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ORIGINAL PAPER

Haematological Malignancy – Clinical

Patients', clinicians' and research's priorities on important outcomes in multiple myeloma: A mixed-methods study

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Summary

Research in multiple myeloma increasingly relies on surrogate end-points to expedite approvals, yet these may not reflect patient priorities. We conducted a mixed-methods study to identify outcomes valued by patients and clinicians and compare them with end-points used in randomized controlled trials (RCTs). Interviews with 10 patients and 6 clinicians identified treatment priorities, which, together with end-points from a systematic review of myeloma RCTs, informed tailored surveys, completed by 117 patients and 105 clinicians. Both groups ranked quality of life (QoL) as most important (odds ratio [OR] 1.01; 95% confidence interval [CI] 0.55–1.87). Clinicians more often prioritized overall survival (OS) (OR 1.92; 95% CI 1.05–3.50) and progression-free survival (PFS) (OR 5.37; 95% CI 1.95–14.79), whereas patients prioritized pain reduction (OR 0.03; 95% CI 0.00–0.23). Compared with RCT end-points, patients emphasized QoL (OR 0.03; 95% CI 0.01–0.07) and pain elimination (OR 0.05; 95% CI 0.02–0.11), while trials favoured PFS (OR 6.33; 95% CI 2.53–15.83) and response (OR 17.75; 95% CI 5.56–56.61). Clinicians aligned with trials on PFS but valued QoL and OS more highly. QoL emerged as a shared priority, underscoring the need for patient-centred trial designs that better capture outcomes meaningful to those living with myeloma.

KEYWORDS
mixed-methods research, multiple myeloma, outcomes

INTRODUCTION

Multiple myeloma research has advanced substantially, with novel agents and supportive care improving survival.[1,2] Historically, randomized controlled trials (RCTs) in myeloma relied on overall survival (OS) as the primary end-point. However, a shift has occurred towards surrogate outcomes, with progression-free survival (PFS) and response becoming common primary end-points.[3,4] Recently, the U.S. Food and Drug Administration (FDA) has endorsed measurable residual disease (MRD) as a valid end-point for regulatory approval in myeloma trials.[5] In contrast, quality of life (QoL) as a primary end-point remains infrequent.[6]

While surrogate outcomes might be easier to measure and can yield statistically significant results more quickly, they often fail to reflect patient priorities.[7] This misalignment is not unique to myeloma; across chronic diseases, patients and clinicians often prioritize different outcomes.[8,9] Addressing this discrepancy requires integration of patient perspectives into research governance and design.[10,11] As myeloma becomes a chronic condition, patient-centred healthcare and shared decision-making are essential,[12–14] despite the known challenges in implementing shared decision-making in oncology.[15,16] Recent efforts to involve patients in the development of core outcome sets (COS) offer a structured approach for ensuring that research priorities are better aligned with patient values and that outcomes are more comparable across studies.[17–19]

Qualitative research methods, useful for capturing individual perspectives, are not commonly used in myeloma.[20,21] While existing survey-based studies often emphasize QoL, their findings are seldom integrated into clinical decision-making processes.[22–26] Furthermore, mixed-methods research in myeloma remains limited.[27,28] To address this gap, we conducted a mixed-methods study to capture and compare preferences of patients and clinicians regarding myeloma treatment outcomes by integrating qualitative data from interviews with quantitative survey responses. In addition, we compared these preferences with the outcomes prioritized in myeloma RCTs, using data from a published systematic review.[3]

METHODS

This exploratory sequential mixed-methods study consisted of interviews with patients and clinicians (qualitative phase) and tailored surveys (quantitative phase) (Figure 1). The qualitative component was guided by a grounded theory[29] approach within the constructivist research paradigm.[30]

The study was approved by the Bioethics Committee of the Department of Medicine, Aristotle University of Thessaloniki. We report our study in accordance with GRAMMS reporting guidelines for mixed-methods research.[31]

Qualitative study participants

We used purposive sampling, a non-random sampling technique in which participants are deliberately selected based on specific characteristics relevant to the research question, to recruit patients with myeloma for individual interviews.[32] Eligible participants were Greek-speaking adults with myeloma across different treatment stages and backgrounds, to capture a wide range of perspectives. Participants were approached during routine visits to haematology outpatient clinics or while admitted for inpatient treatment and provided written informed consent prior to the interviews.

