PANEL: From Small Populations to Robust Evidence: EU Methodologies for Paediatric and Rare Disease Drug Development

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Challeges for small populations



- Lack of science
- Preclinical models not always available
- Few patients data
- Geographic dispersion of experts and sites
- Age-related differences in paediatrics
- Lots of diseases with genetic nature

Main challenges of paediatric clinical research

Regulatory requirements

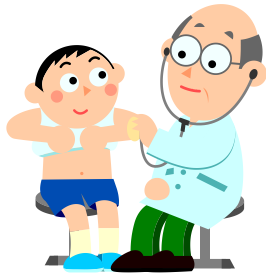
- ✓ GCP-ICH E11

Ethical concerns

- ✓ Children are vulnerable and unable to provide legal consent
- ✓ Concerns for enrolling children in trials
- ✓ Concerns for placebo
- ✓ Pain, discomfort, fear, distress to be minimized

Methodological

- ✓ Age-related differences in drug handling
- ✓ Few patients
- ✓ Sample as small as possible but sufficient statistical power necessary
- ✓ Need for age-appropriate formulations
- ✓ Alternative approaches necessary
- ✓ Unability to communicate adverse events
- ✓ Need for specific biomarkers



Multi-national studies

Jeopardized legal and ethical frameworks

Families

- ✓ Unfamiliar with trials
- ✓ Worries about risks of new treatments

To gain evidence (efficacy, safety) with reduced timing and efforts
⇨ *speeding up licensing of medicinal products*



- to complement
- to replace

traditional health research



Small number of patients



best evidence from results

regulatory compliance

sample as small as possible

identify responders and increase statistical power



need for protecting participants

…don't forget consent and assent!





need for protecting participants

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PANEL purposes

Examine how innovative methodologies can move from methodological development to scientifically *robust*, regulatorily *credible* and operationally *feasible* use in small populations like paediatrics



Where conventional trials are impractical

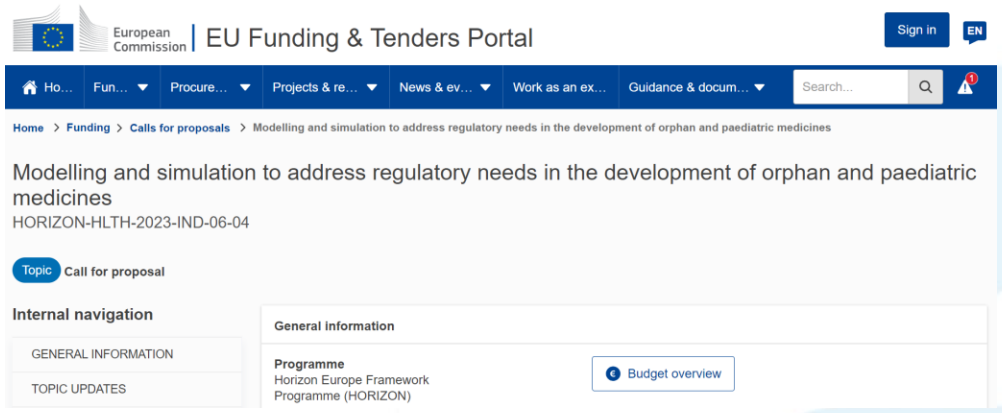
Designs, models and simulations

Acceptability, qualification, early dialogue

Networks, sites, data

Concrete cases and collaboration

EU-funded research projects on small populations with regulatory impact







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PANEL:
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