AstraZeneca

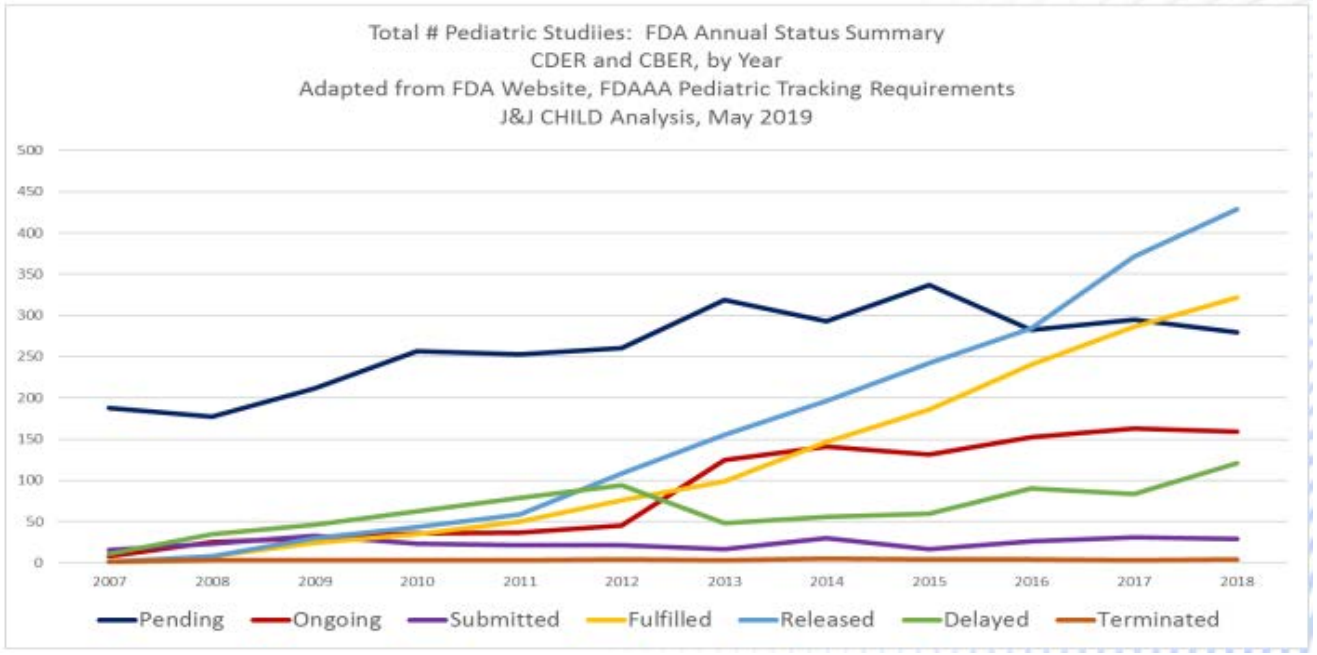
IMI IMPACT on PAEDIATRIC MEDICINE

Dr Solange Corriol-Rohou, M.D.

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

conect 4children
COLLABORATIVE NETWORK FOR EUROPEAN CLINICAL TRIALS FOR CHILDREN

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

conception
SAFETY EVIDENCE ECOSYSTEM

ITCC P4 PAEDIATRIC PRECLINICAL PROOF OF CONCEPT PLATFORM

EU-AIMS
Autism Research in Europe

RESCEU
REspiratory Syncytial virus Consortium in EUrope

HARMONY

EU-PEARL
EU PATIENT-CENTRIC CLINICAL TRIAL PLATFORMS

AIMS2TRIALS

INNODIA
A PRIVATE PUBLIC PARTNERSHIP AGAINST TYPE 1 DIABETES

SCREEN 4CARE

Pre-Clinical → **Clinical**

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