Feedback from conference ‘Medicines for rare diseases and children: learning from the past, looking to the future’

83rd Pharmaceutical Committee
11 July 2019

Health and Food Safety

Outline

1. Programme

2. Feedback from the break-out sessions

3. Next steps

European Commission

17 June 2019

- **Introductory speeches**
  - Commissioner Health and Food Safety
  - Former MEP Françoise Grossetête
  - 'Scene setter' European Commission and EMA

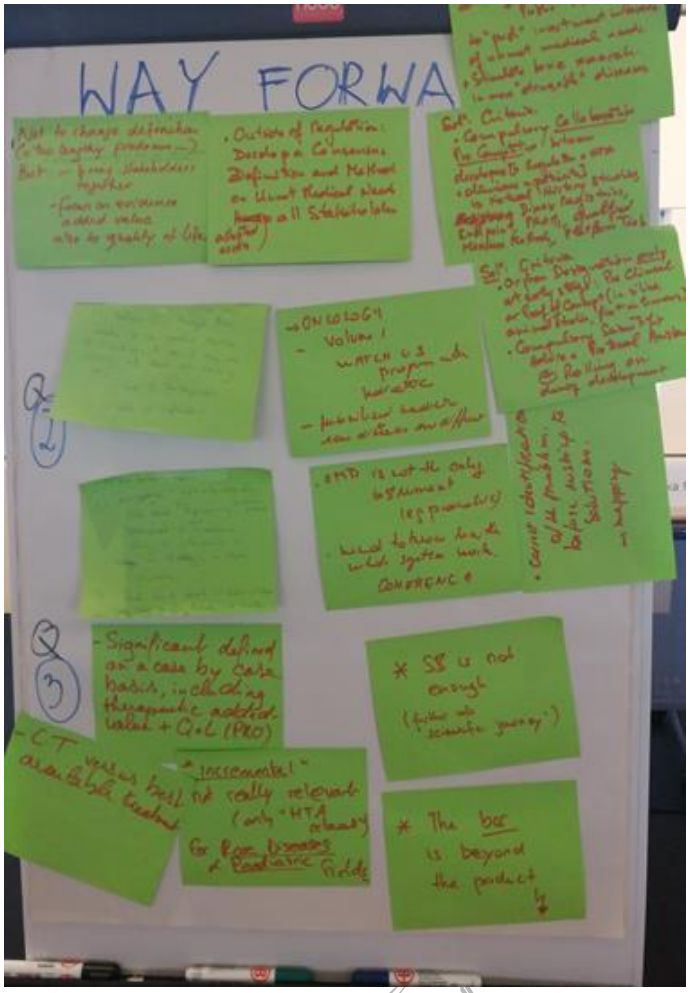
  - **Breakout sessions**

    1. Unmet medical need
    2. Incentives
    3. Medicines for children
    4. From R&D to patients
    5. Scientific developments

- **Open Space (all participants)**

Breakout sessions

Some of the ideas harvested in 5 breakout sessions….



European Commission

Unmet medical need

- Common understanding and quantification of unmet medical need
  - **Input "expert patients" to define unmet needs**

- Global cooperation and data sharing (medicines for children)

- Early granting orphan designation (concept stage)

Incentives

- Need for incentives
  - **supporting real innovation in orphan landscape**
  - **incentivise collection and sharing of data**
- Connection between financial reward and cost of development not always clear
- Need for better coordination and identification of priorities
  - **Financial rewards and incentives not only solution to improve situation**
  - **Stimulation of basic research?**

Medicines for children

- **Return on investment** → important factor for paediatric developments
- **Paediatric 'Masterplans'** → all stakeholders on-board
- **'Orphan-like' designation**: for conditions not meeting orphan criteria (but small subpopulations with special formulation)
- **Two regulations not aligned** and proportionate in relation to definitions → review of rewards and incentives?

