Zorginstituut Nederland

Can we use every single-arm trial (SAT) in HTA's?

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| Van goede zorg verzekerd |

Increasing number of reimbursement dossiers are based on SAT’s

EUROPEAN MEDICINES AGENCY
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Management of the basic health care package

The four package criteria

1. Effectiveness (knock out criterion)

   Is here evidence that a given treatment works?

2. Necessity

   Is the disease serious enough? Is insurance the right instrument?

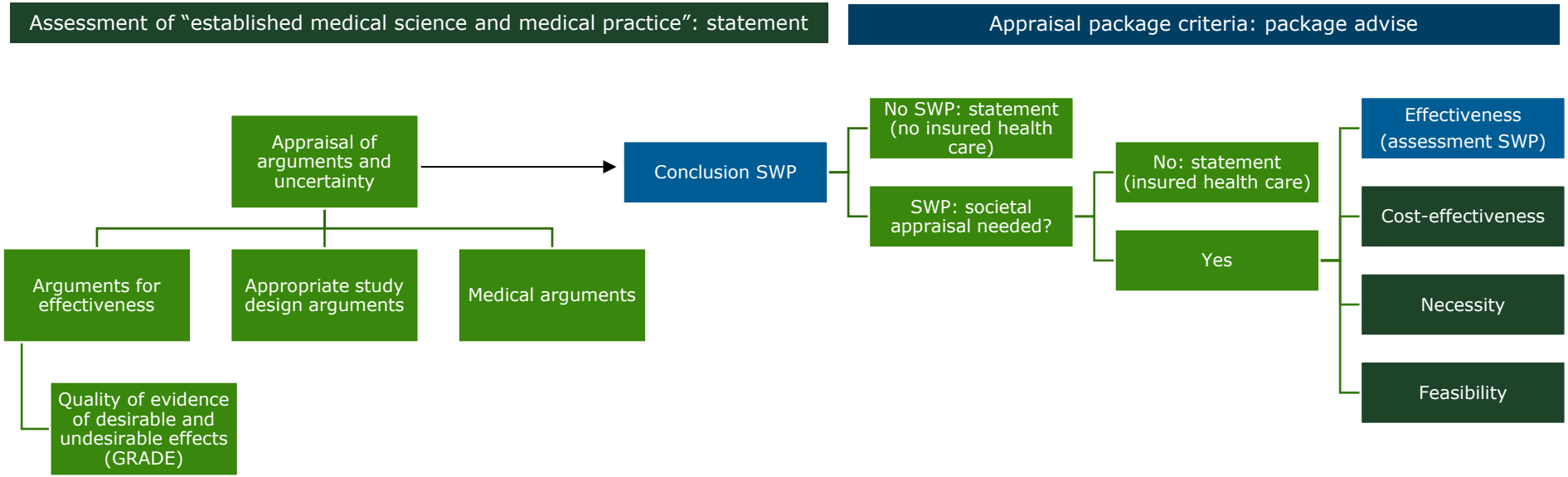
3. Cost-effectiveness

   Is the ratio between the costs of a treatment and its results acceptable?

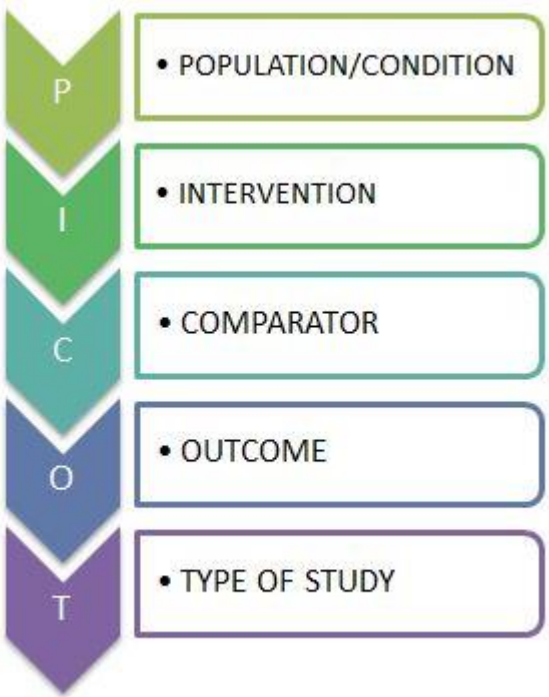
4. Feasibility

   Is inclusion of a given treatment in the package sustainable and feasible?

