EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Breakout session 4

Special Areas: Orphan drugs / ATMPs / Paediatrics / Personalized medicines / Vaccines

Moderators

- **Regulatory:** Bertil Jonsson
- **HTA:** François Meyer
- **Industry:** Thibaut du Fayet

Objectives

Scope: **Special scientific, procedural considerations and requirements for limited populations, OR particular situations** (Orphan drugs, ATMPs, Paediatric, Personalized medicine, Vaccines)

1. **Main issues**
2. **Current gaps**
3. **Possible short & long term solutions**

Main issues

- **Target population indication specificity** (Orphan, Paediatrics, Personalized medicine): *limited and/or specific population*
- **Complex therapeutic interventions** (ATMPs, Orphan, Paediatrics, Personalized medicine): *high product complexity with new MoA, often in « niche » diseases, with increasing co-developments (Rx/DX)*
- **Development & Market access burden** (Orphan, ATMPs, Paediatrics, Personalized medicine): *level of requirements similar to standard products (assesment process & guidelines)*
- **Companies lack of expertise** (Orphan, ATMPs, Paediatrics): *Innovative SMEs, often positioned in these particular situations, with limited internal expertise & dedicated resources*
- **Vaccines specificities** (Vaccines): *medicinal products, part of public health intervention but with today no specific HTA / P&R evaluations*

Potential solutions

Category	Proposals
Scientific	• Anticipate prospective meta analysis • Data on non-clinical models • Pharmaceutical paediatrics formulations
Process	• Early HTA value assessment (non binding) within the scientific advice • Methodological guidelines alignment between HTA / EMA • EMA / HTA parallel Qualification advice for novel methodologies • HTA to attend EMA working parties / committees? • Share of expertise network, managing conflict of interest • Make use of the EU experts network (meta network Enpr-EMA, EUCERD, Paediatrics) • Reassessment of Long Term follow-up to be discussed during early parallel advice (ATMPs)
Policy	• Make transparent contribution to advice from COMP and PDCO • Disease Guidelines production, based upon EMA existing guidelines • Patients involvement • Organizational challenges (ATMPs)