From R&D to patients

- Academia limited knowledge of regulatory requirements and incentives

- Need for **more basic research** in general and broader sharing and dissemination of data

- Focus Real World Evidence on overcoming lack of consistent data availability and development of standardised infrastructures

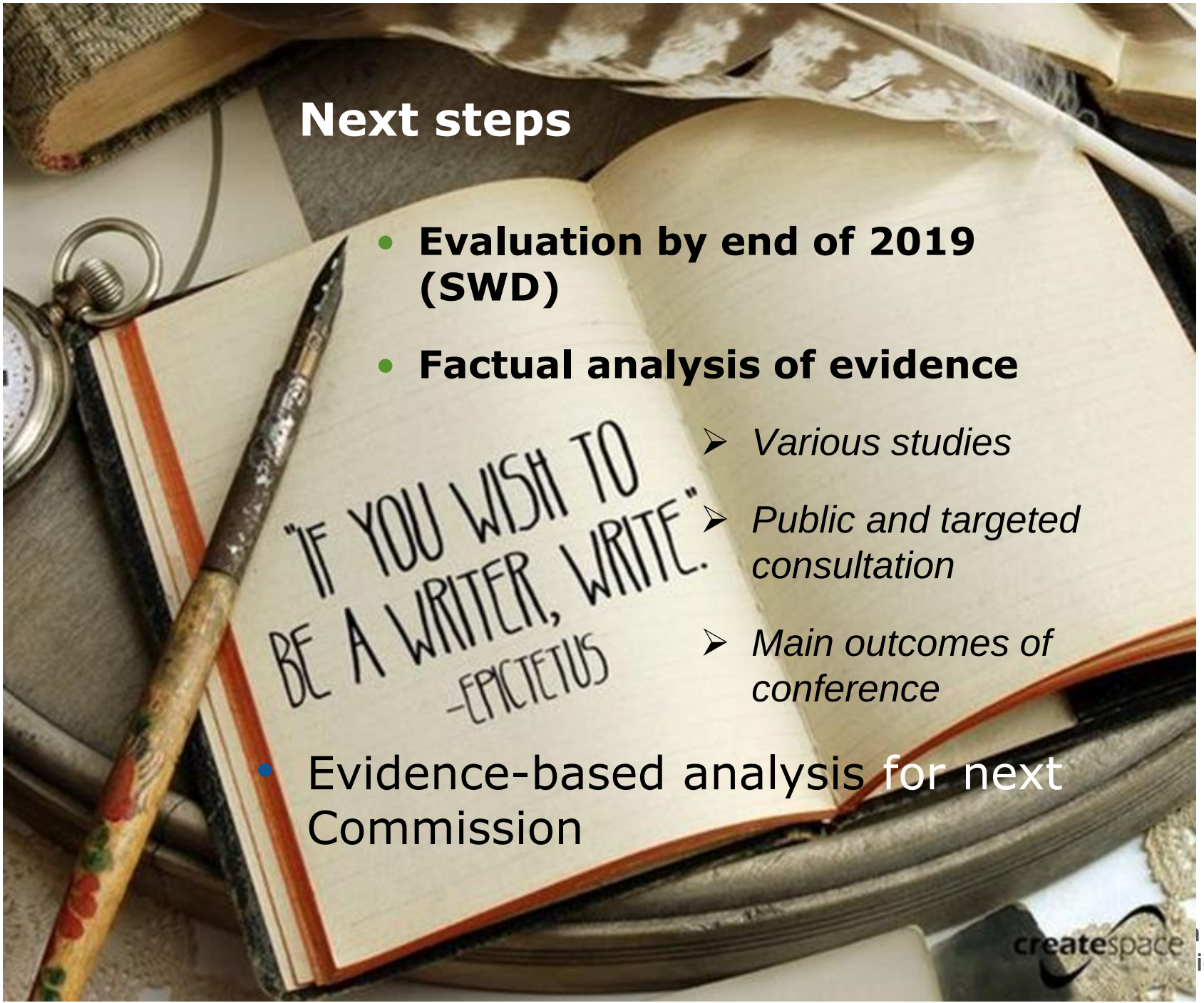
Scientific developments

- Better link needed between genetic sequencing, biological data and outcomes

- Better **cross-committee operations** at EMA

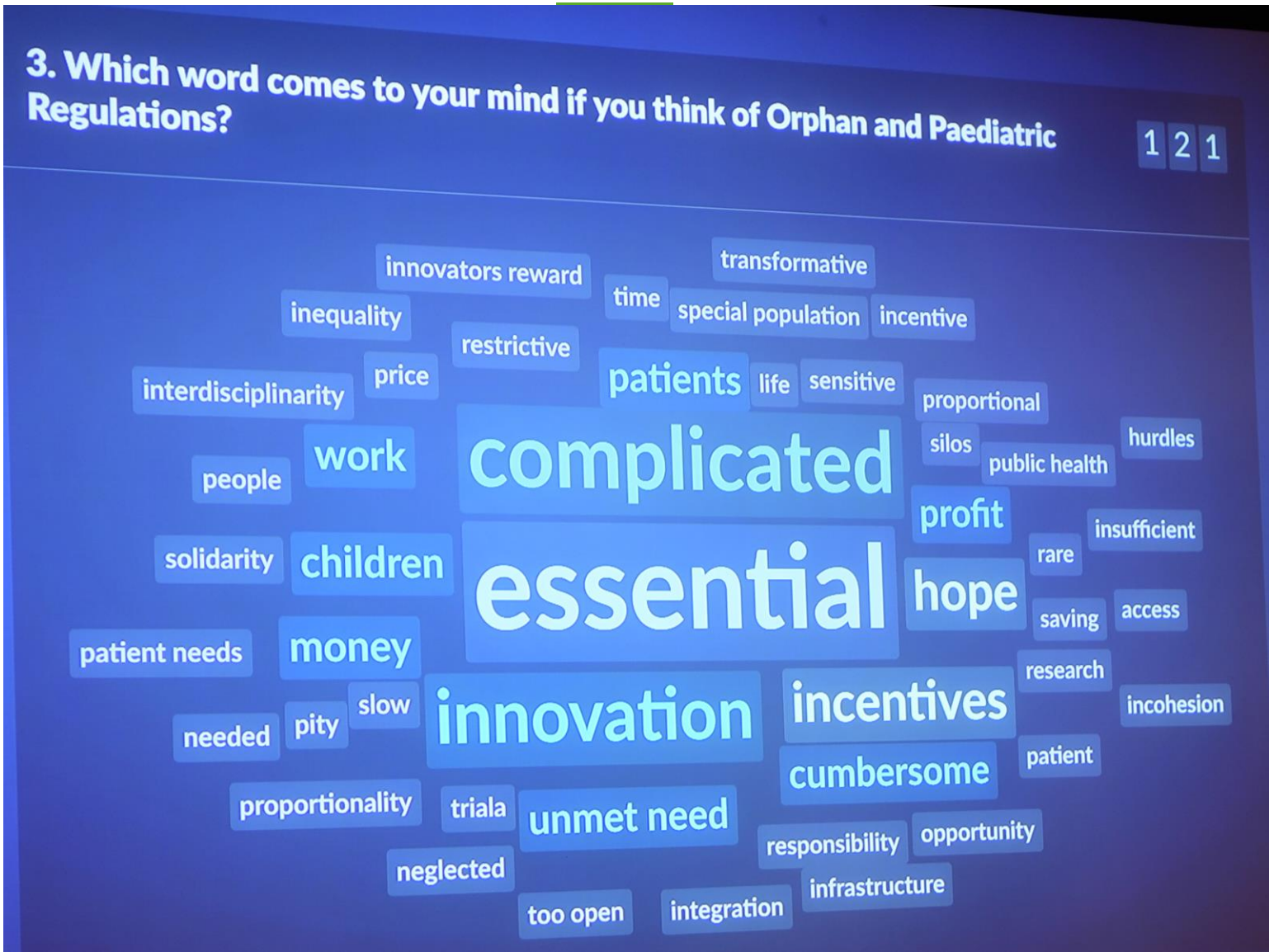
- Need for **multi stakeholder engagement** → change of evidence standards and scientific advances

- Revision definition of 'orphan medicine'?
  - **not only about condition or prevalence but number of patients treated**



Next steps

- **Evaluation by end of 2019 (SWD)**
- **Factual analysis of evidence**
  - *Various studies*
  - *Public and targeted consultation*
  - *Main outcomes of conference*
- Evidence-based analysis for next Commission


3. Which word comes to your mind if you think of Orphan and Paediatric Regulations?
1 2 1
innovators reward
transformative
time
special population
incentive
inequality
restrictive
price
patients
life
sensitive
proportional
interdisciplinarity
work
complicated
silos
public health
hurdles
people
profit
insufficient
solidarity
children
essential
rare
hope
saving
access
patient needs
money
research
incohesion
slow
innovation
incentives
needed
pity
cumbersome
patient
proportionality
triala
unmet need
responsibility
opportunity
neglected
infrastructure
too open
integration