



ACT EU Multi-Stakeholder Workshop on Methodology Guidance: A patient- centred approach to methodologies

23 November 2023

Virtual meeting / EMA, Amsterdam, Meeting Room 1C and Webex

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Challenges to be addressed in Paediatric Clinical Trials In Europe

- **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) Promote the design of bespoke patients involvement plan targeted to each clinical study. Regulators need to request the evidence of it in the clinical trial application.
 - 4) Meaningful involvement of children and young people with experts.
 - eYPAGnet
 - 5) Training of stakeholders.
- **Patient Experience Data, PROMs and QoL scales.** Consider their value as secondary endpoints. To be developed with patients when not existing.

Challenges to be addressed in Paediatric Clinical Trials In Europe

Cross-border clinical Trials – Regulation will ensure the patients rights and balance the same opportunities accross Europe.

Clinical trial design for PIPs - Consider them as the “first in pediatrics”, not replicating the adult design.

Decentralized Clinical Trials - One size doesn't fit all.

Multistakeholder meetings - Strengthen the dialogue between stakeholders: patients, regulatory bodies, commercial sponsors, academia, and ethics committees – Paediatric diseases mostly are rare diseases.

Connect4Children: foster close collaboration with sites and networks. Identify their needs and learn from the “real world”.



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

Dante Alighieri

Thank you so much!
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