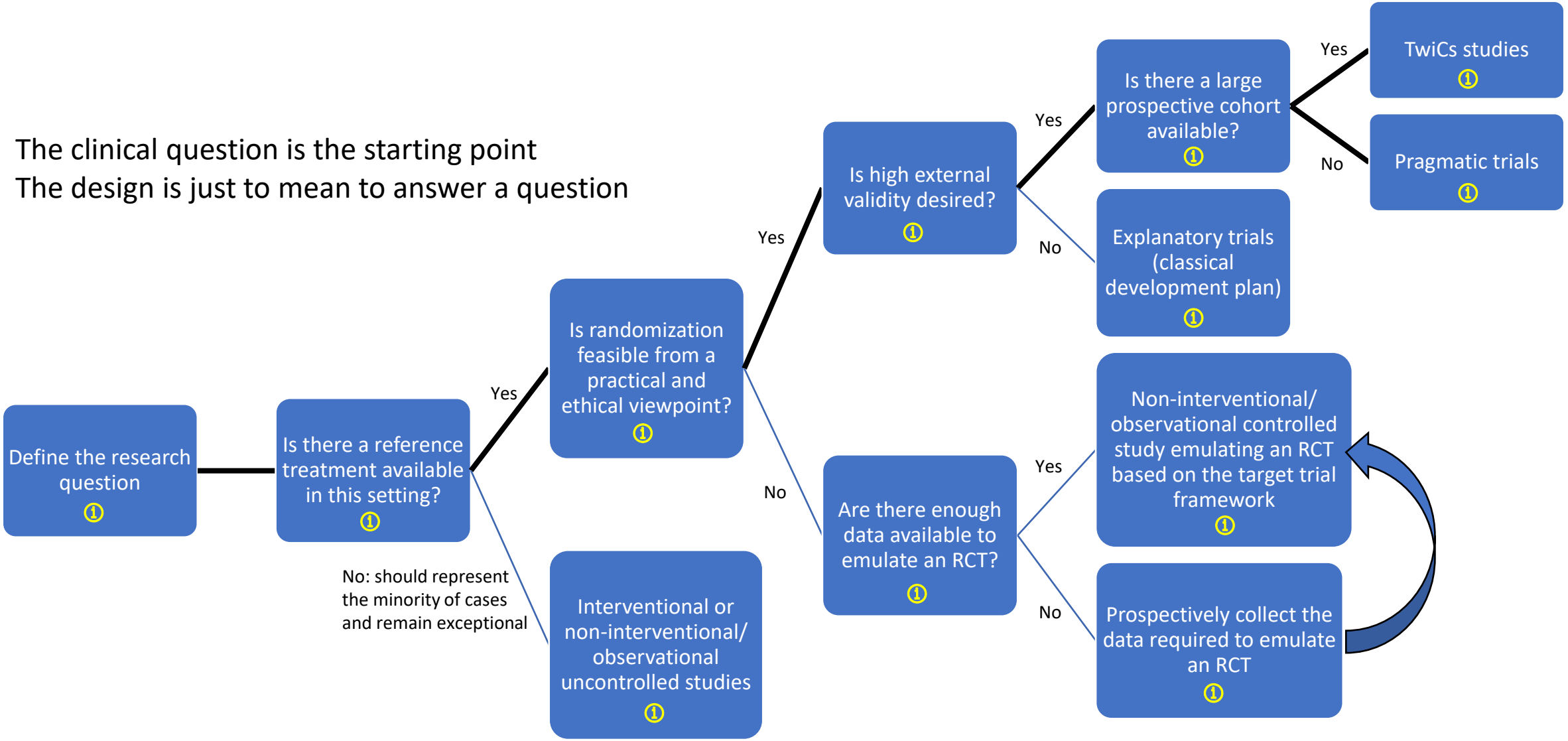
EORTC
European Organisation for Research and Treatment of Cancer

Synthetic and external controls for clinical trials

The clinical question is the starting point

The design is just to mean to answer a question



Thick lines represent the default route to deliver evidence for trial design selection

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Recommendations

- RCT remains the golden standard
- Each synthetic control project has its own challenges: no one solution fits all
- Consider pragmatic clinical trials for rare clinical situations
- Early perception of the lack of equipoise is a disservice to patients and society
- Non randomised approaches are not necessarily faster and cheaper but carry certainly more uncertainty
- Always attempt to produce the highest level of robustness

Definitions and Assumptions

- Any source of clinical data: CT, cohorts, routine, insurance claims, registries
  - Caution: above by order of validity
  - Recent vs older dataset
- Validity and reliability:
  - Dataset
  - Method to create a synthetic group
- Evolution of the stds of care, guidelines, disease stage migration etc…
- Clinical outcome of relevance: quality control in real life
- Bench marking ≠ comparison: uncertainty