Evaluating the use of treatment preference methods in oncology clinical trials

Laurie Batchelder[1], Stephanie Philpott[1], Ana Maria Rodriguez[2]

[1]IQVIA, Reading UK; [2]IQVIA, Madrid Spain

Background

- Treatment preferences assess the relative importance of different characteristics patients or other stakeholders desire in treatments.
- Cancer patients will likely have preferences for the treatment, and different characteristics will drive those preferences.
- Treatment preferences in oncology clinical trials can help developers of oncology therapies understand which aspects of the treatment patients or other stakeholders value most, and their perceptions of benefit-risk trade-offs for a treatment (Blinman et al., 2012).
- Treatment preferences may also relate to study outcomes and can be used to adjust study outcomes for preference biases, increasing outcome precision.
- Preference effects can also provide additional insights into the basis of the study response, beyond assessing treatment effects alone (Walter et al, 2014).
- Currently, there is limited understanding of the degree to which oncology trials determine treatment preferences and whether this data is being used for regulatory purposes.

Aim

- The aim of the study was to:
  - Identify the extent to which oncology trials capture treatment preferences
  - What type of methods are being used to determine treatment preferences in oncology trials
  - How common treatment preference data from oncology trials with the identified methods are incorporated into EU and US medical product labelling.

Methods

- Clinicaltrials.gov was used to identify use of treatment preference methods in oncology trials in Europe and the USA (search conducted 8th October 2021).
- All identified trials were double screened for relevance using the pre-defined inclusion and exclusion criteria, and characteristics were extracted.
- Once selected, FDA and EMA drug label databases, including drugs@fda (accessdata.fda.gov), FDALabel and EMA SMPC, were reviewed to help supplement regulatory information for the selected trials. PubMed was also used to identify published results from the selected trials and provide additional details of treatment preference methods from the identified trials (when applicable).

Records were included if they were:
• Completed cancer or neoplasm studies conducted in Europe or USA
• No limit on trial phase
• Sample represented adult patients (≥18 years of age) with any cancer diagnosis
• Treatment preferences included as the outcome measures (primary, secondary or exploratory) and captured either qualitatively (interviews or focus groups), quantitively (treatment preference surveys or exercises) or mixed methods study design
• No time limit specified
Records were excluded if they were:
• Studies with patients < 18 years of age
• Studies conducted not in Europe or the USA
• Studies not explicitly measuring treatment preference as an outcome (e.g., decision support tools, preferences for end-of-life care)

Results

- Of 126 records identified, 24 oncology clinical trials were included (Europe: n=15; USA: n=9). Study start dates ranged between 2005-2021 (data not shown). Full results from the extracted studies (n = 24) are detailed in Supplementary Table 1 (Handout)
- **Phase type:**
  - › The majority of studies were either Phase 3 trials (n=7), or identified as 'Not applicable' (n=6). Others included Phase 1 (n=2), Phase 2 (n=5), or Phase 4 trials (n=4)
- **Therapeutic area:**
  - › 6 studies included patients with breast cancer, 4 studies with patients with prostate cancer, and 4 studies with various cancer types
- **Number of patients:**
  - › All studies (n=24) generated treatment preference data from patients
  - › Number of patients enrolled in the trials ranged from 5 to 743
- **Treatment preference objectives:**
  - › The majority of studies assessed patient preferences for drug treatment or therapy (n=10) or assessed preferences for different modes of administration for the drug treatment of therapy (n=6) (Figure 1)
- **Treatment preference positioning in outcome hierarchy:**
  - › The majority of studies reported using treatment preference data as a secondary and/or exploratory outcome (n=18), whereas 11 studies reported capturing treatment preference data as a primary outcome (Figure 2)
- **Methods used to capture treatment preference data:**
  - › 7 studies captured treatment preference data using a validated treatment preference questionnaire, 7 studies used an unspecified or unvalidated treatment preference questionnaire, and 7 studies used a single treatment preference question (Figure 3)
- **Planned analysis:**
  - › Most studies reported assessing treatment preference data descriptively (n=14), while other studies did not specify the analysis for the treatment data (n=5)
- **Use of preference data in clinical trials for label claims**
  - › Only 2 studies showed evidence where the treatment preference data was used for label claims; these studies used a validated treatment preference questionnaire (PPQ), and patient interviews

Figure 1. Treatment Preference Objectives in Oncology Trials (n=24)

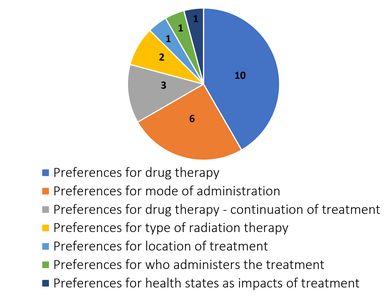


Figure 2. Summary of Oncology Trials Reporting Treatment Preferences as Primary or Secondary / Exploratory Outcomes (n=24)

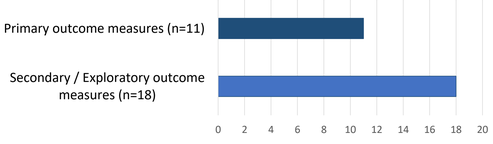
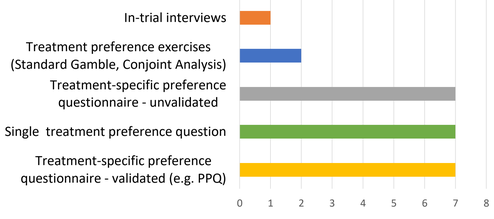


Figure 3. Assessment of Methods Used for Treatment Preferences in Oncology Trials (n=24)



Discussion and Conclusion

- The aim of the study was to identify the extent to which oncology trials capture treatment preferences, which methods are used to determine preference and how common this data is incorporated into medical product labelling.
- This review demonstrated that out of the oncology trials identified capturing treatment preferences, they are being incorporated across the lifecycle of drug development (early to post-approval), and there were a variety of methods used to determine treatment preferences.
- Findings where methods that included a validated questionnaire (PPQ) and patient interviews were included in a label claim in two instances, and it is unclear whether one method should be preferred over another.
- One limitation of the current study is that the true measurement of treatment preferences in oncology trials may not be represented only by trials registered on clinicaltrials.gov. Other non-interventional studies looking at treatment preferences in oncology in relation to clinical trials (e.g., standalone interview studies or surveys including treatment preference questions) may not be registered on the clinicaltrials.gov website.
- These findings have impacts on trial designs, outcomes, and decisions, and there may be a need to standardize approaches to capture treatment preference data for regulatory endorsement.

Blinman, P., King, M., Norman, R., Viney, R., Stockler, M.R. (2012). Preferences for cancer treatments: an overview of methods and applications in oncology. Annals of Oncology, 23(5): 1104-1110.
Walter, S.D., Turner, R., Macaskill, P., McCaffery, K.J., Irwig, L. (2014). Beyond the treatment effect: Evaluating the effects of patient preferences in randomised trials. Statistical Methods in Medical Research, 26(1): 489-507.

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