Paediatric Clinical Trial Preparedness
New Enpr-EMA Guideline
Webinar

Research Networks
point of view

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TEDDY in a nutshell

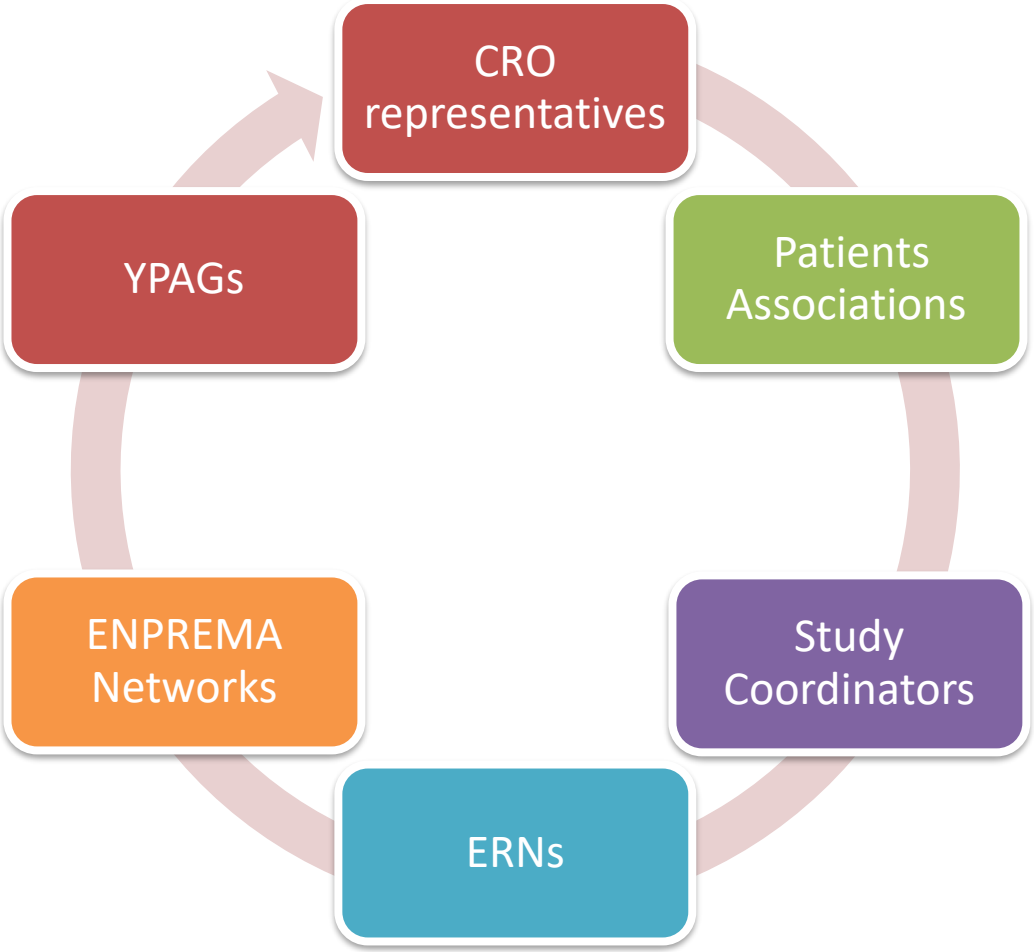
TEDDY was born in the context of the FP6
(start date: June **2005**, end date May 2010)

Since **2010** TEDDY has revised its organisation and gathered new research centres and groups willing to be engaged in developing paediatric clinical research

TEDDY today is a category 1 Network Member of Enpr-EMA
… is continuing at promoting children tailored medicine, guarantying children rights and well-being and providing methodological expertise.

It addresses methodological, ethical, legal and social issues of research and implements good practicesand tools to plan and perform paediatric clinical studies at international level.

TEDDY contribution to the activity (dissemination of the survey to)



TEDDY NETWORK
European Network of Excellence for Paediatric Research

Collected results

CRO Representatives
Belgian Association of CROs (BeCRO)
PHARMASICH
ConresoGmbH
Perfection-CRO Ltd
PPD
ZEINCRO HELLAS S.A
GynCentrum Praha

Patients Associations
Foundation of child neurology
ZIVOT - Life, association against child rare disease
EPIONI - Greek Carers Network
Fundaciòn ALPE acondroplasia
VSOP - Dutch Genetic Alliance
Arthritis and Rheumatism Association Malta
TIF - Thalassaemia International Federation

Study Coordinators
Consorzio per Valutazioni Biologiche e Farmacol.
Pharmaceutical Research Management Srl
Erlangen University

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Collected results

ERNs
European Reference Network on endocrine conditions (Endo-ERN)
European Reference Network on epilepsies (ERN EpiCARE)
European Reference Network on skin disorders (Endo-ERN)
European Reference Network on urogenital diseases and conditions (ERN eUROGEN)
European Reference Network on congenital malformations and rare intellectual disability (ERN ITHACA)
European Reference Network on paediatric cancer (haemato-oncology) (ERN PaedCan)

EnprEMA Networks
FINPEDMED- Finnish Investigators Network for Pediatric Medicines. Clinical Research Institute Helsinki University Central Hospital (CRI HUCH) Ltd., c/o University of Tampere,School of Medicine,Tampere Center for Child Health Research.
Pediatric Rheumatology International Trials Organisation (PRINTO)
TEDDY - European Network of Excellence for Paediatric Research
European Child & Adolescent Psychopharmacology Network - ECAPN
Medicines for Children Research Network (MCRN)-Hungary
SwissPedNet Swiss Research Network of Clinical Pediatric Hubs
TREAT-NMD

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ERNs point of view

PLANNING PHASE

What should be taken into consideration when planning to develop a new treatment?

