TEDDY collaborative group and plan of action

Paediatric clinical trial feasibility

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Paediatric Clinical Trials

The number of paediatric clinical trials in Europe has remarkably increased in the last years in response to the introduction of the first regulations on paediatric subjects.

Significant advances in child health have already resulted allover the world from the conduct of paediatric trials:

- ✓ Well-known trials of polio vaccines and the subsequent rapid translation into practice has lead to the almost complete eradication of polio
- ✓ Recent advances in multicentre cancer trials in children have increased childhood cancer 5 year survival from 28% in the late 1960s to 79% by 2005

Joseph PD, Craig JC, Caldwell PH. 2015;79(3):357-369

The number of clinical trials in the paediatric population still remains lower compared to the number of clinical trials conducted in the adult population

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Challenges in Paediatric clinical trial

- ✓ Lower commercial interest, increased cost and greater risk of liability for pharmaceutical industry
- ✓ Difficulties in the study design and conduct
- ✓ Ethical implications
- ✓ More restrictive regulatory oversight for paediatric trials

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Challenges in Paediatric clinical trial

Economic burdens

- Small patients' population
- Under-developed infrastructures
- Extra costs for paediatric formulation
- Higher safety risks (pecuniary and penal liability)

Drug Formulation

- Extra toxicity tests needed
- Not all excipients are acceptable
- Pharmaceutical form

Trial Design and Conduction

- Small patients' population
- Lack of validated quali-quantitative tools (endpoints, scales, AE-measuring tools)
- Limited volumes of biological specimens allowed

Regulatory process

- Time-consuming
- Inhomogeneity

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Challenges in Paediatric clinical trial

Ethical issues

- Parents' IC and Children's assent
- Data protection for minors
- Biological samples retention
- Higher discomfort and distress
- Insurance's long term liability needed
- Complicated to assess safety

Patients enrolment and retention

- Small patients' population
- Patients and families feeling distrustful towards the trials
- High rates of patients' withdrawal from the trials

Multi-centre international studies

- Multiplied operations, times and costs
- Geographical differences

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Assessment of clinical trial feasibility

Performing a careful **assessment of clinical trial feasibility is a crucial early step in initiating global clinical trials**, particularly in pediatric studies where country selection, site selection and patient recruitment each represents a significant challenge for the conduct of successful clinical trials

To avoid:

- Wastes valuable resources and time
- Potentially delays treatments becoming available to patients

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Clinical trial feasibility

Clinical trial feasibility is a process of evaluating the possibility of conducting a particular clinical program / trial in a particular geographical region with the overall objective of optimum project completion in terms of timelines, targets and costs.

A detailed feasibility assessment is an important step towards conducting a successful clinical study.

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Rajadhyaksha V. *Perspectives in Clinical Research*. 2010;1(3):106-109.

Why a TEDDY collaborative group?

- To compare different experiences within research groups
- To help in standardize the approaches
- To valorize the work done so far in inventoring clinical sites (TEDDY surveys on 2016-2017 also in the context of the PedCRIN project)
- To promote a ground of discussion within different actors, Sponsors, CROs, Regulators, Sites, Investigators, Networks

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How could this group work?

- Based on the work done in inventoring clinical sitse and clinical trials performed at sites, we should analize some trials for each site in order to understand which were strengths and which were weakness
- A similar activity has to be done with Sponsors/CROs in order analyze causes of failure of already planned or initiated paediatric clinical trials
- Networks have to be addressed In order to understand at which degree their involvement is beneficial for speed-up and positively conclude paediatric clinical trials, and at which degree administrative and organizational issues limit a good developmend of paediatric clinical trials
- The use of tools and training has to be promoted

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Which results could this group promote

- Creation of the Study Feasibility ID and a unique feasibility template for basic questions
- Creation of the General Master Confidentiality Agreement (CDA)
- Creation of publicly available network indication specific blinded data base / Registries
- Participate in the process of optimize the feasibility process considering an early feasibility assessment helps determine whether recruitment to a clinical study is likely to be completed successfully, taking into account the practical aspects of trial conduct and the resources required

Feasibility is an investment to ensure a good study

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