New approaches to active monitoring: Patient-reported outcomes and wearables utilisation in Waldenstrom macroglobulinemia.

Kim Summers, Orlando Agrippa, Shirley D'Sa; Sanius Health, London, United Kingdom; University College London Hospitals NHS Foundation Trust, London, United Kingdom

Background: Waldenstrom macroglobulinemia (WM) is a rare haematological malignancy that has seen an emergence of novel targeted therapies. With a range of clinical features impacting quality of life (QoL), there is a need for disease-specific patient-reported outcomes (PRO), outside of existing measures with limited validity in WM. The aim of this work was to enrich understanding of the patient experience and identify potential WM-specific metrics, through wearable-captured physiological metrics and electronic-PROs (ePROs). **Methods:** Informed consent was provided by 79 patients with WM for the analysis of data from an FDA-cleared wearable smartwatch capturing activity, sleep, and heart rate, and a mobile app for patients to input daily ePROs. These ePROs included treatments taken, EQ-5D-5L, and disease symptoms (fatigue, weakness, numbness/tingling, breathlessness, difficulty concentrating/confusion, vision, rash, night sweats, dizziness/light-headedness, constipation, loss of appetite, diarrhoea, and vomiting). Data was integrated within a digital platform, and anonymised extracts analysed as a cohort and by treatment. **Results:** Patients had a mean±SD (range) age of 65±10 (43-88) years. 53% were female. As of the most recent self-reported treatment entry, 22% (17/79) reported Bruton tyrosine kinase inhibitor (BTKi) use. Completion rates over a 142-day snapshot for daily PROs was 60%, and 85% across wearable activity and sleep metrics (devices synced=58/79). A cohort mean EQ-5D-5L Score of 0.760±0.181 and Health State (0-100) of 74±18 was reported. The highest symptom severity scores (1-5) were 'fatigue' (1.9/5), 'weakness' (1.7/5), and 'numbness/tingling' (1.5/5), all other symptoms reporting a mean score of 1.4 or less. Increasing activity levels significantly correlated with decreasing symptom severity and increasing QoL scores ($p<0.05$). Lower symptom severity also correlated with higher total sleep durations, contrasting higher 'difficulty concentrating/confusion', 'dizziness/light-headedness', and 'usual activity impairment' scores with increasing levels of deep sleep. Breathing disturbance intensities (36.8 vs. 17.6, p=0.008) and nightly wakeup counts (1.9 vs. 1.6, p=0.038), tracked by wearables, were significantly higher in patients reporting BTKi treatment compared to the remaining cohort. **Conclusions:** Our data support the enrichment of knowledge around baseline physiological and QoL metrics for patients with WM, demonstrating the feasibility of a digital ecosystem of wearable-captured and ePRO metrics at a high data completeness level. Further work will ensure the generation of more population-representative outputs, through the expansion of the patient cohort, and a greater depth of insight into the impact of health events, disease complications, and therapeutic intervention on these remotely-tracked metrics. Research Sponsor: None.