W18: WhiMSICAL (Waldenström's Macroglobulinemia Study Involving CArt-wheeL): A Global WM Registry for the Patient's Voice

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Introduction

Patient-derived data can increase breadth of knowledge in rare cancers like Waldenström's Macroglobulinemia (WM). Patient-reported outcomes (PROs) are valuable, with integration of electronic symptom reporting in cancer care shown to improve health outcomes including survival.[1]

This study utilized www.cart-wheel.org, an ethically-approved online rare cancer database for patient-derived data, and harnessed the digital connectedness of WM patients globally, to develop a continuously expanding dataset and platform for hypothesis generation around WM.

Methods

An ethics committee-approved WM-specific extension to the www.cart-wheel.org questionnaire, developed by clinician and patient investigators, was released June 2016. Participants complete consent online and enter their symptom, pathology and treatment data. Recruitment strategies driven by the International Waldenström's Macroglobulinemia Foundation (IWMF) investigators utilized multiple social media platforms. A pilot validation study was performed comparing patient- and data-manager-entered data of participants treated at Australian and New Zealand Lymphoma and Related Diseases Registry (LaRDR) sites to evaluate data accuracy.

Results

303 participants from 14 countries have been recruited, predominantly from USA (45%) and Australia (23%), with median age 68 years (range 43-86) and male predominance (61%). Median age at diagnosis was 60 (range 41-83), median IgM 2750mg/dL (IQR 1530-3907mg/dL, n=101) and median hemoglobin 11.1g/dL (IQR 9.4-12.7g/dL, n=106). Of the 224 (74%) patients providing symptoms at diagnosis, fatigue was most common (45%), correlating with median hemoglobin 10.1g/dL (IQR8.8-12.1g/dL, n=48) compared to 12.3g/dL (IQR 10.9-13.4g/dL, n=55) in those without fatigue ($p<0.0001$). Using the Impact of Event Scale for symptoms of post-traumatic stress disorder (PTSD) resulting from a cancer diagnosis, at median 48 months post-diagnosis, the mean score among 254 patients was 6.1 (no stress=0, maximal stress=24), with 30/254 (11.8%) scoring $\geq$13 (PPV 94% for PTSD).[2] Thirty-seven different first-line therapeutic combinations were documented by 180 participants (Figure 1). From diagnosis, median time to first treatment for USA patients was 48 days (IQR 12-391, n=84) vs. Rest of World (ROW) 114 days (IQR 23.8-671.8, n=100), ($p=0.056$). Of the 189 therapies listed by 87 USA patients, 55/189 (29%) were government funded and 8/189 (4%) via clinical trial participation, whereas of the 190 therapies of ROW patients (n=91), 112/190 (59%) were government funded and 24/190 (13%) through clinical trials ($p<0.001$ and $p=0.003$, respectively). Data for 21 patients were available for validation with LaRDR, with completion rates of 74% (78/105 data-fields) and 90% (95/105) by patients and LaRDR, respectively. Where entered by both, diagnosis date, first treatment date and agents had good concordance (78-86%, n=21,15,18 respectively). Diagnosis IgM and hemoglobin had 63% (n=8) and 90% (n=10) concordance, respectively.

Conclusion

The WhiMSICAL study is a robust global patient-derived data platform, providing insight into patient symptoms, including WM-related stress responses, and diversity of therapies. Further recruitment (Project 1000) and encouragement of more complete and continuous data entry, inclusion of the EORTC-QLQ-C30 questionnaire, data linkages with laboratory information systems, and data validation from a larger cohort will increase the utility of WhiMSICAL. As an expanding and increasingly reliable body of data, WhiMSICAL has the potential to map real-world PROs, break down clinician-patient barriers and provide a scientific and ethically-approved portal for patients' voices globally.

