

Opportunities and Challenges for Decentralized Clinical Trial Approaches: European Health Technology Assessment Perspective

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INTRODUCTION

Results from clinical trials can inform health technology assessment and subsequent reimbursement decisions. Digital health technologies and other innovative trial operations enable the conduct of trials closer to participants' proximities instead of traditional (hospital-based) investigative sites. Trials in which trial activities are conducted at participants' homes or direct surroundings are collectively known as decentralized clinical trial (DCT) approaches. DCT approaches could address well-known trial barriers such as limited recruitment and retention rates, high participation burden. However, it is not known how HTA assessors would evaluate these type of studies in their evaluation.

GOAL

To identify opportunities and challenges for DCT approaches to support HTA decision making from a European HTA perspective.

METHOD

Online, one-hour, semi-structured interviews with representatives from HTA bodies. Data were analysed following thematic analysis.

RESULTS

Two main themes were identified from the data. 1) DCT approaches in HTA relates to the perceived suitability of DCT approaches in terms of therapeutic areas, interventions and endpoints, and the relation of DCTs with real-world evidence. 2) Trial-level acceptance and relevance concerns factors that influence the acceptance of data generated in a DCT including data completeness, variability, validation, the impact of biases and the potential to increase generalizability of trial results.

Key opportunities

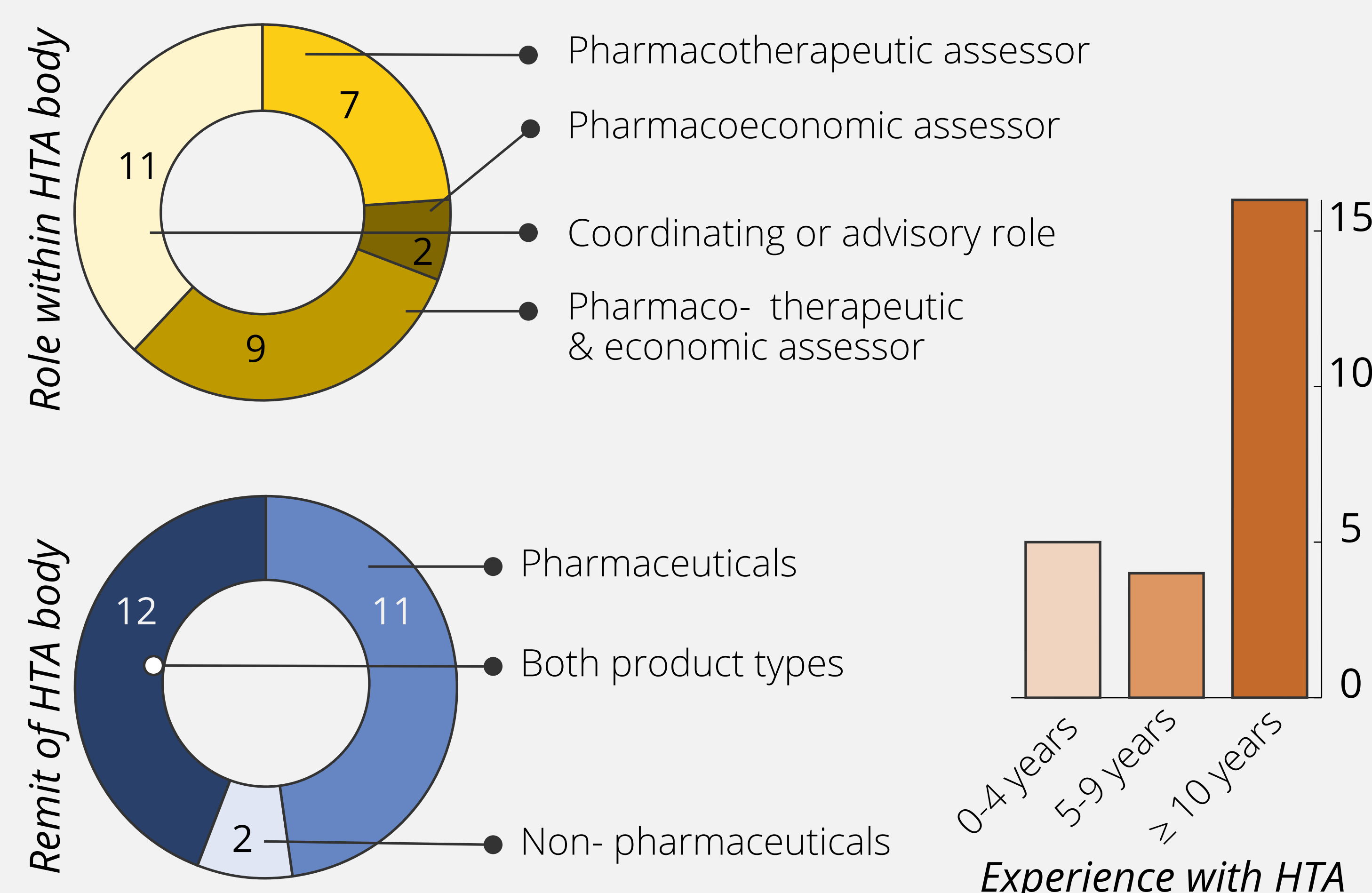
- 1 Data collection reflective of the real-world setting
- 2 Reduced recall bias and Hawthorne effect
- 3 Increased generalizability

"Quality of life data is something that could be collected in this way [in a DCT]. [...] I can imagine that this could lead to less missing data."

Key challenges

- 1 Impact of data collection in less controlled settings
- 2 Change of behavior when outcome data is observable
- 3 Limited knowledge of DCTs among HTA

RESPONDENT CHARACTERISTICS (n = 25)



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CONCLUSIONS

Trial conduct in real-world settings, involving a trained, representative participant group is appreciated by HTA representatives. HTA assessors should be made aware of DCT approaches, while acknowledging the diversity in DCT approaches. When assessing DCTs, attention should be given to data completeness, appropriateness of the (digital) endpoints and trial population characteristics