European Commission

Conference

"Medicines for rare diseases and children: learning from the past, looking to the future"

17 June 2019

SQUARE – Brussels Convention Centre

Draft programme

Health

CONFERENCE
“MEDICINES FOR RARE DISEASES AND CHILDREN: LEARNING FROM THE PAST, LOOKING TO THE FUTURE”

Moderator

Patrick Deboyser,

Professor, European College of Parma

Participatory Leadership Facilitators

Dana Adriana Puia Morel

Mariana Ghițoi

European Commission

MORNING

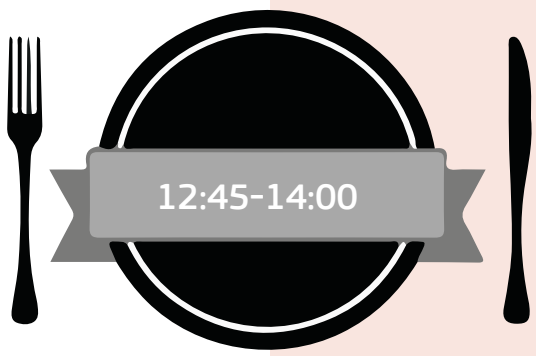
08:45–09:30 — **Registration and welcome coffee**

09:30–09:35 — **Opening of the day by the moderator**

09:35–09:55 — **Keynote opening speech**
Commissioner for Health and Food Safety – **Vytenis Andriukaitis**
Member of European Parliament – **Françoise Grossetête**

09:55–10:10 — **Scene setter for medicines for children and rare diseases**
Head of DG SANTE Unit B5, Medicines: policy, authorisation and monitoring – **Olga Solomon**
Head of Product Development Scientific Support Department in the European Medicines Agency - **Michael Berntgen**

10:10–12:45 — **Break-out sessions**
1- Unmet medical need
2- Incentives
3- Medicines for children
4- From R&D to patients
5- Scientific developments



AFTERNOON

14:00-14:30 — **Debrief from Break-out sessions**

14:30-16:30 — **Open Space – Thinking Together**
Discussion on topics coming directly from participants

16:30-16:45 — **Conclusions from Open Space by the moderator**

16:45-17:00 — **Closing speech and next steps**
Director-General of DG SANTE – **Anne Bucher**

BREAKOUT SESSIONS

Unmet Medical Need

1. Unmet Medical Need: does it mean the same thing to everyone? How to define and better address this need?
2. Orphan condition: how can we support the development of medicinal product for patients still without treatment?
3. Significant versus incremental benefit: how strictly should we follow the EU orphan legislation when applying this principle?

Incentives

1. Market exclusivity (ME): how successfully has this incentive supported innovation? Has there been any unintended impact for availability and patient access?
2. Reward for investment: how can we better guarantee that the reward is proportionate to the return on investment without knowing the cost of development?
3. Other incentives: what other actions could be considered for areas without treatment?

Medicines for children

1. How can we ensure an optimal support for the development of medicines for children without hampering research driven development of medicines for adults?
2. Why are there so few “paediatric only” medicines?
3. EU orphan and paediatric legislation and the wider context: how well do the two regulations interact, including with other national and European policies?

From R&D to patient

1. R&D: what are the main hurdles when developing orphan and paediatrics medicinal products?
2. Accessibility: how can public money for research be better invested in areas of unmet need? How can we make sure that medicines developed based on public funded research will reach patients across the EU?
3. Real World Data/Real World Evidence: how will it influence the development, authorisation and access to new medicines?

Scientific developments

1. What are the main scientific advances, which will influence future pharmaceutical product development? How will personalised medicines impact the landscape of orphan medicines and beyond?
2. What does the innovation pipeline tell us and can the EU orphan and paediatric legislation accommodate these developments in a data driven society?
3. How might scientific and technological developments affect the EU orphan and paediatric legislation?