ENGAGING PATIENTS AND CAREGIVERS IN AN EARLY HEALTH ECONOMIC EVALUATION:

A CASE STUDY OF CHIMERIC ANTIGEN RECEPTOR T-CELL THERAPY FOR HEMATOLOGIC CANCERS

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BACKGROUND

Although patient engagement has been a part of the overall health technology assessment (HTA) process, **less is known about how patients can and want to be engaged** in each HTA component, such as in **economic evaluation**.

To advance health technology innovation, we need to **understand and incorporate patient treatment preferences and priorities** in health economic evaluations so that their true value is reflected in evaluations and the subsequent resource allocation decision-making. (Bridges, 2005)

STUDY OBJECTIVE

To **identify patient and caregiver treatment priorities** to inform the conduct of an early economic evaluation of Chimeric Antigen Receptor (CAR) T-cell therapy for adults with relapsed or refractory B-cell acute lymphoblastic leukemia.

METHODS

We conducted **2 online group discussions** with patients with experience of hematological cancer and their caregivers.

We used an online adaptation of the **Nominal Group Technique**, a structured discussion approach, which generated ideas and priorities in response to questions designed to elicit reflection on the economic evaluation decision problem. (McMillan et al., 2016)

Nominal group discussions

Idea generation	Round robin	Clarification and grouping of similar content	Ranking	Results discussion
Silent brainstorming of ideas	Sequential participant sharing of ideas	Group discussion to clarify, refine, and group ideas	Feedback form to allow participants to rank the ideas and themes	Asynchronous results discussion via email
DURING ONLINE DISCUSSION				FOLLOWING ONLINE DISCUSSION

Nominal group analysis

Member-checking	Primary analysis	Thematic analysis	Secondary analysis
Discussion of participant-generated ***ideas*** with knowledge user and patient partners	Ranking participant-generated ideas via scores and voting frequencies to identify ***priorities***	Independent generation of a coding framework by a researcher and patient partner, and follow-up discussion to agree on the overarching framework to group priorities into overarching ***themes***	Ranking of ***themes*** via the summing of scores and voting frequencies for all ideas coded to each theme

RESULTS

Data on the **sum of scores for each idea** (i.e., 1st priority = 5 points, 5th priority = 1 point) and **voting frequency** (i.e., the number of participants that voted for the idea) were used to inform the **priority list** based on each of these measurements and for each stakeholder group and question. (McMillan et al., 2014)

A **thematic framework** was developed to enable the comparison of priorities between groups by creating overarching themes. Using the thematic framework to group ideas across both groups, the ranked priority list for the top five themes for each question were:

Q1. What are the ***important costs/sacrifices*** that come to mind for you when you think about a blood cancer treatment, such as CAR-T therapy?

1. **Emotional toll of treatment(s) to patients**
2. **Direct cost(s) of clinical care**
3. **Physical toll of treatment(s) to patients**
4. **Influence on employment/education**
5. **Indirect cost(s) of clinical care**

Q2. What ***benefits or potential treatment effects/outcomes*** are important to you when considering different treatment options for blood cancer?

1. **Treatment efficacy**
2. **Treatment side effects**
3. **General quality of life**
4. **Treatment access**
5. **Treatment length**

DISCUSSION & CONCLUSION

Engaging patients to better understand their treatment priorities provides **insight into the ways in which modelers should:**

- **Conceptualize the decision problem**
- **Design health economic evaluation models**

Our findings highlight patient priorities that extend beyond the health care sector, suggesting that a broad societal perspective, which captures all relevant costs and outcomes, although outside of healthcare sector, may better align with patients' lived experience.

Further research should explore how to incorporate societal impacts in economic evaluations and HTA deliberation. In doing so, additional effort should involve communication with patients about the implications of such a change, including a reduction in the operant threshold for determining cost-effectiveness.

This research employed **novel methods to engage patients and caregivers** in the generation of patient priorities to inform an early economic evaluation and at a higher level as a patient partner in the conduct of the research.

Inform similar approaches to **patient and family caregiver engagement** in future early and late stage economic evaluations.

GET IN TOUCH!

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Read our study protocol!

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