

INTERNATIONAL CLINICAL TRIALS DAY

May 20th 2020

PAEDIATRIC MEDICINES & CLINICAL RESEARCH IN NUMBERS

Why clinical trials in paediatrics are so important



OFF-LABEL USE OF MEDICINES IN CHILDREN

~50%



INNOVATIVE MEDICINES

199 vs 563

New active substances with paediatric indications (1996-2018)

New active substances for adult population (1996-2018)

INVESTMENT IN PAEDIATRIC R&D

2.1 billion €
(5% of total R&D)



Total cost per year

Sources:

- Report from the Commission to the European Parliament and the Council" COM (2017) 626 "State of Paediatric Medicines in the EU 10 years of the EU Paediatric Regulation"
- European Paediatric Medicines Database (EPMD) managed by TEDDY - <https://www.teddynetwork.net/european-paediatric-medicines-database-epmd/>



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The Nuremberg Code (1947) excluded children from clinical trials in order to protect them from potential research risks . Until the 1980s, it was often argued that children should be protected from clinical research for ethical reasons.

Since the 1980s and with Regulation (EC) No. 1901/2006 on medicinal products for paediatric use, there has been a shift from protecting children **from clinical research** to protecting them **with clinical research**.

There is now consensus that **children must have the same rights as adults and access to high quality, evidence-based medication** and health care services.



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The lack of clinical trials for children entails the off-label use of adult medicines, with risk of inefficacy and/or adverse reactions, as well scarcity of new and innovative paediatric medicines, which are key to safeguard children and young people's health.

Many of the products used in children are prescribed and administered based on physician's own experience rather than on the results of research.

Increasing paediatric research and the number of new products with specific paediatric indications will ensure that off-label use of adults medicines will decrease and innovative medicines will be available to children and young people.



Children are not small adults!

Paediatric population often responds to drugs differently than adults do.

Children's bodies respond differently to treatments, they have different opinions, and what matters to them may be different to what matters to the adults around them.

Children grow and mature – the biological and physiological characteristics and needs of neonates compared to teenagers are different. Therefore age-appropriate research is often needed.



Paediatric clinical trials have well-known complexities:

- **Safety** concerns
- **Ethical** considerations
- **Lower prevalence of disease**
- Need to test different **age groups**
- **Tailored study** design
- **Child-appropriate medicine formulations.**

Clinical trials addressed to children tend to be multi-centre trials, since the pool of eligible children for trials is often small. Having **few patients per site** often creates **operational challenges, recruitment difficulties**, which can lead to delays in conducting and completing them.



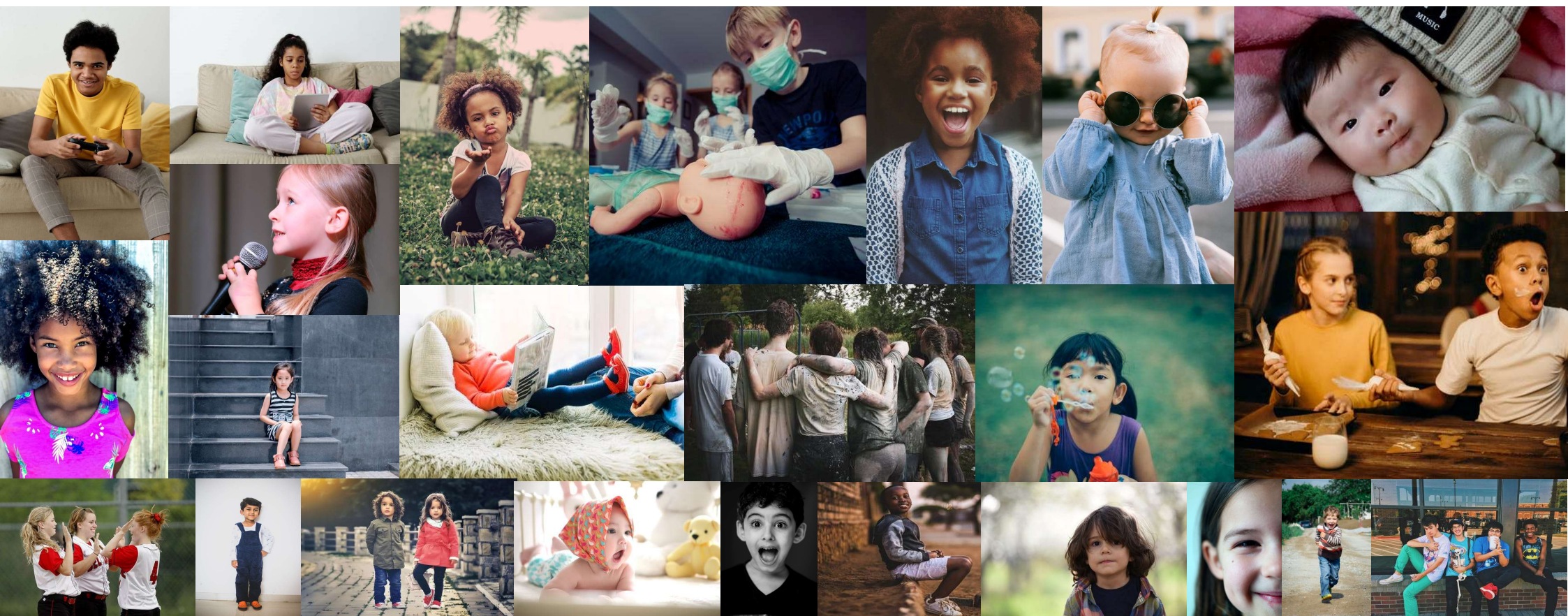
Paediatric clinical trials are essential to ensure that children receive appropriate, safe and effective treatment and care

c4c is committed to meeting the needs of paediatric patients and building capacity for the implementation of **multinational paediatric clinical trials**.

This large collaborative **European network** aims to facilitate the development of new drugs and other therapies for the entire paediatric population.



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Better medicines for babies, children and young people through a pan-European clinical trial network