



# Health Technology Assessment and Pragmatic Controlled Trials

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# Overview

- 1) Point of departure; HTA today
- 2) Pragmatic controlled trials
- 3) Benefits of including PCTs in HTAs
- 4) Conclusion



# 1. HTA today

What is HTA?

*“Health technology assessment, a multidisciplinary activity that systematically examines the **technical performance, safety, clinical efficacy and effectiveness, cost, cost-effectiveness, organizational implications, social consequences, and legal and ethical considerations** of the application of a health technology, has to take into consideration **all aspects that might be influenced by the technology and those influencing the technology.** “*

Source: Busse et al. Best practice in undertaking and reporting health technology Assessments. Int.Jour.Tech.Asses.Health Care, 2002, 18:2; p.364.

# 1. HTA today

What is HTA?

*“The main purpose of HTA is to **inform decision making** in health care, including decisions made at the individual or patient level, the level of the health care provider or institution, or the regional, national and international levels.....*

*HTA is **context specific**, with findings for one health care setting, patient population, or country not necessarily being valid in another. “*

Source: Resources for HTA - HTAi and INAHTA's White Paper to WHO



# 1. HTA today

What do we do?

- Systematic reviews
  - Meta-analysis (first choice)
- Primary research
- Evidence based medicine
  - hierarchy of evidence



# 1. HTA today

What do we do?

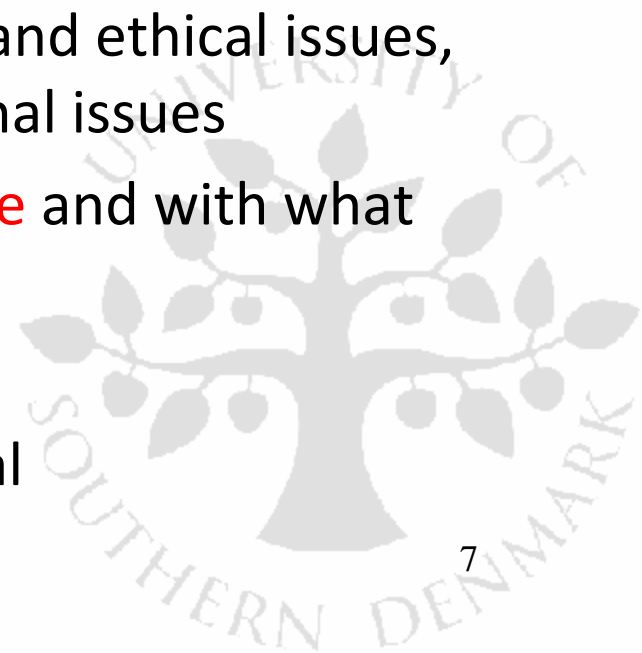
- Content of HTAs
  - In principles:
    - Safety, efficacy, effectiveness
    - Psychological, social, ethical issues
    - Organizational and professional issues
    - Costs and economic evaluations
  - In practice:
    - Much more narrowly defined
    - Primarily clinical issues
    - Secondary costs and economic evaluation



# 1. HTA today

What do we need?

- What are we in need for?
  - To answer the policy question?
- Perspectives of the decision-makers
  - High correspondence: efficacy/effectiveness, safety and disease severity
  - **Low correspondence**: psychological and ethical issues, equity, organizational and professional issues
  - How the technology **works in practice** and with what consequences?
  - How to **apply to own local context**?
  - **Context-dependent factors** in general

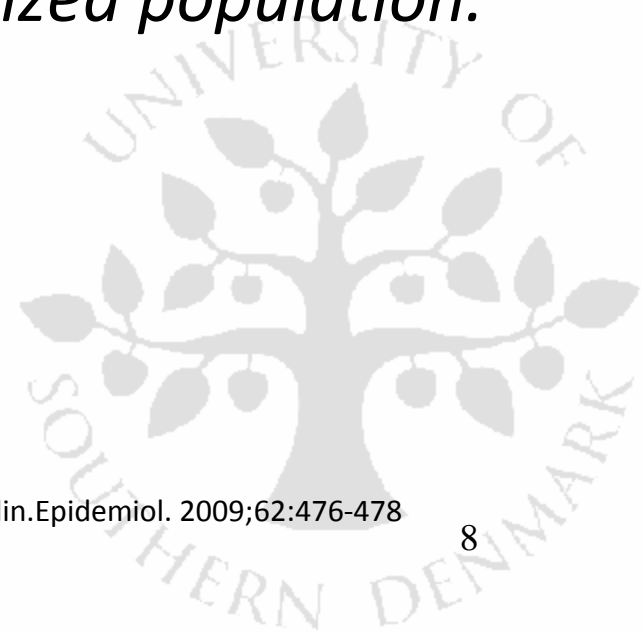


# 2. Pragmatic controlled trials

What is it?