We also recruited haematologists with diverse myeloma expertise, for one-on-one semi-structured interviews via purposive sampling. Haematologists were informed about

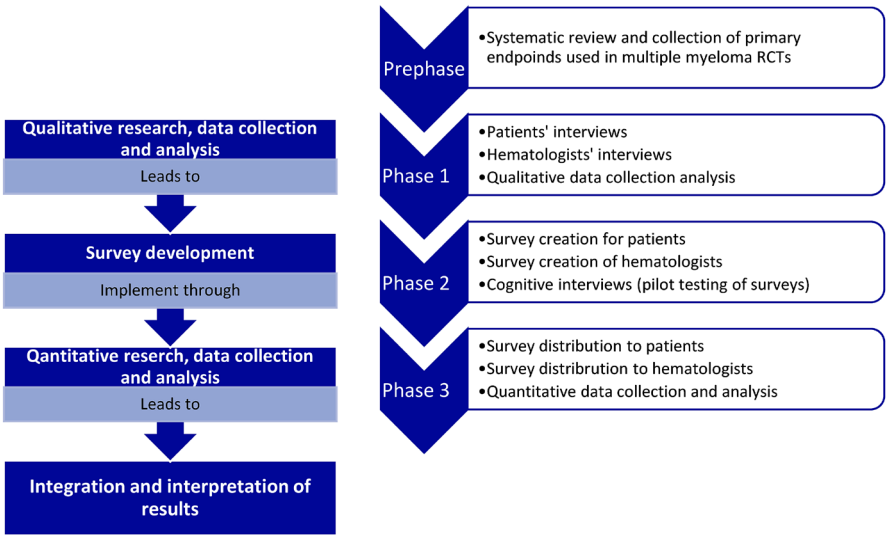

FIGURE 1 Schematic representation of mixed-methods study.

the study's procedures and objectives and provided written informed consent before the interviews.

Qualitative data collection and analysis

Semi-structured interviews (10–20 min) were conducted in Greek, audio-recorded and transcribed verbatim by the researcher. We created a flexible interview guide with open-ended questions to facilitate the interviews (supplement).

We employed thematic analysis to analyse the interview transcripts.[30,33] The process began with a broad review of the data to develop a general understanding and assess whether data saturation had been achieved. Each transcript was systematically coded; codes were grouped into categories, from which overarching themes and subthemes were developed. Finally, the themes were organized and interrelated according to the study's research aims. Initial analysis was conducted by one of the researchers, with final interpretation refined and validated through consensus among the full study team. The most frequently identified themes were then used to inform the development of the quantitative survey.

Survey development

We developed two surveys, one tailored for patients and one for haematologists. From the interview data, we identified the most consistently mentioned and thematically significant patient and clinician reported priorities, while from the top 10 RCT outcomes, items were selected to ensure coverage while avoiding redundancy. This dual-source approach ensured that the survey addressed outcomes meaningful to both patients and clinicians, while also reflecting the current landscape of clinical trial design.

The survey underwent pilot testing with 10 patients and five clinicians to ensure content validity and ease of comprehension. Feedback gathered informed important revisions to improve clarity. We identified nine key outcomes to be included in the survey presented in Figure 2. Given the conceptual overlap between several time-to-event outcomes—such as PFS, time to progression and duration of response—we chose to include only PFS, as the most commonly used primary end-point in contemporary myeloma trials[3] and its definition was refined to ensure accessible language.

The final survey included two main questions: (1) selection of five most important outcomes and (2) choice of the single most important outcome. The first question was designed to capture a broad view of each participant's priorities without forcing them to commit to a single choice, while the second question allowed for a deeper understanding of how participants rank their top priorities, helping to explore both general patterns and decisional hierarchies within each group.

The survey was administered in paper format during patient routine visits, while haematologists completed the survey electronically via a centralized data capture system (REDCap, Vanderbilt University, available at https://redcap.med.auth.gr). All responses were collected anonymously.

