Phase 3

Clinical Trials

Number of Patients/Subjects

20 - 100

100 - 500

1,000 - 5,000

6 – 7 Years

EMA Filing

EMA Approval for Sale

HTA Approval

Negotiation for Reimbursement

27 member States

PhV Monitoring

Total Cost: $2 - $4 Billion USD

First authorization of a CFTR modifier

Discovery/Manufacture
Pre-clinical development
Clinical development

Orphan Drug Designation (July 2008)
Protocol assistance (Oct-Dec 2010)
MAA (27 Oct 2011)
CHMP MA 24 May 2012

Very few chloride ions move into or out of the cell, leading to thick, sticky mucus

Mean Absolute Change (± SD in % Predicted FE) vs Study Weeks

PLACEBO
VX-770
Everyone wants to be found.

BILL MURRAY  SCARLETT JOHANSSON

Lost In Translation

The new film written and directed by Sofia Coppola
Reasons for reciprocal distrust between regulators and academia

From one side

• Regulators believe that academics are carriers of unacceptable conflicts of interests because of their preferential relationships with industry

From the other side

• Academics believe that regulators are bureaucrats, to be treated with the same respect due to dogwatchers.

“...the regulatory environment....is so strict to be, frankly, punitive”

Sir Mark Pepys, Eur Drug Target Rev 2014;1:34-36
Reasons for collaboration between regulators and academia

- Biologics, biosimilars, stem cell therapies, gene therapies as well as stratified medicine ask for a close collaboration between regulators and academia in order to face the complex challenge in research, development, evaluation, use and monitoring of these innovative drugs and approaches to treatment.

- Without collaboration, regulators risk to be confined in a close environment, unaccessible to scientific progress, while academics will not fully appreciate what is requested to allow translation and innovation.
Initiatives implemented at the EMA in order to facilitate communication and collaboration between EMA and Academia, with the final aim of enabling an early access for patients to innovative medicines

(Rasi G. Eur Drug Target Rev 2014;1:7)

• Revision of the policy on CoI
• Early involvement in the regulatory pathway (HCPWG, SA, adaptive licensing, joint HTA SA) and in post-registrative trials
• Communication/Education
• Joint independent research projects
• Open data
• Big data
Current scenario:
Post-licensing, treatment population grows rapidly; treatment experience does not contribute to evidence generation.
Adaptive licensing: after initial earlier license, number of treated patients grows more slowly, due to restrictions; patient experience is captured to contribute to real-world information

*Courtesy of Hans-Georg Eichler, EMA Senior Medical Officer*
EMA-HTA parallel SA: Experience so far

• **35 parallel EMA–SA procedures**
  with EU HTA bodies from UK, Italy, Germany, Sweden, France, Netherlands, Spain, Belgium

• **Broad range of indications:**
  Lung cancer, Breast cancer, Pancreas cancer, Melanoma, Asthma, COPD, Diabetes, Heart Failure, Depression, Alzheimer’s, Infections, Rare diseases
Regulatory Sciences Initiatives

- HCPWP Health Care Professionals Working Parties
- Special Office for SME, One-stop shop
- PEERS
  - Exchange of information in drug R&D, evaluation, use and monitoring
  - Harmonized training
  - Clinical trials
  - Exchange of personnel
  - Data analysis
  - Joint access to funding (IMI2, Horizon 2020)
Access to document

- Policy published November 2010
- Average 450-500 requests each month (5,786 in 2013)

Pro-active publication

- After public consultation and 3 Workshops with all Stakeholders, the Policy has been submitted for approval by the EMA MB on June 12, 2014.
- First phase: publication of CSRs
  Second phase: publication of IPD
Access to document requests:
November 2010 – December 2011
Access to document requests: January – December 2013

Transparency in the pharmaceutical sector – what have we learned?
Nobody is the owner of the truth!
The challenge ahead: omics and “Big Data”

• Massive amounts of data
• New kind of follow up, with new medical challenges

Data will be available

• But does this fit into the existing health care products processes?
• Who is there to monitor & interpret this data?
• Are physicians ready to use data collected by their patient?

(Modified from F. Ehman, EMA)
With the final aim that patients may have an early access to effective, safe and affordable drugs.