*“A pragmatic trial is a real-world test in a real-world population whereas an explanatory trial is a specialized experiment in a specialized population.”*

Source: Maclure M. Explaining pragmatic trials to pragmatic policymakers. *Jour.Clin.Epidemiol.* 2009;62:476-478



# 2. Pragmatic controlled trials

What is it?

*Reflection of normal practice*

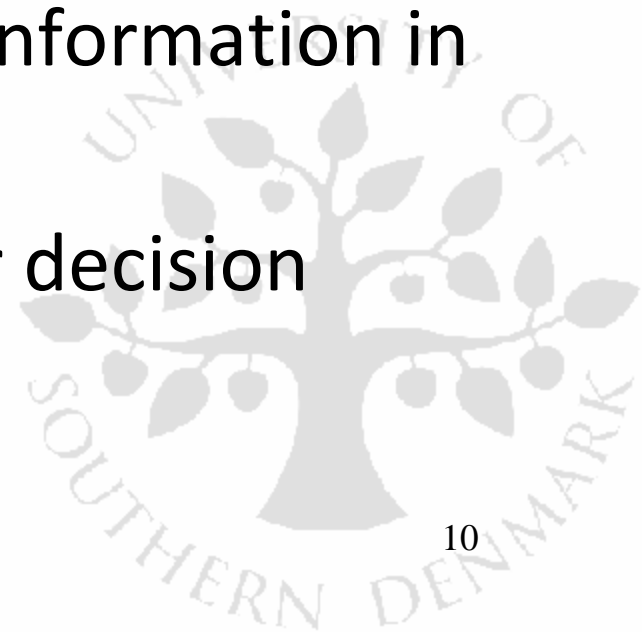
1. Aim of study
2. Study characteristics
  - Settings
  - Participants
3. Interventions
4. Analysis
5. Outcomes



# 3. Benefits of incorporating PCT in HTAs

Three main benefits:

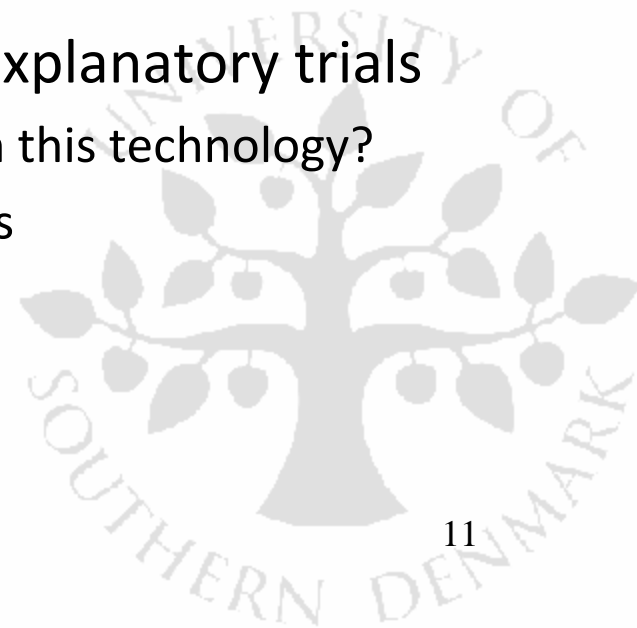
- 1) Expanding the contents of HTAs
- 2) Inclusion of context-relevant information in HTAs
- 3) Information more relevant for decision making



# 3. Benefits of incorporating PCT in HTAs

## Ad 1) Expanding the contents of HTAs:

- PCTs can entail a more varied spectra of assessed issues in HTAs
- Issues which are not assessable in explanatory trials
  - How does the average patient react on this technology?
  - Effects on the daily lives of the patients
  - Organizational issues
  - Etc.



