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 1L Multiple Myeloma (MM) and Early Breast Cancer (EBC)

- **Payers/HTAs** consider mortality to be the most important outcome in 1L MM and EBC, but morbidity, in particular mortality, is not as highly valued (Fig 2).
- **Stakeholders** should work together to define suitable frameworks and methods for the broader adoption of non-OS endpoints in 1L MM and EBC (Fig 3).
- **Key concerns** around the use of non-OS endpoints is the requirement for greater acceptance of non-OS endpoints and PROs (Fig 4).
- **Cross-stakeholder actions** to drive greater acceptance of novel therapies, with greater emphasis on patient-centric evaluations (Fig 5).

Aims

- To support a future in which evaluations of new therapies result in the best outcomes for patients, this research aims to:
  - Review key concerns around the use of non-OS endpoints (Fig 3).
  - Develop cross-stakeholder actions to overcome barriers and drive greater use of non-OS endpoints in the absence of OS (Fig 5).

Methods

- **Literature Review of Non-OS EPs and PROs**
- **Interviews with Payers, Regulators, HCPs & PAGs**
- **Cross-Stakeholder 1:1 and Group Discussions**

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 more emphasis on OS 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).

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

C Cross-stakeholder actions to drive greater acceptance

- 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.
- Give more weight to PROs to complement other non-OS endpoints.

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

- Use conditional access to accelerate access while 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.

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