References

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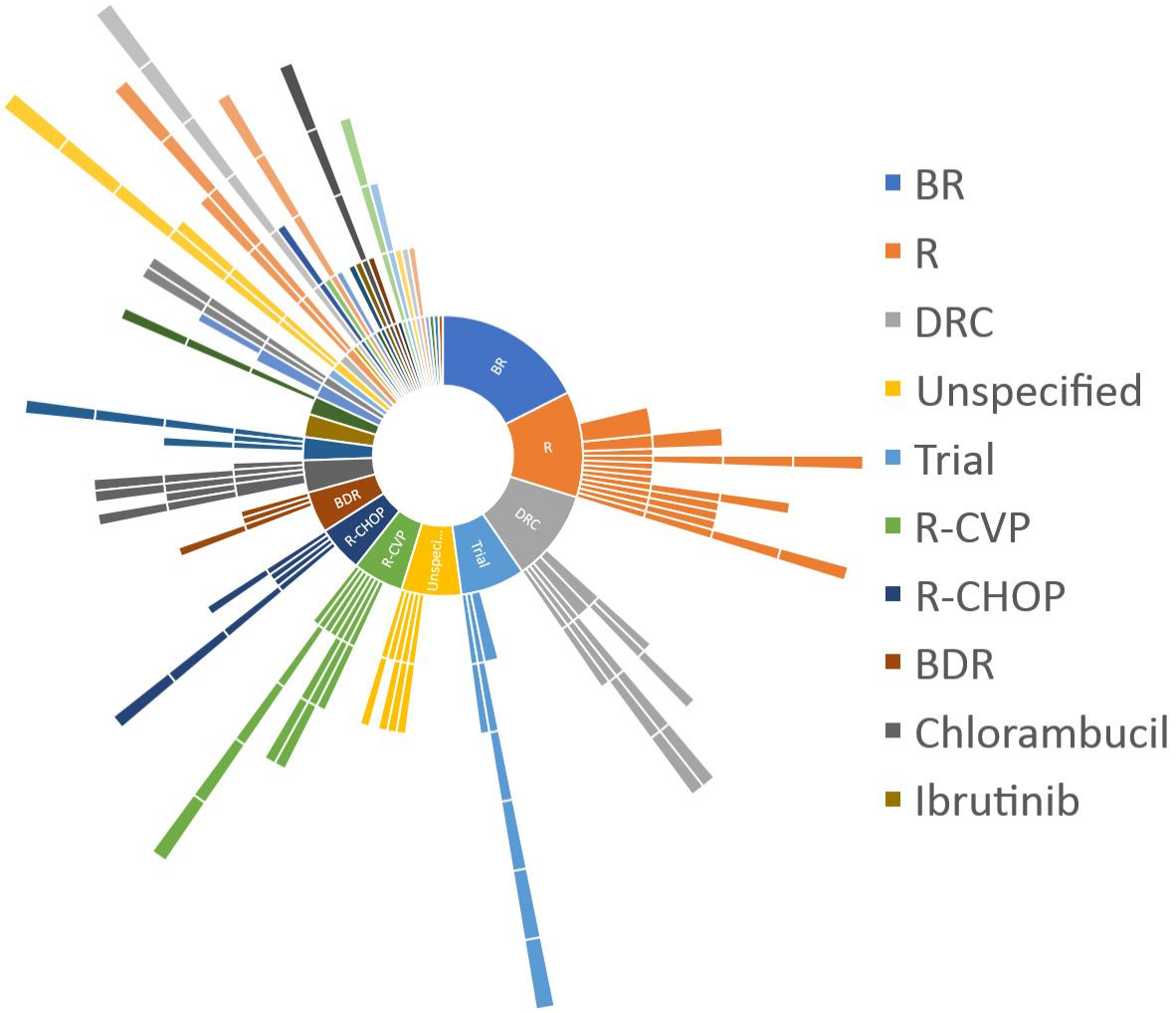

Figure 1. Sunburst chart demonstrating the variation in therapies entered by 180 WhiMSICAL participants. Each color represents a group of patients with the same first line of therapy, shown in the central ring. The subsequent rings represent further lines of therapy of the patient group, with each slice representing a different type of therapy. The ten most common first-line therapies are listed in the legend. All 33 recipients of BR as first-line therapy did not have further treatment at a median 28 months.

BR – Bendamustine Rituximab, R – Rituximab, DRC – Dexamethasone Rituximab Cyclophosphamide, R-CVP – Rituximab-Cyclophosphamide Vincristine Prednisone, R-CHOP – Rituximab-Cyclophosphamide Doxorubicin Vincristine Prednisone, BDR – Bortezomib Dexamethasone Rituximab