Application and importance of HRQoL/PRO assessment from the HTA perspective

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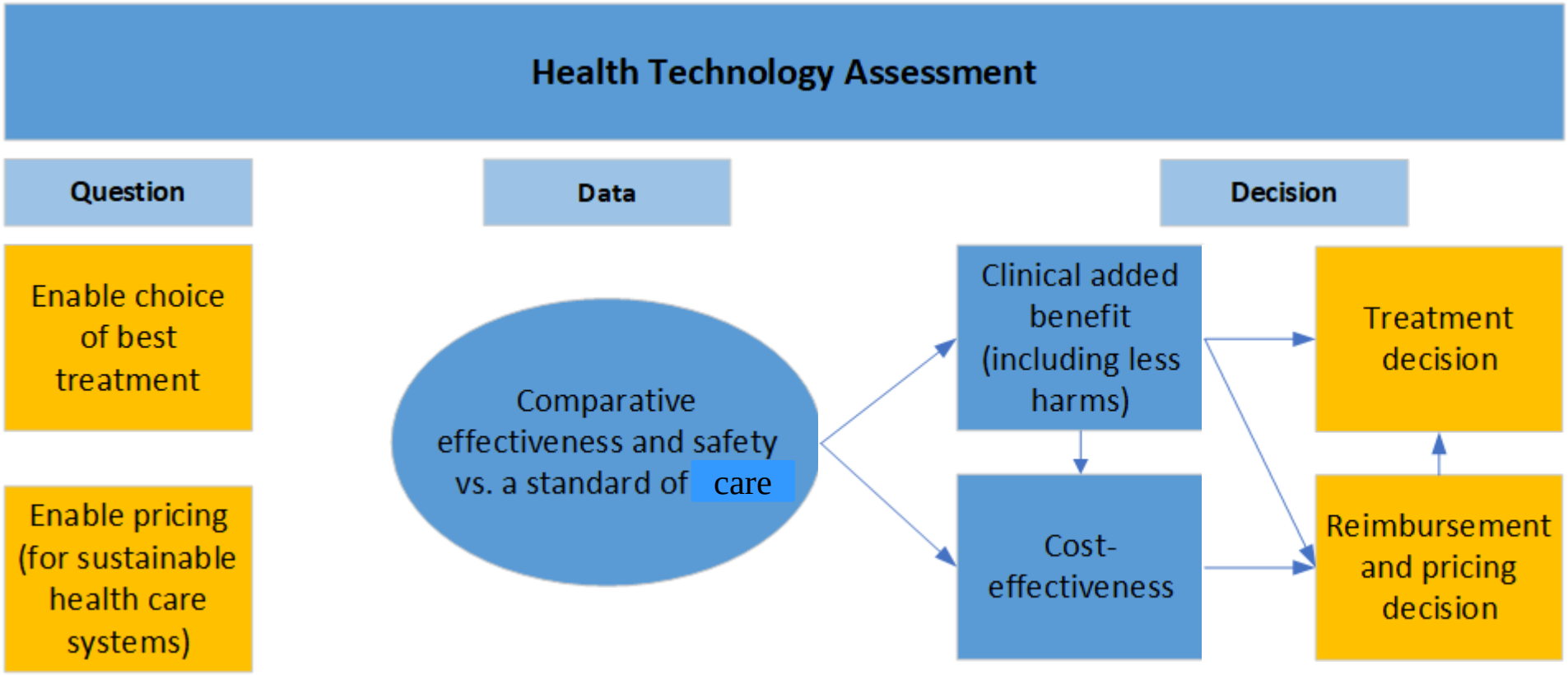
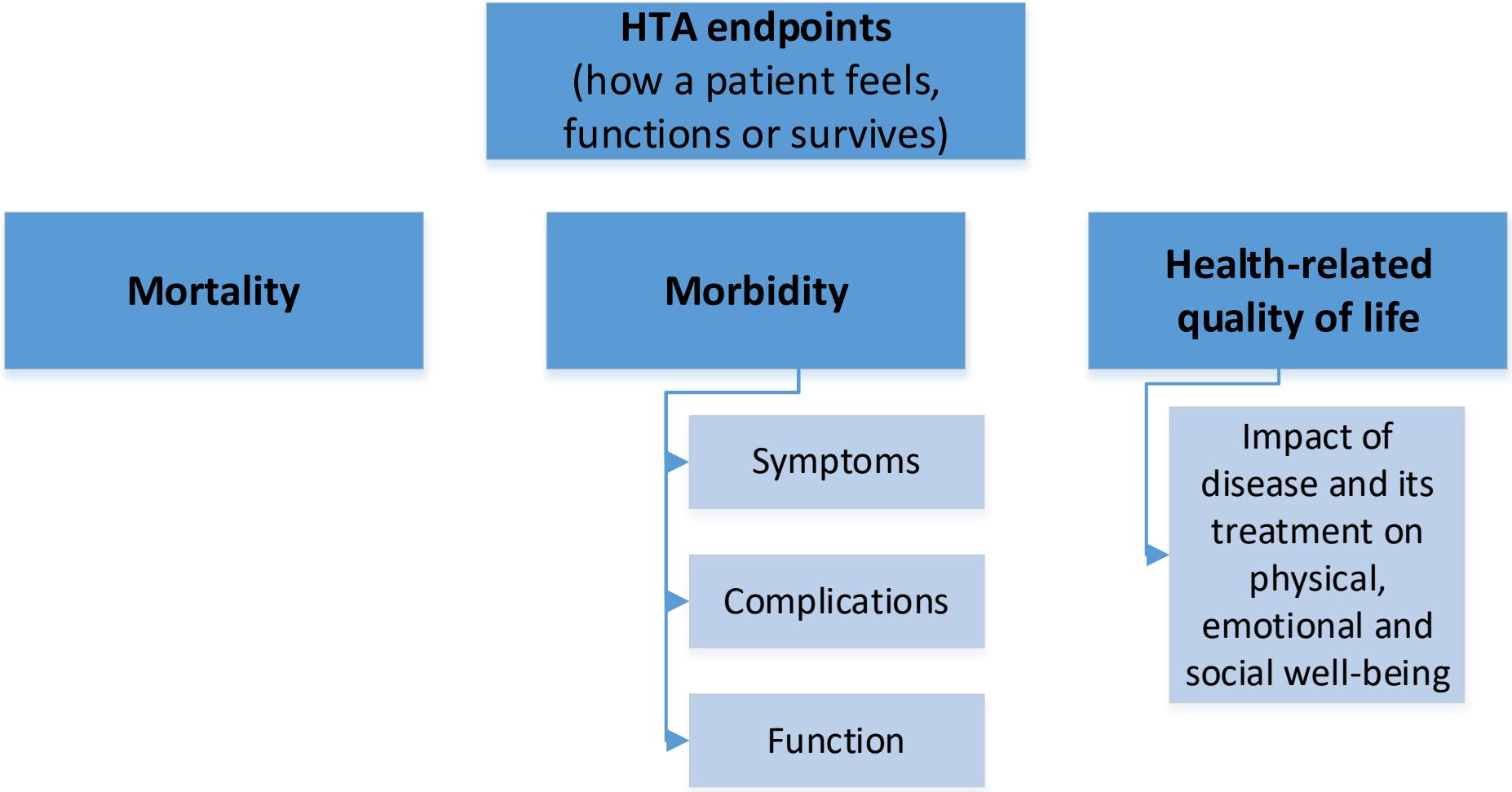
Agenda