HEMATOLOGISTS' SURVEY

- Year of birth:
- Year of Hematology Specialization:
- Have you participated in a conference/seminar as a speaker on a drug used in the treatment of myeloma? **Yes / No**
- Have you participated in clinical trials for pharmacological treatments for multiple myeloma? **Yes / No**
- How many myeloma patients do you see per month?

0-2 5-10 10-30 >30

➢ What do you consider the most important outcome in the pharmacological treatment of multiple myeloma? **Circle the FIVE (5)** most important outcomes for you:

1. Reduces pain
2. Improves quality of life
3. Allows for outpatient treatment and/or reduces the need for hospitalization due to treatment
4. Causes fewer side effects
5. Increases overall survival
6. Extends progression-free survival
7. Leads to response (partial or complete) of the disease
8. Leads to negativity of measurable residual disease (MRD) with available techniques
9. Allows for longer treatment-free intervals
10. Other:

➢ **Of the five you circled, which do you consider the MOST important?**

PATIENTS' SURVEY

- Year of birth:
- Sex:
- Years since myeloma diagnosis:
- Have you undergone bone marrow transplantation? **Yes/No**
- Rate your satisfaction with your current treatment: **Choose 1** of the following:

Very satisfied Satisfied Neutral Dissatisfied Very dissatisfied

➢ What do you consider the most important aspect a multiple myeloma treatment should offer? **Please select the FIVE (5) most crucial outcomes for you**:

1. To not feel any pain
2. To be able to perform daily activities, work and have a good quality of life
3. To not visit the hospital a lot, or not spend a long time there for treatment
4. To not have any treatment side effects
5. To help me live longer
6. To prolong the time without the myeloma getting worse
7. To improve my labs, scans and tests that are related to myeloma
8. To eliminate the cancer cells detected by specific markers in the bone marrow
9. To have treatment free intervals (periods of time with no treatment at all)
10. Other:

➢ **From the above 5 you selected, which one is the MOST critical outcome for you?**

FIGURE 2 Patients' and clinicians' surveys.

Survey sample

We assumed a population of 5367 patients with myeloma in Greece, based on a prevalence of 45.3/100 000 individuals.[34] For haematologists, the population was estimated at 650 (society membership, https://eae.gr/). Using a 5% margin of error and a 95% confidence level, the required sample size was calculated as 359 patients and 242 haematologists. However, considering a response rate of approximately 35%, we calculated that 100 responses from patients and 100 from haematologists would yield a 9% margin of error at the same 95% confidence level, which was considered acceptable for this study.

Quantitative data analysis

We used descriptive statistics to summarize the results. Due to low expected counts, Fisher's exact test was employed to assess differences between groups. Statistical significance was defined as a p-value <0.05. To compare patients' outcome preferences with those of clinicians and both groups' preferences with the most frequently reported end-points in myeloma RCTs, odds ratios (ORs) with 95% confidence intervals (CIs) were calculated. All analyses were conducted in RStudio version 2024.09.0+375.[35]

RESULTS

Qualitative study participants

We interviewed 10 patients with myeloma (median age 65.5 years; range 49–81, 6 women). Patients were intentionally selected to represent various disease stages and treatment: three had newly diagnosed myeloma, seven had relapsed/refractory disease (including one heavily pretreated) and two had undergone autologous stem cell transplants. Additionally, we interviewed six haematologists (median age 50.5 years; range 33–64, 5 women), with varied myeloma expertise.

Thematic analysis

Themes and subthemes derived from patient and clinician interviews, along with the corresponding codes and quotes, are provided in the supplement.

The main themes identified from the patient interviews were pain elimination and symptomatic relief, psychological and emotional well-being, social integration and family life, coping with treatment and adverse events and healthcare system and support satisfaction.

Themes emerging from the clinicians' interviews included the complexity of the disease and treatment decisions, QoL, survival, disease response, patient education and key adverse events.

