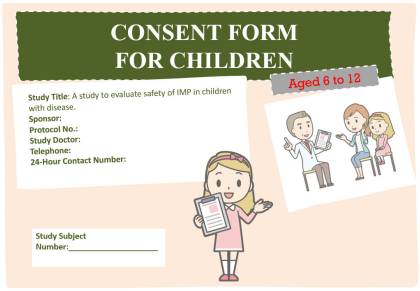
Pediatric clinical trials
Overview and Case Study

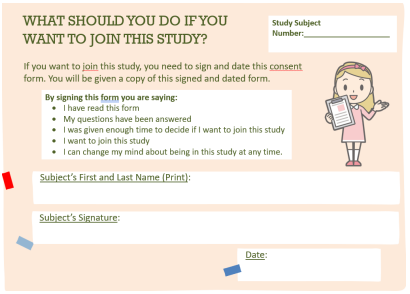
Overview

Pediatric clinical trials have unique ethical and regulatory requirements that must be carefully considered. Children are a vulnerable group, and ensuring their safety and well-being is of paramount importance.

Informed Consent

- Obtaining informed consent from both parents and children is the most important ethical factor in pediatric clinical trials. Aside from the subject, the parents must be fully informed of the risks and benefits of the trial.
- To ensure that the subject understands what they are agreeing to, they must be given age-appropriate information. Special care must be taken to ensure that the information provided is clear and understandable, and that the decision-making process is free of coercion or undue influence.
- Depending on the intended study population, several versions of informed consent need to be prepared for different age groups. The requirement for a minor to sign an informed consent form varies depending on the country's legislation. Similarly, the obligation to acquire signature from either one or both parents varies by country.





Regulatory and Safety

- There are specific regulatory requirements for pediatric clinical trials. The US FDA and European Medicines Agency (EMA) have specific guidelines for conducting pediatric clinical trials, including requirements for age-appropriate formulations in patient documents, safety monitoring, and ethical considerations.
- Pediatric trials may require different endpoints, assessment tools, and study designs than clinical trials for adults.

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- Children may be more vulnerable to the side effects of drugs than adults, thus safety monitoring must be a top priority throughout the trial. Any adverse events must be promptly identified and reported, and the trial must be modified or even stopped if there are any safety concerns for the participants.

Pharmnet's approach and experience

We have rich experience with the unique requirements of pediatric trials, and we work closely with our clients to design and execute safe, ethical, and regulatory-compliant trials. Pharmnet has completed around 30 pediatric trials across various phases and indications, accounting for approximately 15% of all clinical trials undertaken at Pharmnet.

Case study

Pharmnet participated in a large international pediatric phase III study for a major pharmaceutical company comparing IMP to standard of care for the treatment of venous thromboembolism. Pharmnet was responsible for the regulatory and monitoring activities in the Czech Republic. The study was challenging since the IMP was administered in three pharmacological formulations to three age cohorts ranging from birth to 18 years.

Regulatory

During the submission process of the study for approval by the Czech regulatory authority SUKL, there were multiple challenges that Pharmnet and Sponsor had to address. SUKL vigorously reviewed the IMPD, IB and Protocol, issuing numerous requests for clarifications and submission of additional data, resulting in several mandatory notices. SUKL initially approved recruitment only in the oldest age cohort (12-18 years old) and did not allow recruitment to the next age cohorts until the sponsor submitted for approval safety data for children aged 12-18 years and 2-12 years consecutively. Pharmnet provided expert advice to the sponsor on SUKL's specific regulatory requirements throughout the approval process and used its expertise and strong relationship with SUKL in order to find satisfactory solutions to the RA's requests.

Recruitment

The study recruitment was initially slow but due to Pharmnet's understanding of the local milieu, we were able to identify additional sites to boost recruitment. In addition, we proposed collaborating on the study with several hematologists from across the country who had referred suitable patients. CRAs proactively listened to investigators' concerns about recruiting and relayed them to the sponsor, who reflected them in the protocol design. Despite having a smaller population, we were among the top recruiters, accounting for around 17% of the global total. As a result of the Czech Republic's strong contribution, worldwide recruiting recovered and the client's needs were met successfully.