HTA227

A Cross-Stakeholder Approach to Support Acceptance Of Non-OS Endpoints in Oncology HTA Decision Making

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Background

Fig 1. Reliance on OS data in 1L / 2L MM and EBC: Implications for patients

CHALLENGES WITH OVERALL SURVIVAL (OS) RELIANCE
in 1/2L Multiple Myeloma (MM) and Early Breast Cancer (EBC)

LONG DURATION OF STUDIES	CONFOUNDING BY SUB. THERAPIES	INABILITY TO REFLECT FULL VALUE
Time to demonstrate OS is c.10 years in 1L MM, and longer in EBC	*In 1/ 2L MM patients will receive multiple further therapies*	*Patients & physicians have health / treatment priorities beyond OS*
Delayed access to potentially life-saving / life-extending therapies	**Denied access** to potentially life-saving / life-extending therapies	**Reduced access** to therapies with improved outcomes beyond survival

NON-OS ENDPOINTS (EPs)

PREDICTION OF OS	QUALITY OF SURVIVAL
Non-OS EPs can be captured earlier, with less confounding, and may predict survival	Non-OS EPs and patient-reported outcomes (PROs) can reflect broader value (symptoms, function, quality of life (QoL), future treatment)

Aims

- To support a future in which **evaluations of new therapies result in the best outcomes for patients**, this research aims to:
  - A Review value drivers for novel therapies in MM and EBC
  - B Review key concerns around use of non-OS EPs to capture value drivers in MM and EBC
  - C Develop cross-stakeholder actions to overcome barriers and drive greater use of non-OS EPs in the absence of OS

Methods

LITERATURE REVIEW OF NON-OS EPs AND PROs

85 INTERVIEWS WITH Ex-PAYERS, REGULATORS, HCPs & PAGs

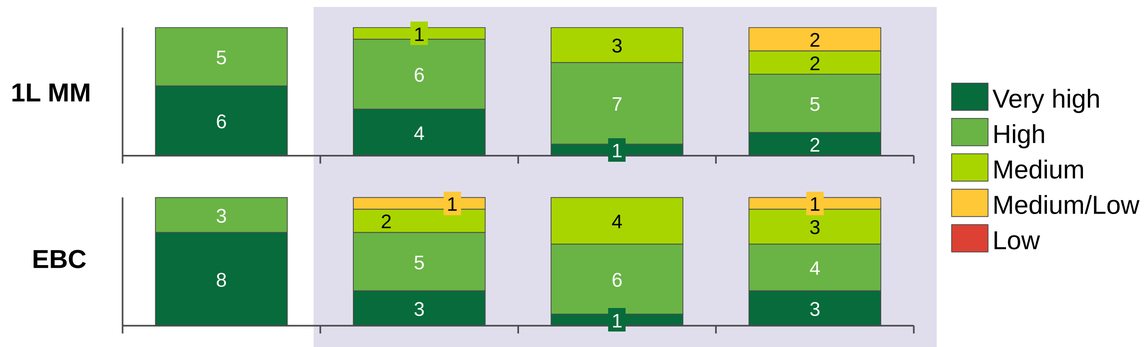
CROSS STAKEHOLDER 1:1 AND GROUP DISCUSSIONS
Round-tables

Results

A Value drivers in multiple myeloma and early breast cancer

- Payers/HTAs consider mortality to be the most important outcome in 1L MM and EBC, but morbidity, QoL and adverse events (AEs) are also highly valued (*Fig 2*)
- In MM, clinicians view OS as most important, followed by QoL, but note that some patients put higher emphasis on QoL; in EBC, clinicians view OS as becoming a less relevant end goal, with the key objective to keep patients disease-free (*Fig 3*)
- From the patient perspective, having a more manageable disease is highly valued (*Fig 4*)

Fig 2. Payer/HTA perspectives on the importance of different outcomes in 1L MM and EBC



Quality of survival

Fig 3. Physician perspectives on the importance of different outcomes in 1L MM and EBC