Survey population

We included 117 patients with myeloma, 61 (52.1%) were male, the mean age was 69.8 years (standard deviation [SD] 9.2) and the mean time since diagnosis was 4 years (SD 4.1). While most patients agreed to complete the survey, the proportion of responders relative to those invited was not recorded. A total of 105 clinicians completed the survey, 40 (38.1%) were male and the mean age was 51.3 years (SD 9.3), representing 16.2% of the 650 haematologists contacted via the Greek Society of Hematology (Table 1).

TABLE 1 Characteristics of patients and haematologists included in quantitative analysis (surveys).

Patients (n = 117)	**No. (%)**
Age	
<65	31 (27.0)
65–80	71 (61.7)
>80	13 (11.3)
Male	61 (52.1)
Years with myeloma	
<2	36 (32.1)
2–5	44 (39.3)
>5	31 (27.7)
Bone marrow transplantation	23 (19.7)
Satisfaction with myeloma treatment	
Very satisfied	83 (70.9)
Satisfied	28 (23.9)
Neutral	5 (4.3)
Dissatisfied	1 (0.9)
Very dissatisfied	0 (0.0)
Haematologists (n = 105)	
Age	
30–49	47 (44.8)
50–60	42 (40.0)
>60	16 (15.2)
Male	40 (38.1)
Year of haematology specialization	
Before 2000	21 (21.2)
2000–2010	32 (32.3)
2011–2024	46 (46.5)
Speaker in conference on myeloma drug	40 (38.5)
Participation in clinical trials	58 (55.2)
Patients per month	
<5	18 (17.5)
5–10	44 (42.7)
10–30	27 (26.2)
>30	14 (13.6)

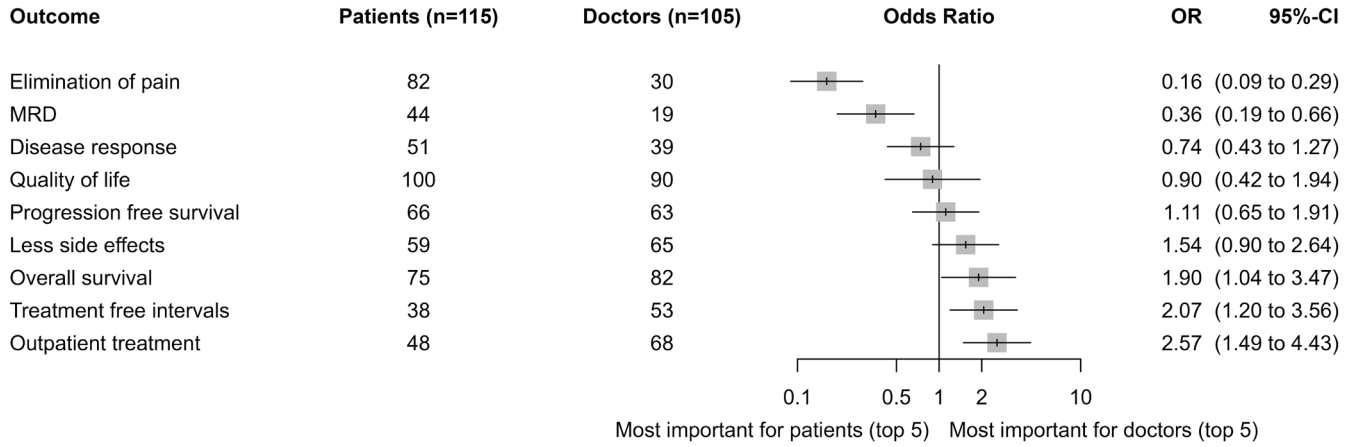

FIGURE 3 Forest plot of patient versus doctors' top five selected outcomes.

