Clinial Outcomes in HTA Appraisal Reports of Oncology Medicines in Portugal: A Cross-Sectional Analysis

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OBJECTIVE

In **Portugal**, the pharmacotherapeutic evaluation within the **Health Technology Assessment** (HTA) process applies the **PICO methodology**, ensuring that outcomes with the greatest clinical impact remain central to **assessing therapeutic value**. This work seeks to **characterise the efficacy and safety outcomes** considered in the HTA processes of **oncology medicines** to support reimbursement decisions [1][2].

METHODOLOGY

Included **public reimbursement reports** of **oncology medicines** (INFARMED, I.P., **2023–2024**), only reports with an approval or rejection reimbursement decision.

Data collection
Sep 2024 – Jan 2025

Data organised by **year** and **decision type**

Extracted and analysed **PICO framework** (efficacy and safety outcomes)

Outcomes classified as **critical** or **important** for the assessment of added therapeutic value and reimbursement[3][4]

RESULTS

55 Reports Analysed

Total: 52 approved and 3 rejected.
2023: 23 approved and 1 rejected.
2024: 29 approved and 2 rejected.

Table 1- Proportion of reports identifying efficacy outcomes as critical and as prioritised for reimbursement decisions.

Outcome	Critical in reports (n)	Prioritised for reimbursement decisions (n)
Quality of Life (QoL)	49 (89.1%)	2 (3.6%)
Overall Survival (OS)	53 (96.4%)	30 (54.5%)
Progression-free Survival (PFS)	2 (3.6%)	24 (43.6%)

Table 2 - Proportion of reports identifying safety outcome as critical.

Outcome	Critical in reports (n)
Mortality	50 (90.9%)
AE grade 3-4	51 (92.7%)
Discontinuation due to AE	50 (90.9%)

- **Quality of Life (QoL)** was identified as a **critical** outcome in **89.1%** of cases (n=49), yet it was **prioritised** for reimbursement decisions in **only 3.6%** (n=2).
- **Overall survival** (OS) was cited as a **critical** outcome in 96.4% (n=53) and prioritised in 54.5% (n=30).
- **Progression-free survival** (PFS) was considered a **critical** outcome in **only 3.6%** (n=2), but it was **prioritised** in **43.6%** (n=24) of reimbursement decisions.
- Regarding safety outcomes:
  - **Mortality** was reported as **critical** in **90.9%** (n=50).
  - **Severe or grade 3–4 adverse** events were reported as **critical** in **92.7%** (n=51).
  - **Discontinuation due to adverse events** was reported as **critical** in **90.9%** (n=50).
- **None** of the reimbursement reports **prioritised safety** over efficacy outcomes in decision-making.

CONCLUSION

- **Overall Survival** predominates as the most clinically relevant outcome in the reimbursement of oncology medicines.
- In the absence of OS, PFS is preferred, although it is not always considered critical.
- QoL, often critical, is rarely used due to data sparsity.
- **Safety outcomes**, although frequently critical, are **underutilised in decision-making,** even when data on critical efficacy outcomes are lacking.
- **Greater integration** of safety outcomes into HTA evaluations warrants debate, given their influence on patient quality of life and clinical outcomes.

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CLEVIDENCE

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