The Value of Discrete Choice Experiments to Identify Gaps in Care for Patients with Rare Diseases

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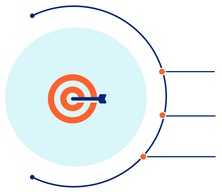
INTRODUCTION

- Discrete Choice Experiments (DCEs) are increasingly recognized within Health Economics and Outcomes Research (HEOR) as a robust method for quantifying treatment preferences and enhancing the interpretability of real-world evidence (RWE).
- When combined with precision-patient- finding enabled with medical and pharmaceutical claims, DCEs can be used to understand treatment preferences in rare diseases and amongst specific patient groups providing even more valuable insights and learnings.
- By systematically eliciting trade-offs individuals are willing to make among competing attributes—such as efficacy, safety, cost, and convenience—DCEs provide insights into value perceptions that traditional RWE sources may overlook.

OBJECTIVE



This study aims to:

Describe the literature where DCEs are utilized to quantify treatment preferences

Summarize the methods used and insights gained

Identify areas for improving the inclusion of patient-reported preferences in treatment decision making..

METHODS

A targeted review of recent literature was conducted to identify studies that evaluated patient reported/directly elicited preferences for oral, intravenous (IV) or subcutaneous (SC) treatment for non-small cell lung cancer (NSCLC). The initial search was executed in March 2025 in the PubMed database, limited to human studies published in English between January 2015 and December 2024. A grey literature search was also conducted using Google and bibliographic reviews of relevant reviews.

PICOTS-G
Population
Oncology patients - cancer, oncology, oncology field, oncologies, growth, tumor, malignancy, malignance, melanoma, sarcoma, malignant cells, lymphoma
Intervention
Oral - orals, oral by mouth, per os, per oral route, oral route, oral route of drug; Intravenous - Intravenous, injection, injecting, intravenous route, intravenous route of drug; Subcutaneous - subcutaneous, subcutaneous injection, hypodermic, subq, superficial fascia
Comparator
Oral, IV, SC (compared either or all treatment routes)
Outcomes
Treatment Preference
Time Frame
2015-2024
Geography
United States

RESULTS

The initial PubMed search yielded 2,891 articles. After screening and exclusion, 42 studies were selected for full-text review. After review, 15 studies[1-16] were identified and included in this analysis, five[13-17] from grey literature.

Design	Cross-sectional (13) Randomized Controlled Trial (3)
Patient sample size	≤100 (4) 101-200 (7) 201-300 (1) >300 (3)
Methodology	Patient preference questionnaire (4) Discrete choice experiment (11) Best-word scaling (1) Modified threshold technique (1) Treatment satisfaction questionnaire (1)
Diagnoses	Breast cancer (3) Follicular lymphoma (2) Hepatocellular cancer (1) Leukemia (1) Melanoma (1) Multiple myeloma (1) Myelodysplastic syndromes (1) Non-small cell lung cancer (2) Ovarian cancer (1) Prostate cancer (2) Any cancer (1)

KEY FINDINGS

Preference Elicitation Methods in Patient-Centered Research

11 studies used **Discrete Choice Experiments (DCE)**[1,3-5, 7-10, 12, 14, 15]
1 study combined **DCE + Best-Worst Scaling** [3]
1 study used **Modified Threshold Technique** [13]
4 studies used **Preference Questionnaires** [2, 6, 11, 16]

DCEs addressed:

- Efficacy (progression-free survival, life extension, overall survival, etc._
- Safety (side effects changes, reduction in adverse events and toxicity-free days, etc.)
- Treatment requirements (mode and frequency of administration, duration of treatment, etc.)
- Costs (out-of-pocket and insurance-related)

Patient preference questionnaires addressed:

- Reasons for preferences included: less emotional distress, less clinic time, lower injection-site pain, more comfort during administration

Patient preferences for modes of treatment administration

9 studies compared **Oral vs. IV infusion**

- Patients preferred oral therapy over IV infusion when no other attributes were considered.[13]
- In a study of patients with prostate cancer,[10] mode of administration was least important, compared to pain control, side effects and time to metastasis.
- In a study of patients with ovarian cancer,[4] dosing regimen was less influential than personal cost or progression-free survival (PFS).
  - When cost and PFS were held constant 49% chose monthly IV (cognitive symptoms, no nausea/neuropathy), 47% chose daily oral (nausea, no cognitive/neuropathy), and 4% chose weekly IV (mild neuropathy + cognitive symptoms)[4]
- In a study of advanced hepatocellular carcinoma[13] the therapy's adverse event profile offset its utility compared to IV therapy.

KEY FINDINGS, cont.

- In relapsed/refractory follicular lymphoma[8] patients preferred all-oral treatments and were wiling to trade PFS for oral administration over weekly IV infusions.
- Patients with NSCLC [7,17] preferred a therapy profile with the longest PFS and the lowest severe safety risk. DCE assessed trade-offs between benefits, safety and dosing convenience. Patients were willing to trade 7-8 months of PFS to switch from IV to oral treatment.

1 study compared **Oral vs. SC injection**[9]

- In a study of prostate cancer patients, those who favored an oral route of administration included the highest proportion of ADT-naïve patients while those who favored SC injection preferred infrequent injections, symptom resolution and lower out-of-pocket costs and had the highest proportion of ADT-experienced patients.

4 studies compared **IV infusion vs. SC injection**[2,11,14,16]

- 3 of 4 studies reported strong patient preference for SC injections [2,11,16] citing reduced clinic time and comfort during administration.
- In a study of breast cancer patients, preference was for life extension followed by out-of-pocket costs, route of administration and availability of tests to evaluate treatment efficacy.

1 study compared **Oral vs. IV infusion vs. SC injection**[5]

- While disease recurrence has the highest relative attribute importance, level of fatigue, number of health care visits, route of administration and frequency of administration were also identified as important.
- Patients reported being willing to accept a 2-3% increase in risk of recurrence as a trade off, preferring oral therapy to IV or SC administration.
- Where oral therapy was not available patients preferred SC.

Additional findings

- 6 studies reported associations between patient characteristics and patient preferences[3,6,9,13,14,15] including:
  - age (specifically 65 years or older, compared to younger patients),
  - family history,
  - education (e.g., patients with a 4-year degree or higher education readily switched from oral to IV infusion considering the risk of hand-foot skin reaction and diarrhea),
  - disease state (metastatic vs. not metastatic),
  - stage at diagnosis, and
  - mutation status (endocrine refractory HR+ vs. TNBC breast cancer).

  suggesting that patient preferences aren't one-size-fits-all.

- Patients were willing to trade 1.3-11.4 months of PFS in order to decrease side effects or change between modes of administration. Patients with fewer lines of therapy (LOTs) (≤1) were more likely to try more toxic treatments or those with lesser benefits, while patients with higher LOTs (≥2) were likely to trade more PFS for a less intensive mode of treatment administration than those with fewer LOTs.[17]

CONCLUSION

The most frequently reported attributes influencing patient treatment preferences were **efficacy (especially progression-free survival)**, **safety (side effect profiles)**, **mode of administration**, and **cost**. Patients consistently valued treatments that offered longer survival, fewer adverse events, greater convenience (e.g., oral over IV), and lower out-of-pocket expenses.

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