IMI IMPACT on PAEDIATRIC MEDICINE

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Challenges with Paediatric Drug Development

• Conceptually
  • Unmet medical needs
  • Target population
  • Timing

• Content
  • Standard of Care
  • Study design – endpoints; placebo; use/acceptance of complex innovative designs, decentralised trials, RWD...

• Operational
  • Different regulations
  • Research network infrastructures and capabilities
  • Trial enrolment
As a result...

~60% of Paediatric Trials Have Not Yet Enrolled Patients
The value of IMI Public Private Partnerships

• Public Private collaboration bringing together all involved in drug development, and who are now used to work together in the pre-competitive space

• To address complex areas, relevant to public health needs

• Generate high-quality science

• European focus but global impact

Adapted from Nathalie Seigneuret, IMI
IMI – Advancing Paediatric Research

• Addressing childhood cancers
• Building clinical trial networks and improving their design
• Identifying biomarkers and driving personalised medicine
• Tackling infectious diseases in children
• Putting parents and children at the centre of research

IMI is pushing the boundaries of science to develop faster, better and more personalised treatments for childhood illnesses.

The impact of this work will be felt by children, their families and wider society for decades to come.
IMI – Advancing Paediatric Research

Innovative trial designs, e.g. Master Protocol
Use of RWD, Big Data, Artificial Intelligence...

Pre-Clinical

Autism  Resp. Syncitial Virus  Neurofibromatosis  Blood cancers  Type 1 Diabetes  Rare diseases

Clinical

Autism  Pregnancy & Breast feeding
To conclude

- A lot has been achieved already through IMI.

- Gap analysis should be performed to agreed collectively where collaborative research would be useful and could help progress paediatric drug development and children access to transformative medicines.

- The future of pediatric research depends on concerted action to develop a science-driven research, research infrastructure and true engagement with children, young people, and families.

- This needs support from all, including policymakers, regulators and the broad child health community.