Enpr-EMA working group on International Collaborations

OCTOBER 10TH 2023

Jurisdictions

- Regulators
  - USA: Food and Drug Administration (FDA)
  - EU: European Medicines Agency (EMA)
  - Australia: Therapeutic Goods Administration (TGA)
  - Japan: Pharmaceuticals and Medical Devices Agency (PMDA)
  - Canada: Health Canada
  - UK: Medicines and Healthcare Products Regulatory Agency (MHRA)
- Networks
  - USA: Institute for Advanced Clinical Trials for Children (i-ACT) and Pediatric Trials Network (PTN)
  - EU: European Network of Pediatric Research at the European Medicines Agency (Enpr-EMA)
  - Australia: Australian Pediatric Trials Network (PTN)
  - Japan: Pharmaceuticals and Medical Devices Agency (PMDA)
  - Canada: Maternal Infant Child & Youth Research Network (MICYRN)
  - UK: National Institute for Health Research Clinical Research Network-Children (NIHR CRN-Children), and the National Health Services Scottish Children's Research Network (NHS Sco CRN)

Survey of pediatric clinical trial site requirements: Sponsors and CRO perspectives

- Demographics
- Principal Investigator Experience
- Site Study Personnel Requirements
- Study Site Selection – Site Requirements
- Preparedness for Remote Visit Practices
- Legal, Ethics, Budget

Survey of pediatric clinical trial site requirements: Sponsors and CRO perspectives

- Results from industry/contract research organization (CRO) survey (Enpr-EMA). This survey was conducted between April 2022 and August 2022
- The requirements were divided into four sections: investigator and supporting staff qualifications, site infrastructure requirements, administrative cycle times, and decentralised processes.
- The survey was followed up with optional interviews. The results were summarized using descriptive statistics, obtaining overall 33 responses from 21 countries as well as 7 virtual interviews.
- The results showed that sponsors had the same expectations in terms of required experience for the principal investigator as well as for sub-investigators, namely that they required a site investigator to be a medical doctor having at least1 to 2 years of experience and having performed at least 3 to 5 paediatric clinical trials.

Survey of pediatric clinical trial site requirements: Sponsors and CRO perspectives

- For the coordinating staff less years of experience and no previous involvement in paediatric clinical trials were required.
- The documentation required to prove experience included a curriculum vitae (CV), a certificate of attendance of Good Clinical Practice (GCP) training, license, list of clinical trials performed, certificate of biospecimen training and a financial disclosure form.
- Regarding infrastructure requirements, laboratory and pharmacy services as well as reliable IT infrastructure were considered essential, while imaging services, a dedicated paediatric unit and paediatric appropriate hospital level clinical environment were only considered optional.
- In terms of timelines, on average 90 days was considered an acceptable timeframe for the contract revision with a budget finalisation within 60 days, and an average timeline of 60 days was found appropriate for the institutional review board (IRB)/research ethics board (REB) revision.

Survey of pediatric clinical trial site requirements: Sponsors and CRO perspectives

- In addition, some remote capabilities were considered required for sites to conduct decentralised clinical trials. In general, the requirements for infrastructure, staffing and decentralised processes were considered trial and population dependent.
- During follow-up interviews the main challenges for conducting paediatric clinical trials at a site were elucidated, such as recruitment issues, lack of resources, time pressure and lack of knowledge of clinical trials and regulations.
- The interviews also brought to light the that paediatric research networks could have a role in increasing site attractiveness for global study participation e.g. via standardisation of site capabilities including training, providing recruitment tools and templates, building on existing efforts to reduce study start up timelines.

Next steps: dissemination

- Publication of the survey/interview results (in conjunction with data from c4c?)
- Compare the pediatric regulatory and ethics clinical trial requirements, submission and review processes in Australia, Canada, EU, Japan, UK and the US
  - Two manuscripts: Regulatory Requirements for Drug Trials Across Six Jurisdictions and Special Considerations for Trials with Children:
    - 1. Clinical Trial Application Review and Approval *(ready for submission)*
    - 2. Research Ethics Review and Approval *(pending data from one jurisdiction)*