EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Follow-up from the EC/EMA workshop on paediatrics

3rd Industry Stakeholder Platform on R&D support, 18 May 2018

Presented by Ralph Bax
Head of Paediatric Medicines Office

Workshop to improve implementation of Paed Regulation

- Broad participation of all relevant stakeholder groups (~160 participants):
  - Patients/carers
  - Academia (incl. networks)
  - Health Care Professionals
  - Industry
  - EMA/PDCO/EC
  - FDA
  - CTFG
  - WHO
  - EP
  - Ethics committees

Topic areas discussed

1. Identifying paediatric medical needs (methodology)
2. Ensuring timely completion of PIPs
3. Improving the handling of PIP applications
4. Strengthening of international cooperation between regulators
5. Increasing transparency around paediatric medicines

1. Identifying paediatric medical needs (methodology)

- Assessment of the disease burden
- Characterisation of the properties of medicinal product concerned
- Availability of scientific understanding of relevant research area
- Alignment of actions by national and international partners

2. Ensuring timely completion of PIPs

- Optimisation of development programmes from early stages onwards:
  - Knowledge/information sharing between all relevant stakeholders (patients, academia/research, networks, industry)
  - Consideration and early regulatory discussions of trial designs and methodologies
  - Optimise the estimation of patient availability
  - Consultation and involvement of patients and young people along the drug development process
- Clinical trials:
  - Guidance for planning clinical trials
  - Sustainable infrastructure and funding
- Training and exchange of information between assessors of clinical trials (NCAs), ethic committees and regulators (involved in PIP-process and marketing authorisation).

3. Improving the handling of PIP applications

- Optimisation of procedural guidance
- Optimisation of submission requirements
- Evolutionary approach to PIP agreement

4. Strengthening of international cooperation between regulators

- Initiatives to further increase cluster interactions
- Collaboration in the context of molecular targets for paediatric oncology
- Collaboration in the context of unmet paediatric needs

5. Increasing transparency around paediatric medicines

- Community register of medicinal products for human use to include PIP information
- Implementation of the Clinical Trial Regulation

Publications of workshop outcome

Presentations and video recording:

- Already published on EMA website: News & events

Workshop report:

- Publication planned by end of May

Development of Action Plan

EC/EMA Action plan to cover 2 years

- Working groups (EC,EMA & PDCO) on topic areas
- Discussion and finalisation at PDCO meetings in coordination with EC.

Publication of Action Plan planned by end of July