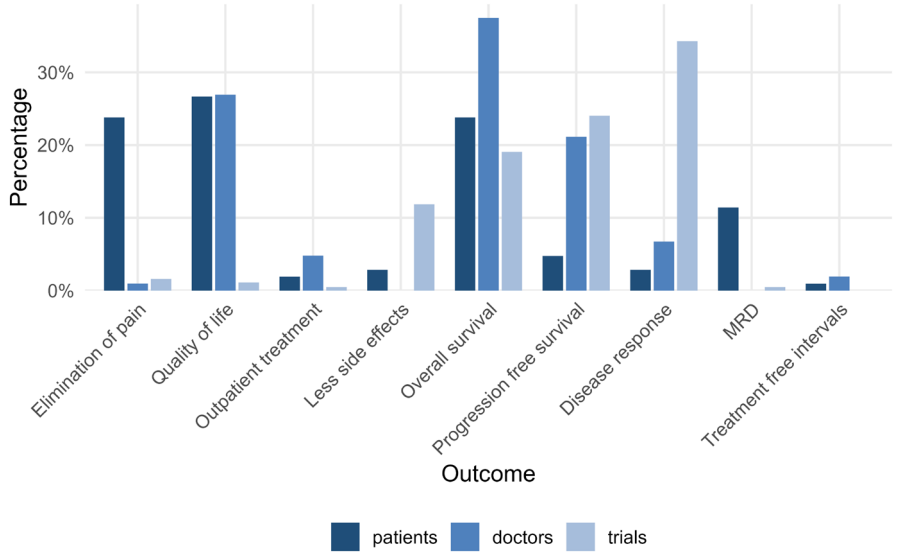

FIGURE 4 Most important outcomes for patients and doctors and most selected as primary outcomes in myeloma trials.

Agreement and disagreement in myeloma outcomes

For question 1, the most frequently selected outcomes from patients were QoL (100, 87.0%), elimination of pain (82, 71.3%) and OS (75, 65.2%) and from clinicians were QoL (90, 85.7%), OS (82, 78.1%) and reduction in the need for hospitalization (68, 64.8%) (Figure 3).

For question 2, patient preferences in descending order were QoL, elimination of pain, OS, MRD negativity and PFS, while for clinicians, they were OS, QoL, PFS, response and reduction in the need for hospitalization (Figure 4).

Patients' priorities compared to clinicians' priorities

When comparing responses to the first question, patients and clinicians showed general agreement on the importance of QoL (OR 0.90; 95% CI 1.04–3.47) and PFS (OR 1.11; 95% CI 0.65–1.91). However, patients were significantly more likely to prioritize elimination of pain (OR 0.16; 95% CI 0.09–0.29) and MRD negativity (OR 0.36; 95% CI 0.19–0.66), while clinicians more frequently selected OS (OR 1.90; 95% CI 1.04–3.47), treatment-free intervals (OR 2.07; 95% CI 1.20–3.56) and feasibility of outpatient treatment (OR 2.57; 95% CI 1.49–4.43) (Figure 3).

In responses to the second question, patients and clinicians showed general agreement on the importance of QoL (OR 1.01; 95% CI 0.55–1.87), along with similar prioritization of outcomes such as outpatient treatment, fewer side effects, disease response and treatment-free intervals. However, notable differences emerged between the two groups (Figure 5). Clinicians were more likely to prioritize OS (OR 1.92; 95% CI 1.05–3.50) and PFS (OR 5.37; 95% CI 1.95–14.79), while patients more frequently selected elimination of pain (OR 0.03; 95% CI 0.00–0.23) and MRD negativity (OR 0.04; 95% CI 0.00–0.61) (Figure S1). However, findings related to MRD