- Decision making based on HTA
- Use of PRO (including HRQoL) in HTA
- General challenges with PRO
- Specific issues from a HTA perspective
  - Post-progression PRO data collection
  - Item lists

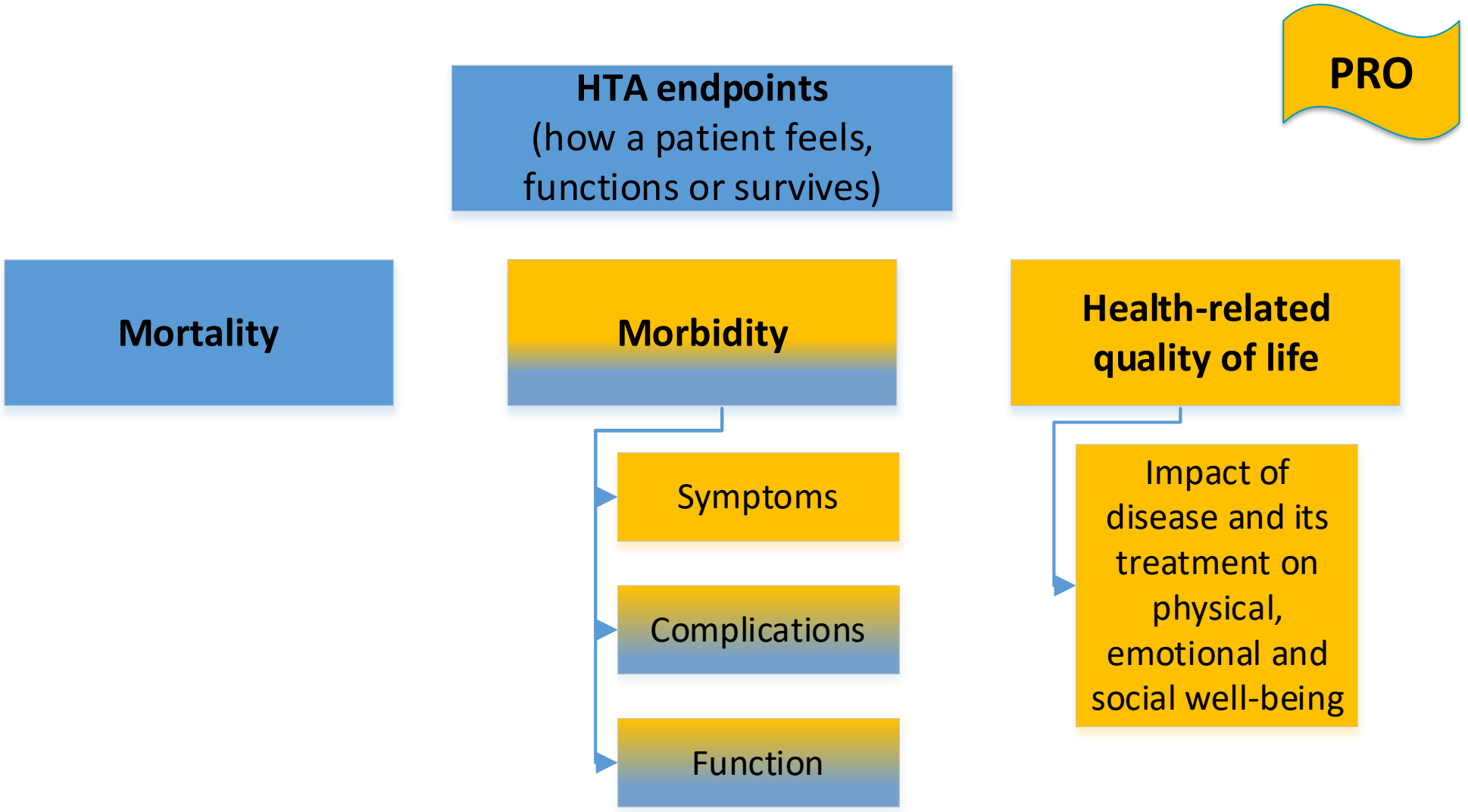
Decisions supported by HTA



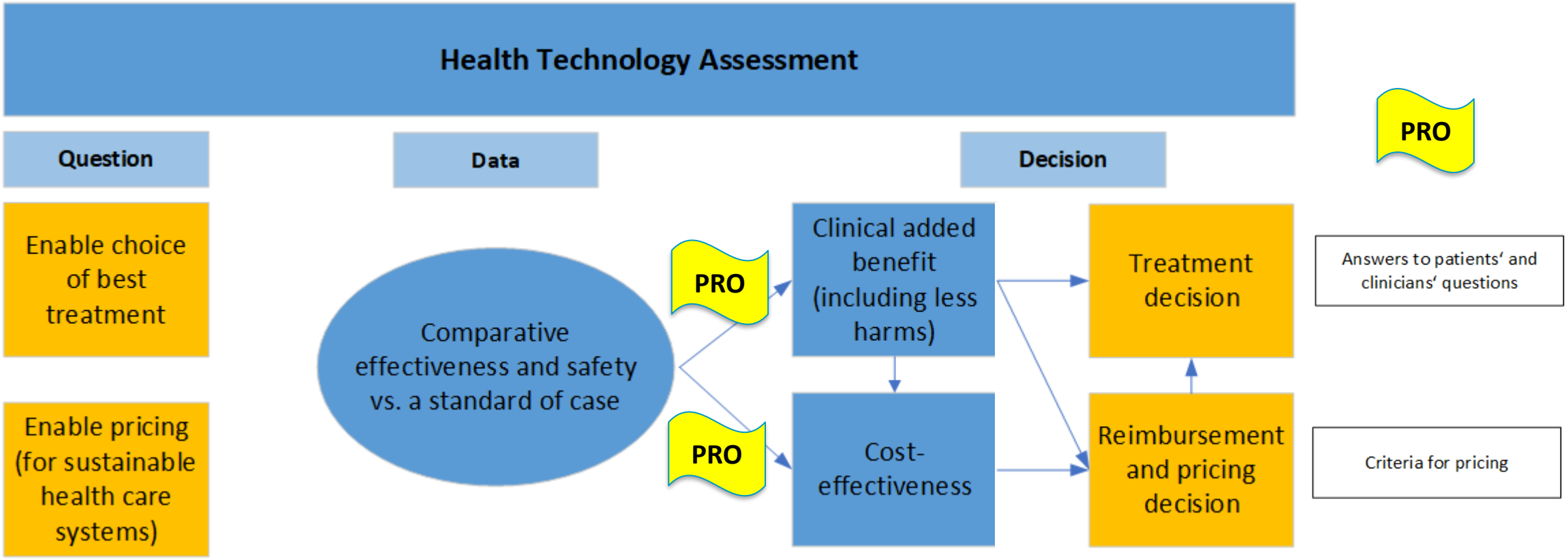
Relevance of PROs in HTA



Relevance of PROs in HTA



Decisions supported by HTA



Current challenges with PROs

- Methods used for planning studies as well as collecting, analysing and reporting PRO data are often less robust tha[illegible] other endpoints
  - insufficient pre[illegible]of PRO data collection and analysis
    - PROs as explora[illegible]
    - no pre-specification of a[illegible]
    - even not reported in the main Cl[illegible]
  - issues with interpretation of PRO resu[illegible] / treatment group differences)
  - problem of missing data due to insufficient follo[illegible]llection
  - most of this can easily be solved by applying standard [illegible]O endpoints
  - specific recommendations for the analysis of PRO endpoints are[illegible]der development by the SISAQoL initiative
  - approaches to avoiding missing data include patient involvement in the planning of the studies

We have to insist on robust methodology because PROs are such important endpoints for HTA

Post-progression PRO data collection

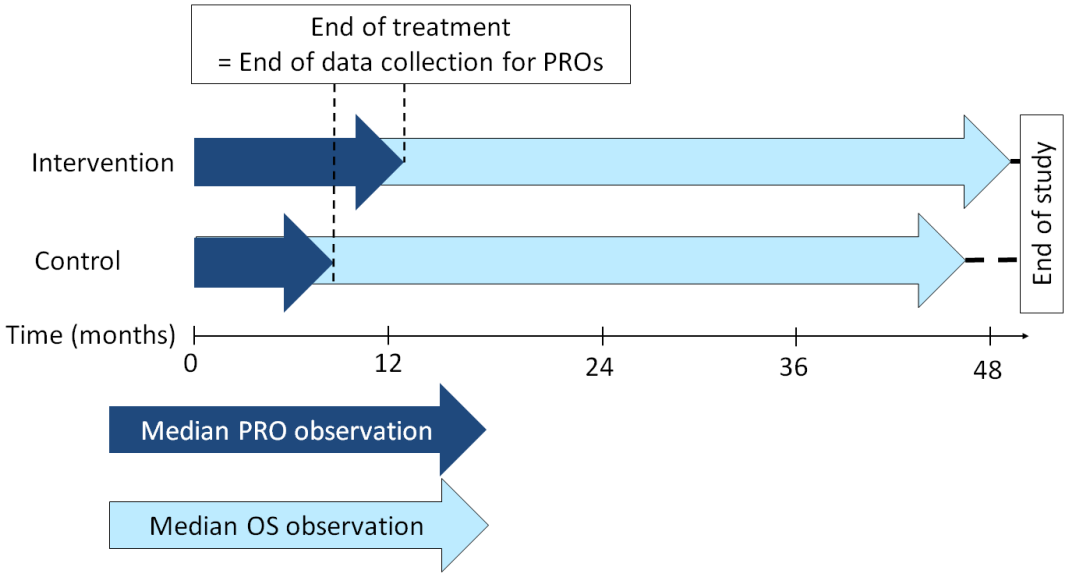


Figure 1: Median observation periods for PROs and overall survival in the clinical study MONARCH 2

Current standard of study design

- Limited PRO data collection does not answer our questions
- Differences in observation period between treatment arms pose additional problems

King-Kallimanis BL et al. Perspectives on Patient-Reported Outcome Data After Treatment Discontinuation in Cancer Clinical Trials. doi: 10.1016/j.jval.2023.06.019.

Item lists

- Item lists may be a possibility to optimise data collection for a specific disease or treatment
- Robust methods for item list development required to avoid selective compilation of items (not covering the complete construct/symptoms of interest)
- Caveat:
  - HTA is interested in (fair) comparative effects, therefore, item lists need to capture the characteristics of both the test intervention and the comparator(s)
  - HTA may use indirect comparisons across studies, therefore, instruments need to be standardised between studies and over time
  - A set of studies with different isolated item lists would be less relevant

Conclusion

- PROs (including HRQoL) provide important information for HTA, they have the same relevance as other endpoints
- Collection of high quality PRO data in a clinical trial is an important step of patient involvement because these data represent the patients‘ voice
- The relevance of this data requires robust methodology for study planning, data collection, analysis, reporting and interpretation

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