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PROMising Developments: Trends of Patient-Reported Outcomes in Waldenström Macroglobulinemia Clinical Trials

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INTRODUCTION

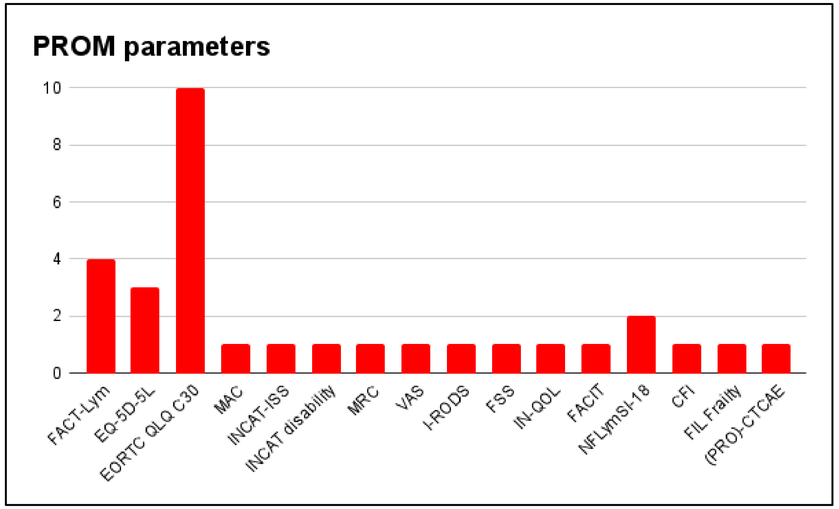
- WM is a rare chronic B-cell disorder with 1000 to 1500 cases annually.
- Although incurable, novel therapies exist.
- Patient Reported Outcome Measures (PROMs) are important measures to assess patient experience compared to the traditional clinical outcomes.
- We aimed to evaluate trends in usage of PROMs for patients with WM as a measure of endpoints across global clinical trial databases.

METHODS

- We searched the clinicaltrials.gov database for phase II, III, and IV trials involving WM patients from 1998 to 2024.
- A comprehensive search was conducted from clinical trial databases from the US, European Union, Australia/New-Zealand, and WHO International Trial Registry.
- The data extraction included primary endpoints, secondary endpoints, PROMs, PRO parameters, and duration of clinical trial. Descriptive statistics were used to summarize the data.

RESULTS

- A total of n=184 trials were included: Phase II (n=161; 87.5%), Phase III (n=18; 9.7%) and Phase IV (n=5; 2.7%).
- 70% (n=125) of studies were completed and 27% (n=50) are ongoing.
- Only 13.6% (n=25) of trials included PROMs. 32% (n=8) completed and 68% (n=17) ongoing studies used PROMs as one of their secondary outcome measures. None of the studies included PROMs as their primary outcome measures.
- From 1998-2005, zero studies reported any PROMs, 32% (n=8) trials were from 2006 to 2016 and 68% (n=17) had inclusion of PROMs as part of secondary outcome measures. Sixteen different parameters were utilized to measure PROMs. EORTC QLQ C30 was most frequently utilized in 40% (n=10) of the studies.



CONCLUSIONS

- While PROMs are increasingly being incorporated as secondary outcome measures in WM clinical trials, their overall usage remains limited.
- This can possibly be attributed to the significant heterogeneity in measuring PROMs by sixteen different scales as per our reporting.
- There is a significant unmet need for standardized and validated tools for PROMs in WM which can be used for better understanding of patient experience on these novel therapies.

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