

Decentralized trials: Pediatric Considerations



General Considerations I

- ▶ Increasing diversity and equity through decentralization is of foremost importance for children and their families but there are caveats that need to be addressed (language, access to internet, privacy,..).
- ▶ New methods and technologies (telehealth systems, wearable device, remote monitoring, digital) adapted to the pediatric population could increase enrollment, retention and satisfaction.
- ▶ Decentralization means different things : Academic hospitals - Community/regional hospitals - Home
- ▶ Involving patient partners in the design, execution and conduct of a trial is critical to ensure elements of decentralization are relevant and feasible

General Considerations II

- ▶ “A sick child is a sick family”: decentralization has huge potential for minimizing logistical hurdles affecting families of children enrolled in CT.
- ▶ Indirect cost is significantly higher for families with children enrolled in a trial compared to adults (transportation, arranging childcare, school interruption, taking time off from work, etc..). This is amplified in the Rare Diseases space.
- ▶ Responsiveness to DCT from families is likely dependent on multiple factors:
 - ▶ nature of the disease and its severity
 - ▶ family functioning
 - ▶ relationship with and trust in the health care provider team (having an appointment with a doctor in the hospital setting is a standard that some families will be reluctant to renounce to). Families preferring a centralized approach should be financially supported.

Informed consent/assent process

- ▶ IC Interview - as well as opportunity to ask questions - must be provided to both child participant and the legal representative - takes more time - child must have right to express own views (disagreement / dissent option)
- ▶ Information must be provided per age group according to the maturity - may need several e-consent / e-assent form versions
- ▶ Explaining trial procedures and flowchart can benefit by using digital formats (video, animations, multimedia etc.)
- ▶ Children may need adult support throughout the IC process (requires parallel/simultaneous entry to database e-consent platforms)
- ▶ e-Informed consent / e-assent signature may need double-step identification (high level personal data security) such as bank credentials
 - ▶ minors under certain age limit per jurisdiction may not have access to such credentials
 - ▶ may need specific legally accepted procedure e.g. “acting on behalf” requirements
 - ▶ may need 2-3 signatures; child + 1-2 legal representatives according national requirements

Trial Related Procedures at Home

- ▶ Trial related procedures at home
 - ▶ RD with pediatric onset (75%) have distinct and sometime unique characteristics that create additional decentralization challenges.
 - ▶ Clinical Outcome Assessment (COA) and measurement of endpoints: many scales and instruments not validated for virtual administration. Administration to young infants at home may require special training of personal.
 - ▶ Development of minimal Invasive blood sampling methods will increase recruitment and retention.
- ▶ Roles and responsibilities - use of digital tools (diary, ePRO, wearables etc.)
 - ▶ Requires adult oversight, control and support (= extended training)
- ▶ IMP storage at home may need extra security setting
 - ▶ To guarantee safety and preventing children's access to IMP