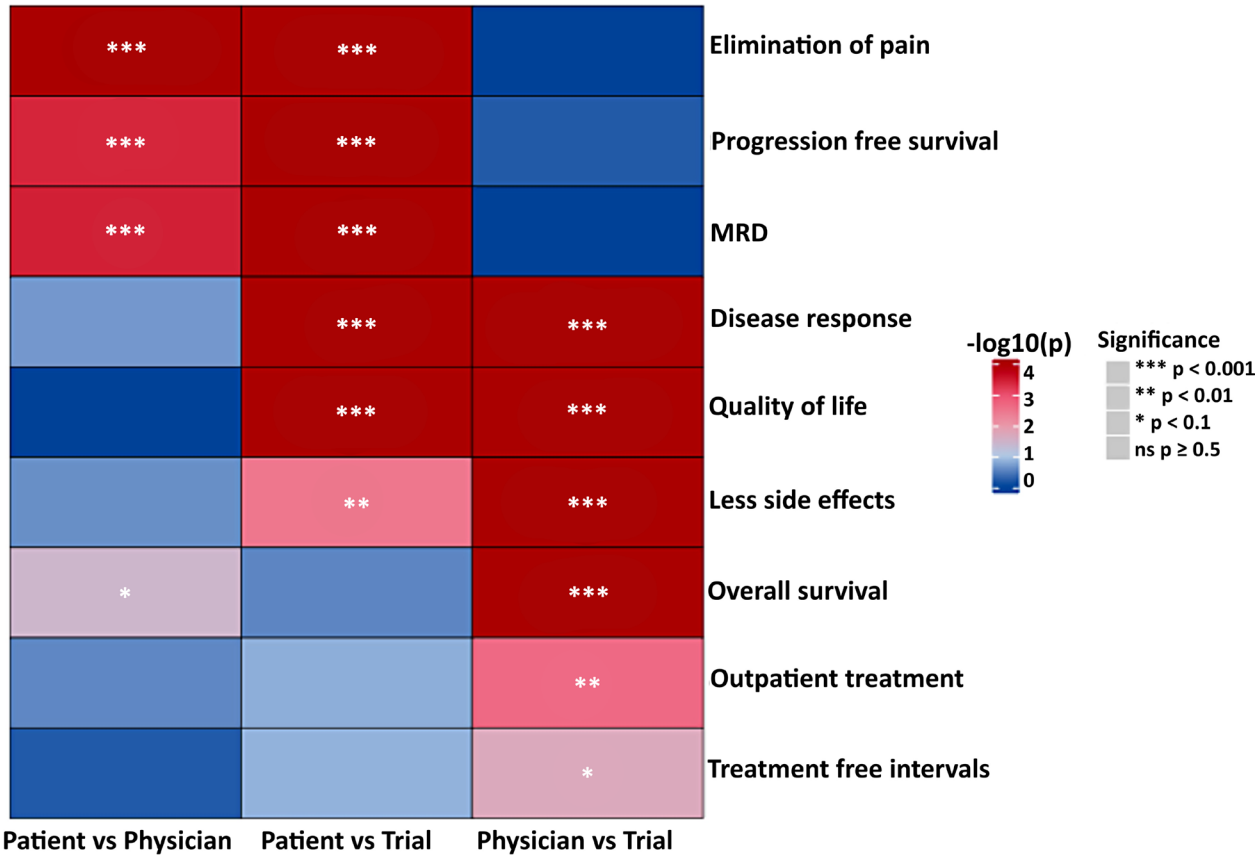

FIGURE 5 Heat map of agreement and disagreement between patients, doctors and trials.

should be interpreted with caution due to the small number of respondents selecting this outcome.

Patients' priorities compared to clinical trial end-points

When comparing patients' preferences with the most commonly used primary outcomes in myeloma trials, alignment was observed only for OS. Across all other outcomes, there were notable discrepancies between what patients prioritized and what trials typically measure (Figure 5). Patients placed greater importance on QoL (OR 0.03; 95% CI 0.01–0.07), pain elimination (OR 0.05; 95% CI 0.02–0.11) and MRD negativity (OR 0.04; 95% CI 0.01–0.14). In contrast, clinical trials relied more on PFS (OR 6.33; 95% CI 2.53–15.83), disease response (OR 17.75; 95% CI 5.56–56.61) and side effects (OR 4.57; 95% CI 1.41–14.79). However, again findings regarding MRD negativity were limited due to a low number of patients (Figure S2).

Clinicians' priorities compared to clinical trial end-points

When comparing clinicians' preferences with the most commonly used primary outcomes in myeloma trials, alignment was observed on the importance of PFS (OR 1.18; 95% CI 0.71–1.95). In contrast, notable differences emerged for several outcomes (Figure 5). Clinicians placed greater importance on QoL (OR 0.03; 95% CI 0.01–0.07) and OS (OR 0.39; 95% CI 0.25–0.61), whereas clinical trials gave more weight to disease response (OR 7.23; 95% CI 3.30–15.85). Clinicians valued treatment-free intervals (OR 0.03; 95% CI 0.00–0.69) and feasibility of outpatient treatment (OR 0.10; 95% CI 0.02–0.41). However, these comparisons were based on a small number of responses (Figure S3).

