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

83rd Pharmaceutical Committee
11 July 2019
Outline

1. Programme
2. Feedback from the break-out sessions
3. Next steps
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....
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?