Sneak Peek

Paediatric Drug Development & Clinical Trials

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ONLINE
TRAINING

Establish Efficacy in Children

01

- need to develop, validate, and use **different endpoints** for specific age subgroups
- **responses may vary** among patients due to different duration of the disease in different developmental stage of the patient
- many diseases in the preterm and term newborn infant are **unique or have unique manifestations**
- such aspects **limit the extent of extrapolation** of efficacy from older paediatric patients or adults
- need for **novel methods** of outcome assessment

Establish Safety in Children

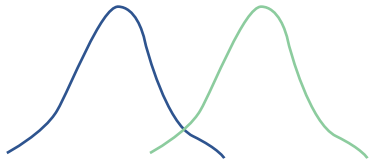
02

- the **adverse event profile may** differ in paediatric patient
- some adverse events and drug interactions that occur in paediatric patients **may not be identified in adult** studies
- **age-appropriate, laboratory values and clinical measurements** should be used in adverse event reporting.
- unintended exposures may provide the opportunity to obtain safety and pharmacokinetic information
- medicinal products may affect **physical and cognitive growth** and development, may not manifest acutely, but at a **later stage** of growth
- **long-term studies or surveillance data**, either while patients are on chronic therapy or during the post-therapy period, may be needed to determine possible effects on skeletal, behavioral, cognitive, sexual, and immune maturation and development.

Study Design

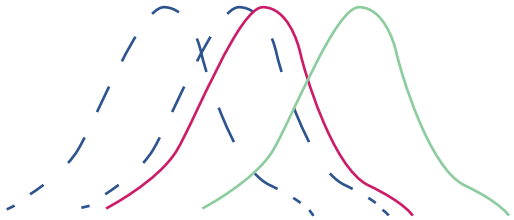
RND DB PLC -sample size1

- The sample size should be large enough to show a difference between treatment and control group



- This is easier between placebo and active,

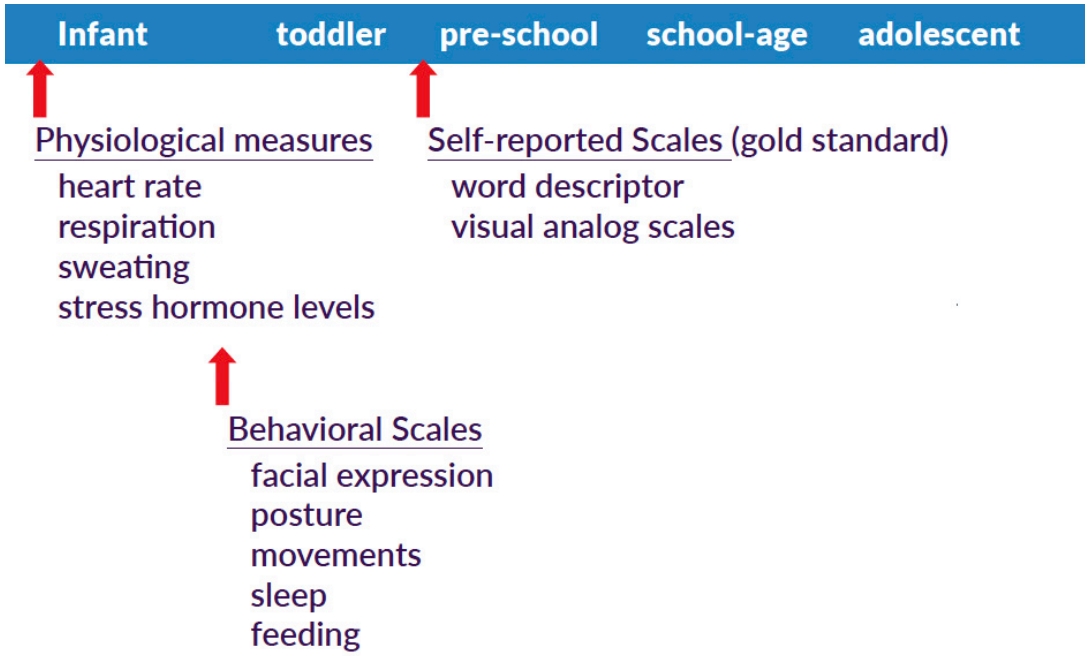
- as in an active comparator the difference is smaller
- However, with an active comparator you have no clear information on the effect size, i. e. the sensitivity of your trial



Study Design

RND DB PLC – endpoints

Specific problems in children: often you will need different endpoints in different age ranges, e.g. pain



Sensitivity and accuracy of pain scales will be different in different age ranges, would need to be analysed separately.

Other examples:

- 6 min Walk Test
- Perimetry
- Forced exspiratory volume (FEV1)

A clinicaly established outcome is not necessarily a good primary endpoint

Feasibility/interpretability of an endpoint is not a sufficient justification not to include this age range into clinical development

Registration

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