- What is the best period of life that can better benefit from the new treatment
- Current status and knowledge. ATMP for children
- Rationale for treatment and how good the existing evidence base is, e.g. from animal and other studies

Which are the factors influencing the timelines of the planning?

- administrative procedures, complexity of the protocols
- rarety of condition, financial interests of pharma
- ethics, contracting

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ERNs point of view

PLANNING PHASE

What could be the key lessons learned that can help optimizing a clinical trial in pediatrics at the planning phase?

- simplified administrative procedures, shorter delay between the information delivered to families and the recruitment; database of well characterized population; implementation of the protocol in expert centres with a specific dedicated structure but also an expert multidisciplinary team (medical and no medical) for pediatric population and the specific disease
- There is a real need for common harmonised agreements and infrastructure.
- A trial office
- Patient and public involvement from Day 1.
- Ensure acceptability of trial protocol to patients
- Having access to good data which can be shared in this phase will be paramount

Which key elements of protocol design should be universally covered with early preparation activities regardless of the drug/therapeutic area?

- RCT, otherwise evidence will be lacking
- age of patients and number expected to participate and tools specific designed for the children to comply with the requested information

Enpr-EMA Networks point of view

PLANNING PHASE

What should be taken into consideration when planning to develop a new treatment?

- Targeting patients/diagnoses/age groups with medical unmet needs, potential impact of the treatments, choose meaningful treatment outcomes, feasibility of trials, representativeness of subjects and clinical situations included, appropriate design (i.e. lack of relapse prevention studies, implement methods to minimise placebo effect...)
- Maturational/developmental changes, especially in neonates and infants
- Appropriate formulation for all pediatric age groups
- Assessment of the current situation and therapeutic need, customized for each particular context. Evaluation of risk/benefit status
- Maturational/developmental changes, especially in neonates and infants
- Different age groups -> developmental stage / organ maturation - also over the 18-yrs

Which are the factors influencing the timelines of the planning?

- Time to agree to a right design
- Available data in adults on tolerability, safety, PK and PD. In-depth knowledge on the mechanism of action

Enpr-EMA Networks point of view

PLANNING PHASE

What could be the key lessons learned that can help optimizing a clinical trial in pediatrics at the planning phase?

- Taking into consideration particularities of pediatric populations, i.e. in psychiatry high placebo response (more in particular conditions such as depression), site selection (number of sites, sites able to collect quality data, with experienced qualified raters, able to maintain participants in the trial as long as needed)
- Understanding epidemiology of diseases (especially that of rare diseases) in pediatrics to avoid unrealistic sample sizes of clinical studies in children
- Early involvement of Principal Investigators and experts in the field
- Understanding epidemiology of diseases (especially that of rare diseases) in pediatrics to avoid unrealistic sample sizes of clinical studies in children
- Very early stage consultation (expert advice)

Which key elements of protocol design should be universally covered with early preparation activities regardless of the drug/therapeutic area?

- Appropriate formulation, dosing and sampling design; feasibility of conducting trial in age group of interest
- Study objective and rationale
- Appropriate formulation, dosing and sampling design; feasibility of conducting trial in age group of interest
- Real clinical needs

YPAGs point of view

Not all questions were answered by YPAGs. Some were judged out of frame or difficult to answer.
3 YPAGs (Kids Bari, Kids Albania and Kids France) participated corresponding to young people aged from 12 to 18 years of age

Method: Questionnaire review during YPAG meetings.
Facilitator asking questions to young people. Facilitator did not give his/her point of view but only tried to facilitate the understanding of the questions. Some of the steps were explained to young people as needed.

PLANNING PHASE

8 – Patient engagement and public awareness

Some feedbacks:

- Patients' or their parents' inputs could provide a fundamental support in the definition of the assent/consent process, eligibility criteria, recruitment phase and feasibility in terms of patients, study procedures., impact on quality of lives. They can bring an appropriate language. To the study documentation.
- An informed patient can contribute to achieve a best performance during the conduction of a CT, favoring patient's retention and fostering treatment compliance

YPAGs point of view

PLANNING PHASE

1 - Planning (e.g. Pediatric development plan/program)

Some feedbacks:

- While planning the development of a new treatment, the principal drivers should be the characteristics of the target disease, the unmet needs linked to that disease, an age-appropriate formulation, the characteristics of the patient as well as existing literature.
- Experts advice is very helpful in planning a clinical trial to allow facilitating the planning of the project. Advices from all experts is crucial. Patients and young people's voice is important too. Experience of the patient should be taken into account. Young people non patient can have a different point of view which can be interesting. Advices can be helpful in the design, materials, raising awareness on CT. experts could be reached through online tools, team leaders…
- There is a possible risk of raising patients expectations by involving them early in the process but groups think that informing them correctly can erase this risk.. However, it is still important to involve them at this stage to outline research. It is important to correctly inform patients of the research stage and that the possible treatment might not arrive at the end of the process. Need for training. Transparency in the process is needed