Dutch assessment framework



Establishing the scope of the assessment



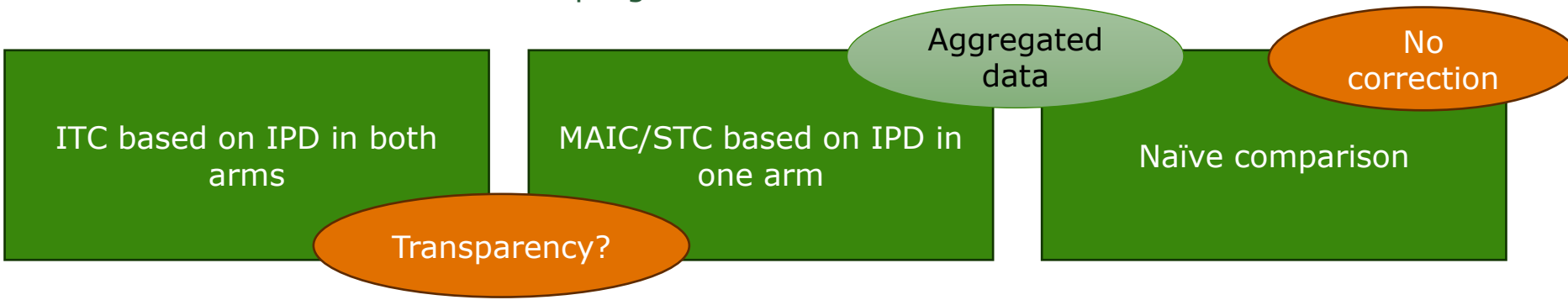
Assessing the quality of the evidence

Evidence based medicine → GRADE method

- RCT: highest quality of evidence
- SAT's: start at low quality of evidence
  - High risk of bias due to risk of confounding

Different types of indirect treatment comparisons

The quality of the comparison depends on several factors such as completeness of data and inclusion of relevant prognostic factors



Correction for differences in trials

Appropriate study design

Several factors at play:

- Willingness of patients to participate in a (blinded) RCT
- Disease characteristics
- Clinical equipoise

Example: Gene therapy for a rare disease
- Highly experimental and irreversible therapy
- Issues with long-term follow-up in control-arm
- Difficulties with blinding due to home assessment of certain outcomes

Is a SAT appropriate in this case?

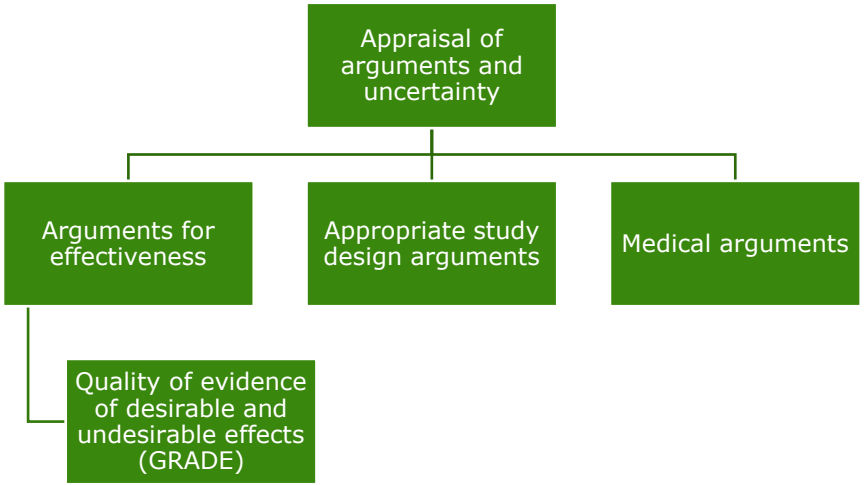
Example: Rare disease with no preferred treatment

- *Comparator consists of "physician's choice"*
- *Several interventional SAT's as well as observational studies have been carried out*

Would a RCT with an open control-arm have been feasible?

Evidence to conclusion

- Additional contextual argumentation is needed to conclude SWP with **increasing uncertainty**



But how to assess cost-effectiveness with all these uncertainties?

Important difference between SWP and cost-effectiveness:

- To assess SWP we answer a yes/no question

- Cost-effectiveness quantifies the amount of added health benefit

Thank you for your attention!

Questions?

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