# 3. Benefits of incorporating PCT in HTAs

## Ad 2) Inclusion of context-relevant information in HTAs

Questions like:

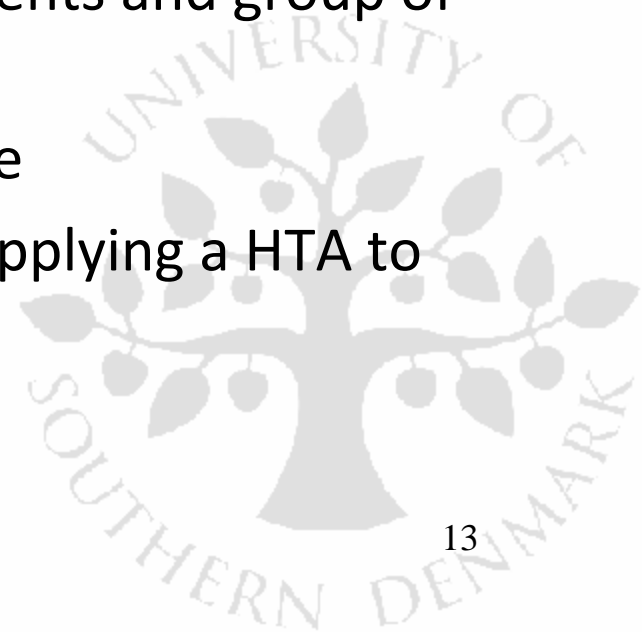
- How does the technology works in our area with our setting?
- What are the expected costs in our setting if we implement this technology?
- Which impact will the technology have on our personnel and way of organizing our setting?



# 3. Benefits of incorporating PCT in HTAs

## Ad 3) Information relevant for decision making

- Assessments in normal practice and daily use
- Including the variations in real-life patients and group of patients
- Including the effects of non-compliance
- Reduce the time and workload when applying a HTA to own local practice



## 4. Challenges to overcome

- Demands different methodological skills
  - experiences with quasi-experiments
- Demands extensive knowledge and skills in the field of validity
  - experiences with quasi-experiments
- Extends the time and increase the costs to produce HTAs



# 5. Conclusion

In general: PCT can diminish the discrepancies between what decision makers wants and what is actually presented in HTAs

PCT can:

- **Widen the contents** of HTAs
- Include **context-relevant** information
- Present information on how a technology works in **daily practice** and **normal settings**  
→ *wants of the decision makers*



## 5. Conclusion

*“It is time to shift our design choices so that they match our usual purpose in conducting a trial, most often to directly inform the decisions of real-world patients, clinicians, and third-party funders.”*

*- no replacement, but supplement*

Source: Zwarenstein, Treweek. Making Randomized Trials More Useful: The Pragmatic Attitude to RCT Design. Commentary. What kind of randomized trials do we need?. Jour.Clin.Epidemiol. 2009;62:461-463

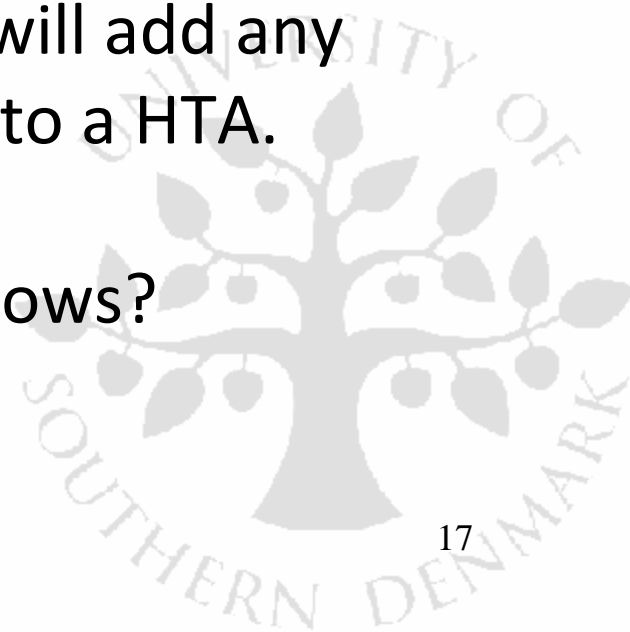


# 5. Conclusion

But.....

It might be the time to ask oneself the question whether another RCT will add any new and beneficial information to a HTA.

- or confirm what we already knows?



## 5. Conclusion

Once a technology's efficacy and safety has been established to a degree of certainty –  
**using explanatory trials....**

- its beneficial to establish the technology's overall effectiveness and other impacts –  
**using pragmatic controlled trials**



**Thank you for your attention!**