*".. In MM, survival and QoL are ultimately the most important things to patients, survival is still key but **the balance between the two depends on the patient**…"*
MM clinician

*"…The **more you are able to make cancer a chronic disease, the less OS is a valid endpoint**, that is the balance. HIV is an excellent example and EBC is going in the same direction…"*
EBC clinician

Fig 4. Patient perspectives on the importance of different outcomes in 1L MM and EBC

Patients are more accepting of non-OS EPs than other stakeholders, as part of their desire for a longer life (or cure), focusing on how to **make the disease more manageable**, and are **strong advocates of PROs**

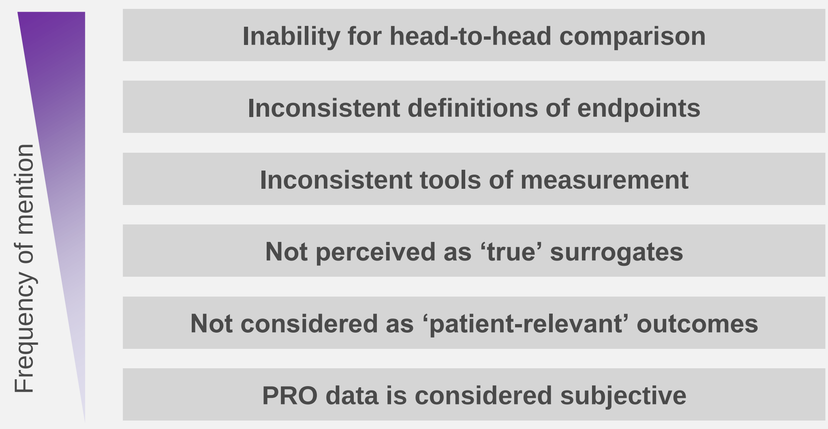
*"…There is a need to make treatment side effects and QoL more central to HTA decision making as **it is important to understand the patient journey**…"* PAG / patient

Results (*cont.*)

B Key concerns around the use of non-OS EPs to capture value drivers

- Payers are open to the use of non-OS endpoints and PROs, but see a number of barriers to greater adoption of these endpoints in HTA decision-making (*Fig 4*)

Fig 4. Potential barriers to greater acceptance of non-OS endpoints and PROs



C Cross-stakeholder actions to drive greater acceptance

- Stakeholders should work together to define suitable frameworks and methods for the broader adoption of non-OS endpoints (*Fig 5*)

Fig 5. Strategic imperatives to increase the use of non-OS EPs in oncology

Improve knowledge and build confidence in the CORRELATION OF ENDPOINTS WITH OS

- Develop **'payer-friendly'** guidelines for the use of non-OS endpoints as surrogates to build a common understanding
- **Increase standardisation** of non-OS endpoints across trials to increase ability to detect surrogacy
- Establish robust **RWE / observational data registries** to instil confidence in correlation of non-OS endpoints with OS

Ensure more PATIENT-CENTRIC EVALUATIONS of novel therapies, with greater emphasis on QUALITY OF SURVIVAL

- Development of **'patient-friendly' educational materials**, to ensure patients understand the meaning of PROs
- Understand evidence for the **clinical, social and economic value** of disease related measures (delay to progression, disease-free survival, tumour eradication with no detectable disease) beyond predicting survival
- **Give more weight to PROs** to complement other non-OS endpoints

Address payer concerns around ATTRIBUTION OF VALUE TO NON-OS ENDPOINTS AND PROs

- Use **conditional access** to accelerate access whilst generating evidence to reassure payers that price reflects long-term benefit & value
- **Early engagement with stakeholders to gain** buy-in to trial design and approach

Conclusions

One size may not fit all, and new approaches may be needed to ensure patients get access to life-extending or life-improving drugs in a timely way.

Reliance on OS data in MM and EBC presents key limitations that may hinder patient access to innovative therapies; there is now an urgent need for all stakeholders to work together to address concerns around non-OS endpoints in order to ensure that future HTA assessments of novel therapies result in the best outcomes for patients

Patient advocacy group · Oncologist · Health Economist · Regulator · Payer

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