DISCUSSION

Our study shows partial alignment between patients with myeloma and clinicians. While both consistently identified QoL as central, clinicians emphasized extending OS and patients prioritized daily functioning. Patients described QoL and symptom relief as overarching goals, whereas clinicians focused more on survival outcomes while acknowledging treatment burden, side effects and frequent hospital visits. The divergence in prioritization of pain reduction, however, should be contextualized. Clinicians use corticosteroids, radiotherapy and vertebroplasty where appropriate, which can provide rapid and effective relief. As such, clinicians may not perceive pain control as a direct therapeutic goal of anti-myeloma treatment per se, but rather as a critical

aspect of adjustive care. This framing likely contributes to its lower ranking by physicians as it is not expected to alter the natural history of the disease.

Clinical trials, by contrast, have prioritized response and PFS over QoL and OS. While clinicians valued PFS, reflecting its widespread use, patients rarely chose it as the top, which is somewhat unsurprising given its complexity and limited relevance to their lived experience.[36,37] Explaining PFS to patients poses inherent challenges due to its complexity, encompassing death, clinical relapse and biochemical progression. These difficulties became evident during the development of the survey, when the initial definition of PFS required revisions following pilot testing. Notably, PFS was selected by a substantial proportion of patients when asked to identify their top five outcomes but was infrequently chosen as the single most important outcome (Figures 3 and 4 and Figure S1).

MRD negativity was not a top priority for clinicians but received some patient endorsement. However, this finding was based on a small number of responses, resulting in wide confidence intervals, and was not supported by the qualitative data. This discrepancy between the qualitative and quantitative results[38] may stem from how MRD was framed in the survey, as 'elimination of cancer cells', a phrase that may have conveyed a simplified and optimistic interpretation. Patients may not fully appreciate the technical nuances associated with MRD detection,[39,40] instead viewing it as a definitive indicator of treatment success. These findings highlight the need for careful communication when MRD is used as a trial end-point.

Our results align with prior research showing patients and clinicians often value different outcomes in chronic diseases.[8,9] Similar discrepancies have been observed in myeloma, where the study by Mohyuddin et al.[41] showed that patients might not be willing to prioritize PFS over QoL when survival remains unchanged. Another study agreed that, while clinicians acknowledge the importance of QoL for patients, they still regard extending OS as their primary therapeutic goal.[24] Consistent with our own qualitative data, other studies have confirmed that patients prioritize QoL and symptom relief over extended survival, particularly in relapsed or frail populations where treatment tolerability may be more relevant than maximal response.[21,26,42] These preferences become more pronounced in treatment settings where the main anticipated benefit is improved PFS. For instance, studies on maintenance therapies show that patients' preferences often shift towards maintaining QoL when they are presented with clear information on risks and benefits.[22]

Despite recognition of its importance by both clinicians and patients, QoL remains inconsistently captured and rarely used as a primary end-point, often limited to short-term assessments and rarely captures patient experience during disease progression.[6,25,43,44] However, clinical decisions in real-world settings are often guided by their impact on QoL.[45] Our findings underscore that QoL is a point of convergence between patients and clinicians, and its more consistent integration into trial design would enhance relevance to both groups.

The disconnect between stakeholders reinforces longstanding concerns that surrogate end-points, while convenient for regulatory approval, may not reflect what matters most to patients. This recognition has led to the development of core outcome sets (COS), including the myeloma-specific COS that highlights emotional well-being, fatigue and treatment burden—domains echoed in our findings.[17] Similarly, broader initiatives, like COMET and PCORI, emphasize the importance of engaging patients and other stakeholders throughout the research lifecycle, from prioritizing outcomes to designing protocols and interpreting results.[11,19,46,47] Our mixed-methods study contributes to this agenda supporting context-aware, patient-informed research design, consistent with broader calls for ethically grounded, stakeholder-driven oncology research.[17,19,48–50]

