

OBSTACLES TO A TIMELY COMPLETION OF PAEDIATRIC DEVELOPMENT PLAN STUDIES

Methodological aspects:

- ✓ Non adapted protocols for children (small adults version).
- ✓ Non feasible inclusion and exclusion criteria.
- ✓ Non patient centered (quality of life of patient/family).

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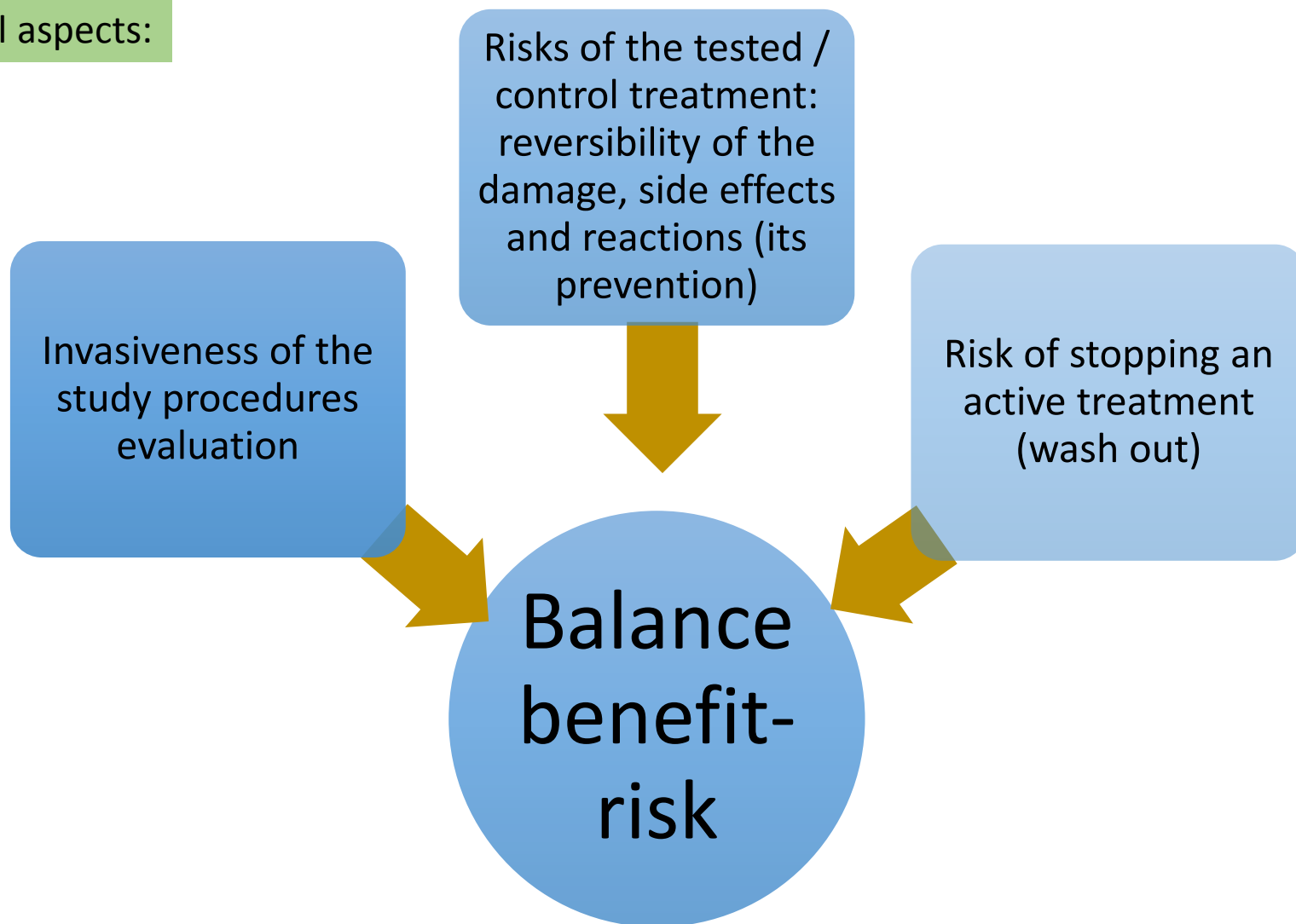
Use of
validated tools
and suitable
outcomes for
the age

Only use
placebo when
there is no
appropriate
active control

Use
appropriate
designs for
small samples-
recruitment
difficulty

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Ethical aspects:



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Regulatory aspects:

- **Harmonization** among the European countries about:
 - Local regulation
 - Processes for patient information and consent/assent
 - Ethics Committees' approvals
- Not consider that beyond of feasibility patient involvement can have benefits on the **ROI** and **ROE**. We need to be aware about the need to promote young patients participation from the role of every stakeholder involved in paediatric clinical trials.
 - Nowadays there isn't direct involvement of young patients in specific phases and activities, e.g. from regulatory bodies.

MEASURES TO ADDRESS THESE OBSTACLES ACCORDINGLY WITH THE AGREED PAEDIATRIC PLAN

- Adapt the **design of the paediatric clinical** trials to the real world.
- Include **patients participation in the early phase of clinical trials:**
 - From the definition of research priorities to protocol design
 - Requested as mandatory in the regulatory process at least in the most complex studies.
- Promote the **standardization of the young patient involvement** along the life cycle of medicines in the among the different clinical trials network.
- Increase the expertise and best practices sharing about young patient engagement among all the **stakeholders**. Main goal of eYPAGnet.
- **Education and training addressed to patients**, relatives and the other stakeholders about the meaning and the feasibility of patient involvement.