Session 3:
Timely completion of the studies of a paediatric investigation plan (PIP)

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Timely completion of the studies of a PIP

A multifaceted challenge…

Finding the right indication and population	Lack of sufficient trial infrastructure	Use/acceptance of innovative study designs
Divergent view of Ethic Committees	Contradictory local regulations	Diverse standard of care across Europe
Small patient populations – competing developments	Impact on daily lives of patients and families	Dose, route of administration, application device

Timely completion of the studies of a PIP

Elements experienced as obstacles – during planning & design

PIPs often agreed before sufficient information from adults are available

- Limits scientific discussion on right indication, population and endpoints
- Limits understanding of viability of clinical program
- Leads to multiple, time and resource consuming modifications

Lack of acceptance of innovative study designs & new digital approaches

- Missed chances to reduce patient numbers while increasing value of generated data (e.g. adaptive designs, extrapolation)
- Increased burden to patients, parents and sites
- Time and resource consuming study procedures

Diverse medical practice and standard of care often not valued sufficiently

- Different medical practice and standard of care at sites
- Disrupts life of patients, parents, and caregivers

Improving the efficiency of PIPs through more scientific and regulatory dialog

- Global alignment on scientific approach
- Extended expert base to agree on PIP and viability of clinical program
- “iterative PIP process” not affecting time of PIP submission
  -> relates to session 4

Foster innovative approaches

- Multi-stakeholder Workshops to discuss suitable approaches and foster global alignment
- Provide clear guidance for use of new study designs and technical innovation
- Early, open scientific dialog between sponsors, PDCO and experts on value of using such approaches

Reduce the burden on patients, investigators and sites

- Regular, early involvement of Young Patient Advisory groups, parents and networks
- Allow more flexibility of study procedures following standard of care

Timely completion of the studies of a PIP

Elements experienced as obstacles – during – start-up and conduct -

Lack of acceptance of agreed PIP by local HA or Ethic Committee

- Contradicting regulations across single countries delaying implementation of study (e.g. different rules for ICF/Assent)
- Non alignment of ECs across countries/ sites pose high hurdle for implementation (e.g. inclusion of Adolescent in adult studies)

Scattered, insufficient clinical trial infrastructure

- Competition about resources between industry and academic research
- Delay in study conduct and finalization
- Delay in availability of innovative Medicines to children

Lack of appreciation of need for clinical research in children in society

- High (emotional) hurdle for parents to allow study participation of their child

Better alignment across EU member states

- Ensure EU Clinical trials directive is implemented consistently
- Foster harmonization of local regulations surrounding clinical research in Children
- Harmonized processes for patient information and consent/assent

More efforts needed to build-up sufficient trial infrastructure

- Number of initiatives ongoing, incl. IMI initiative to create pan European Paediatric Clinical Trials Network
- Foster government initiatives in each member state to increase quality and capacities and ensure sustainability

Foster better information and education of the public on need for more research in Children

- Make the processes safeguarding these activities more visible
- Give Young Patient Groups and Networks a role

Timely completion of the studies of a PIP

Measures to be taken - summary

- **Iterative PIPs**
  - Built progressively with earlier, better scientific and regulatory dialogue
  - Better understanding and utilising the standards in paediatric care
  - Readily acceptable by National Competent Authorities and EC
  - Allow for more readily adaptation to evolving data
- **Foster use of innovative approaches for study design and/or data collection**
  - Scientific Workshops to define suitable approaches and provide clear guidance for use
- **Sufficient infrastructure for the conduct of paediatric clinical trials in Europe**
- **A better education of society as a whole of the benefits of paediatric research and measures to ensure safety during the trials**