HealthTree Foundation

PA-174. Patient Perceptions of MRD Negativity as a Treatment Outcome and Regulatory Endpoint in Multiple Myeloma

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BACKGROUND

Minimal residual disease (MRD) negativity has emerged as a key biomarker in multiple myeloma (MM), linked to deeper responses and improved progression-free survival. **In April 2024, the FDA's Oncologic Drugs Advisory Committee (ODAC) endorsed MRD negativity as an early endpoint for accelerated treatment approval.** While this regulatory change may shorten drug approval timelines, patient perspectives on MRD as a treatment goal and trial endpoint remain underexplored. **This study is crucial for aligning clinical trial design and regulatory strategies with patient values and preferences regarding MRD as a treatment goal.**

METHODOLOGY

This cross-sectional online survey was conducted among **MM patients (≥18 years) enrolled in the HealthTree Cure Hub®.**

- The IRB-approved survey included 13 MRD-related and 6 demographic questions.
- Patients were asked about their MRD testing experience, treatment perceptions, and regulatory awareness.
- The survey compared patient insights on MRD-based accelerated approval before and after the ODAC vote concerning MRD.

Total Participants: 192 Patients, 85% with MM diagnosis (n=163). All responses were de-identified and analyzed descriptively.

CONCLUSION

Patients with MM strongly view MRD negativity, particularly when sustained, as a meaningful indicator of efficacy. A substantial proportion reported willingness to modify treatment strategies to achieve MRD negativity, and educational content significantly increased support for MRD-based regulatory pathways. These findings highlight the importance of incorporating patient-reported preferences into clinical trial endpoints and regulatory decision-making, reinforcing MRD's relevance not only as a biomarker but as a patient-valued measure of treatment success.

ACKNOWLEDGEMENT

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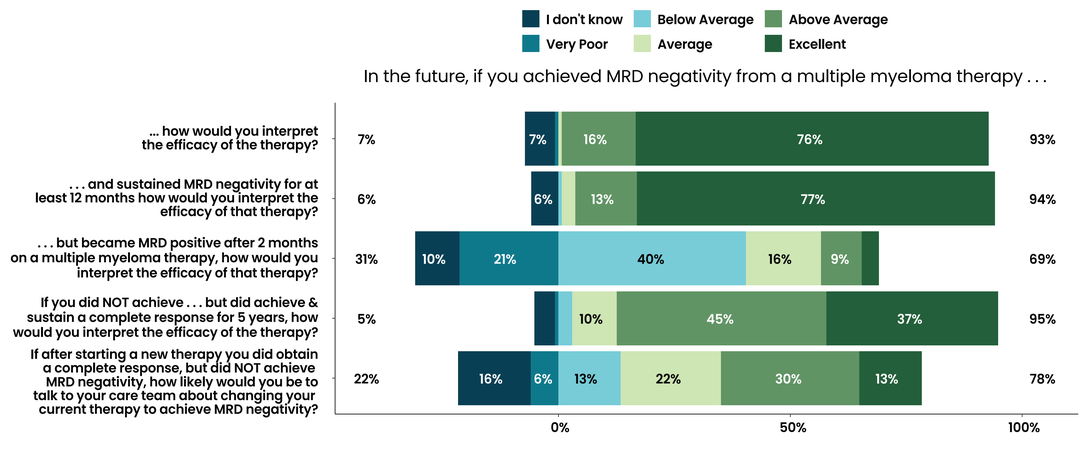
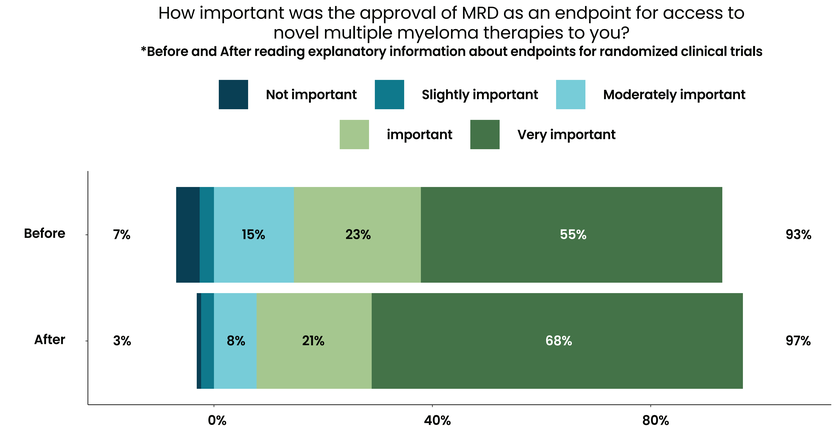
RESULTS

MRD NEGATIVE QUESTIONS



MRD ENDPOINT



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