Cultural and systemic differences, such as access to healthcare resources and societal values, could influence treatment preferences. Thus, our exclusively Greek recruitment may limit the generalizability of the findings. Second, while sample size was sufficient for exploratory analysis, it was smaller than initially calculated. This increased the margin of error and limited the precision of survey findings, preventing subgroup comparisons. Third, patient understanding of surrogate end-points like PFS and MRD was variable and may have influenced how these outcomes were ranked. This limitation was further compounded by the exclusion of other time-to-event outcomes due to their conceptual complexity for patients. In addition, our study design did not capture treatment trade-offs, giving patients and clinicians the opportunity to weigh benefits such as efficacy against risks such as toxicity, as done by discrete choice experiment methods in other studies, where efficacy was prioritized.[51] Such an approach could have yielded more nuanced insights into patient preference. Another survey has shown that most patients would not accept a PFS benefit if it did not improve OS, especially if it was associated with much toxicity.[41] Finally, interviewer bias may have influenced the qualitative component of the study. Although interviews were conducted using a semi-structured guide, and with the understanding that researcher involvement is an inherent characteristic of qualitative research, the clinical background of the interviewer may have shaped how questions were framed or how responses were interpreted. Participants may also have tailored their answers, consciously or unconsciously, based on perceived expectations from a clinician-researcher.

Our findings underscore the need for clinicians to systematically incorporate patient preferences into care. Yet, shared decision-making in oncology faces challenges: patients may feel overwhelmed at diagnosis, and decisions are often clinician-driven, with participation limited to informed consent. In our interviews, many patients emphasized trust in their physician's expertise, reflecting cultural norms in Greece where older patients often defer to clinicians.[52–54] Improving patient–clinician communication is therefore essential. Clinicians should explain treatment

trade-offs and clarify surrogate end-points. Plain language, concrete examples and balanced discussions of benefits, limitations and uncertainties can facilitate more informed and patient-aligned decisions.

The discordance between clinical research and patient priorities highlights the need for more patient-centred trial designs. While recent studies have shown that most myeloma trials now include patient-reported outcomes (PROs), these data are often heterogeneous, inconsistently analysed and rarely integrated into regulatory decisions or drug labelling.[55] Incorporating PROs as key secondary end-points and consistency of reporting could improve their influence on both approvals and real-world relevance. Use of COS developed with meaningful patient input[17] and broader initiatives[56] like common sense oncology provide a practical framework. More broadly, embedding patient and stakeholder engagement across trial design, conduct and interpretation will help generate evidence that is ethically grounded and aligned with patient values.

CONCLUSION

This mixed-methods study shows that, while patients and clinicians share a priority for QoL in myeloma care, they diverge on the perceived importance of other outcomes like survival metrics. Bridging these gaps through more inclusive clinical decision-making and trial design is essential to ensuring that new therapies deliver outcomes meaningful to patients.

AUTHOR CONTRIBUTIONS

Maria Mainou, Apostolos Tsapas and Thomas Karagiannis conceived and designed the study; Panagiotis Malandrakis, Evdoxia Hatjiharissi, Sofia Chissan, Evdokia Mandala, Efthymia Vlachaki, Maria Papaioannou, Evangelos Terpos and Maria Mainou contributed to data collection and coordinated responsibilities; Maria Mainou, Aris Liakos, Eirini Pagkalidou, Oliver Van Oekelen and Ghulam Rehman Mohyuddin analysed and interpreted data, Maria Mainou and Thomas Karagiannis drafted the manuscript, which was critically revised and approved by all authors.

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The authors have nothing to report.

CONFLICT OF INTEREST STATEMENT

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DATA AVAILABILITY STATEMENT

Data are available upon request from authors.

ETHICS APPROVAL STATEMENT

The study was approved by the Bioethics Committee of the Department of Medicine, Aristotle University of Thessaloniki (protocol No 6.247).

PATIENT CONSENT STATEMENT

Informed consent was obtained from all participants prior to inclusion in the study.

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SUPPORTING INFORMATION

Additional supporting information can be found online in the Supporting Information section at the end of this article.

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