eYPAGnet
European Young Persons' Advisory Group Network

SJD Sant Joan de Déu Fundació de Recerca

Patient Engagement in Research Area

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Paediatric Patient Involvement in Clinical Trials In Europe (Regulatory and operational challenge)

- **The challenge:**
  1. Meaningful patient involvement is not integrated as part of the process to design and execute clinical trials.
  2. Specific challenges of designing pediatric clinical trials can be prevented by involving patients at the right moment in the drug development process (e.g., formulation, study design, PREMs, etc.).
  3. Children and young people should be involved when feasible, respecting their rights and based on proper methodologies.
  4. Feedback to the participants in patient involvement activities should be part of the process for meaningful involvement.
  5. Regulation or recommendation guidances should encourage the involvement of peadiatric patients as part of the drug development process.

Paediatric Patient Involvement in Clinical Trials In Europe (Regulatory and operational challenge)

- **Paediatric patient involvement in the conception and design of clinical trials.**
  Move from a recommendation to a requirement.
  - 1) Involve the patients/parents before finalize the clinical trial protocol (PIP stage).
  - 2) Consider patients/parents as a key partner along the drug development process.
  - 3) Meaningful involvement of children and young people with experts.
    - E.g. eYPAGnet
  - 4) Ensure that key stakeholders have knowledge and skills to involve children in the drug development process.
  - 5) Lessons learned and good practices of patient involvement activities in paediatric and adult clinical studies should be published and disseminated.

Paediatric Patient Involvement in Clinical Trials In Europe (Regulatory and operational challenge)

- **FINAL GOAL:**

Promote the design of bespoken patients involvement plan in paediatric clinical trials targed to each clinical study. Regulators need to request the evidence of it in the clinical trial application.

Cross-Border Paediatric Clinical Trials with no language discrimination of the patients (Regulatory and operational challenge)

- **The challenge:**
  - **Mother tongue** and **country of residence** are used as eligibility criteria in clinical trials. This practice is unethical when there is a potential benefit of participating in a clinical study.
  - There is no regulation in Europe about cross-border clinical trials.

Cross-Border Paediatric Clinical Trials with no language discrimination of the patients (Regulatory and operational challenge)

Legal framework:

- **Convention on the Rights of the Child**. Art. 24: "*Children have the right to the best possible healthcare*".
- **Belmont Report** recognised that it is unjust "*to offer potentially beneficial research only to some patients who are in favour of specific subgroups*".
- **Helsinki Declaration**. Ethical principles for medical research involving humans should be applied to facilitate access to clinical trials.
- **Non-discrimination by language** according to the Charter on Fundamental Rights of the European Union (2012).
- **Clinical trials** are often the **only potential therapeutic option** for most paediatric patients affected by a rare disease.

Cross-Border Paediatric Clinical Trials with no language discrimination of the patients (Regulatory and operational challenge)

- **FINAL GOALS:**

Integrate the outputs of the research that is being done by a Working Group at EnprEMA.

Provide guidance at European level to facilitate the inclusion of paediatric patients in cross-border clinical trials to ensure that no language discrimination is done in terms of eligibility of the patients.

Three things we have left of paradise: the stars, the flowers and children.

Dante Alighieri

Thank you so much!

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