



Topic for MSP AG meeting

**Innovative Study Designs in Paediatrics:
limited practical implementation**

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European Network of Excellence for Paediatric Research

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Innovative Study Designs in Paediatrics

Nature of the issue: Operational

Background

Innovative methodological approaches for study design such as Master Protocols (Seamless, Umbrella and Basket) and Adaptive studies play a crucial role in improving clinical trials and medicine assessment in paediatrics by ensuring the feasibility of the study and the validity of the results.

Advantages

- Increase of Efficiency
- Improve of Ethical Standards
- Enhance of Data Quality and Reliability
- Personalized Medicine
- Optimization of Resources
- Patient-Centric Approaches
- Safety Monitoring
- Broader Application

Critical challenge faced

Although the fundamentals of these innovative approaches have been already streamlined in comprehensive scientific and regulatory guidelines and accepted at the regulatory level long ago, their implementation in clinical research practice remains very limited.

References:

- [EMA-CHMP, 2007 - Reflection paper on methodological issues in confirmatory clinical trials planned with an adaptive design](#)
- [EMA, 2014 - Qualification of novel methodologies for drug development: guidance to applicants](#)
- [EMA-CHMP, 2006 - Guideline on clinical trials in small populations](#)
- [EMA, 2016 - Guidance for companies considering the adaptive pathways approach](#)
- [FDA, 2019 - Adaptive Design Clinical Trials for Drugs and Biologics Guidance for Industry](#)
- [FDA, 2019 - Enrichment Strategies for Clinical Trials to Support Approval of Human Drugs and Biological Products](#)
- [Mahajan & Gupta, 2010 - Adaptive design clinical trials: Methodology, challenges and prospect](#)
- [Chiaruttini et al., 2018 - Challenges in Paediatric Clinical Trials: How to Make It Feasible](#)
- [Baibaldi et al., 2011 - Innovative study design for paediatric clinical trials](#)

Reasons for limited application

- 1. Conservatism and Resistance to Change** --> Scientific and medical communities tend to be conservative and often prefer traditional and well-established methodologies. New approaches can appear risky or insufficiently validated compared to traditional methods.
- 2. Complexity** --> Innovative study designs can be complex to plan and manage. They require personnel with specific expertise in advanced methodologies and statistical analysis.
- 3. Regulatory Concerns** --> Even though regulatory authorities accept these approaches, there are often concerns regarding the interpretation of results, reproducibility, and generalizability of data collected using non-traditional methods.
- 4. Lack of Familiarity** --> Many researchers and clinicians may not be familiar with the new approaches and may lack the necessary training to implement them correctly.
- 5. Costs and Funding** --> Innovative studies can require higher funding, and funders may be reluctant to invest in unconventional methodologies without a solid track record.

Proposal of possible intervention

Knowledge Transfer of Innovative Study Designs



Promoting Engagement of Medical and Research Professionals

- Regular organization of **Workshops and Seminars** to share insights about innovative study design.
- Incorporation of them in modules in **Continuing Medical Education (CME)** programs.
- Distributing **Publications and Case Studies** to highlight their successful implementation and effects.



Fostering Engagement and Empowerment of Paediatric Patients and Parents

TEDDY for this purpose has already adopted a model based on a **progressive process of involvement, knowledge and experience reinforcement** of children in health and biomedical research.

According to their evolving capacities, children supported by parents should:

- (i) being informed with age-appropriate and easily understandable materials
- (ii) expressing a view
- (iii) influencing decisions
- (iv) engaging peer-to-peer with other stakeholders



***Young Person Advisory
Groups (YPAGs)
TEDDY Kids***