Enpr-EMA Work Group on clinical trial preparedness

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Aim

- The aim of this working group is to create a guidance document on trial preparedness.

Trial preparedness in this context means the set of contributing factors which could increase the ability to complete high quality clinical trials in a timely manner.

Mandate

Facilitation of the conduct of paediatric clinical trials related to drug development by focussing on identification and resolution of feasibility barriers at the planning stage

- Promote **dialogue among different parties to consolidate proposals** for the conduct of paediatric clinical trials.

- Agree on **factors and practicalities** recognised by all parties to have critical impact on successful completion of high-quality paediatric clinical trials.
- Gather relevant **examples of good as well as suboptimal practice** for the development and conduct of paediatric clinical trials in order to feed in to the preparedness-orientated strategic guidance.

- Develop **preparedness-orientated strategic guidance** to facilitate development, implementation and successful completion of paediatric clinical trials.

Working Group Members

Co-Chairs	Angeliki Siapkara (PDCO), Ruth Ladenstein (Enpr-EMA)
EMA	Irmgard Eichler, Roberto De Lisa, Gunter Egger, Ingrid Vilimelis
PDCO	Dimitrios Athanasiou, Siri Wang, Marek Migdal, Sabine Scherer
EnprEMA	Donato Bonifazi, Segolene Galliard, Jackie O'Leary (Geraldine Boylan) Carmelo Rizzari, Samantha Scarlett, Christina Seren Trasorras, Mark Turner, Ivan Foeldvari, Nicola Ruperto, Joana Claverol .
Pharma	James Barnes, Niyati Prasad (EUCOPE – Vertex), Claudio Fracasso (EuropaBIO – Pfizer), Solange Rohou, Ensio Norjavaara (SEBE - AstraZeneca), Tillmann Taube (EFPIA – Boehringer-Ingelheim), Loïc Notelet (Vaccines Europe – Sanofi).

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Action points

1. Review the current regulatory guidance and academic research proposals for the conduct of trials in the paediatric population to identify discrepancies on requirements, impacting on recruitment.
2. Summarise previous outputs from stakeholder meetings on this topic (e.g. DIA/EFGCP, ACCELERATE, ERN and EPAC community) to identify existing valuable guidance in overcoming challenges.
3. Development of survey for all stakeholders (industry, CROs, patients/parents groups, HCPs, regulators) on good practice and lessons learned (questionnaire) to build on experience already made in order to identify the main barriers in paediatric clinical trial characteristics leading to delays, or impairment of study feasibility.
4. Utilise deliverables from other Enpr-EMA WGs which have an impact on paediatric clinical trial conduct.
5. Development of preparedness-orientated guidance document including:
   - Q&A
   - Decision tree for solutions
   - Risk management strategy

Action point 1

Angeliki Siapkara (lead), Cristina Seren, Tillmann Taube, Donato Bonifazi, Mark Turner

- Published guidance and literature was searched for documents discussing trial preparedness, i.e. the preparation and conduct of clinical trials in the paediatric population.
- A total of 59 documents were identified and considered relevant.
- A spreadsheet was prepared capturing all trial preparedness factors and proposed solutions from the reviewed documents.
- Based on the trial preparedness factors that were identified six superior subject categories were defined; each identified preparedness factor was matched to one of the six categories (Engagement activities, Ethics, Rarity of disease/population, Regulatory, Study design, Training and infrastructure)
- Possible solutions discussed in the identified documents are displayed together with the factor identified

Action point 2

Ruth Ladenstein (lead), Dimitrios Athanasiou, Solange Rohou, Donato Bonifazi, Ensio Norjavaara

- Deliverable: List of initiatives on trial conduct

Excel list created and circulated for populating by all WG members

Cover introduction to be drafted

To be completed by mid June

Action point 3

Mark Turner (lead), Pirkko Lepola, Gunter Egger

- Deliverable: Summarise output from previous Enpr-EMA working groups

- Feedback received :
  - The result of PP survey is already available.
  - The actual capabilities of the networks needs to be further investigated – therefore next step survey (PP WG) to networks according to the planned 4-stage development
  - The WG ethics results (the general IC/Assent template) will also enhance the situation, when it will be publicly available on Enpr-EMA website for all stakeholders.
  - The WG Research Nurse training target is to provide existing training programs or even create a new curriculum to be spread across the clinics / research nurses.

Action point 4

Claudio Fracasso (lead), Siri Wang, Niyati Prasad, Segolene Galliard, Donato Bonifazi, Carmelo Rizzari, Solange Rohou, Angeliki Siapkara, Cristina Seren Trasorras, Loic Notelet, Niyati Prasad, Dimitrios Athanasiou

- **55 SURVEYS**: 10 from Patient Associations; 9 Site Study Coordinator Representatives; 7 EnprEMA Networks; 6 ERNs; 7 Pharma Industries; 4 CROs; 3 General Pediatricians; 3 Regulatory Authority Representatives; 2 CMWP-EFGCP; 1 UPPMD; 1 EEC; 1 CTN Representative, 1HTA.

- **13 INTERVIEWS**: 5 General Pediatricians; 2 CTN Representatives; 2 Regulatory Authorities Representatives; 1 CMWP-EFGCP; 1 ERN; 1 Patient Association; 1 Study Coordinator.

  feedbacks from eYPAGNet will arrive late in September and will be used as "addendum" at the time of the public review of the guidelines.

- Data analysed currently and to be presented at this week's f-2-f meeting

Action point 5

Mark Turner (lead), Loic Notelet, Jackie O'Leary, Niyati Prasad, Carmelo Rizzari, Sabine Scherer, Margaret Patton

- First draft of preparedness guidance document has been circulated and received comments
- Awaiting input from other AP activities
- Further deliverables with involvement of all working group members, and timelines: Draft ready for public consultation by June 2018, finalisation by December 2018