Current treatment approaches to Waldenström macroglobulinaemia in the United Kingdom

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Waldenström macroglobulinaemia (WM) has an age standardized incidence rate of 0.55 per 100 000 per year in the UK. Once diagnosed, patients are typically managed in district hospitals by a haematologist within the National Health Service (NHS), the world's largest publicly funded health service.

The National Institute for Health and Care Excellence (NICE) is a public body which appraises the use of health technologies within the NHS. Once approved, the NHS is legally obliged to provide funding for the drug. As yet, no treatment has been NICE-assessed for WM, owing to the orphan status of the disease.

New guidelines for the diagnosis and management of WM in the UK were published in 2014 in the British Journal of Haematology. Patients with symptomatic WM receive a rituximab-containing regimen, the choice taking account of performance status, clinical features, co-morbidities and potential candidacy for autologous stem cell transplantation (ASCT). At relapse, similar options are available with additional access to Bortezomib. Patients are entered in to trials whenever possible. ASCT is a feasible option for relapsed WM in younger, fitter patients with aggressive disease but is not funded in 1st response.

The Cancer Drugs Fund (CDF) provides an additional £200m per year to enable patients to access drugs not routinely funded by the NHS. Inclusion into the CDF is assessed on the strength and quality of the evidence for clinical effectiveness and expected delivery of measurable outcomes e.g. improved survival rate and quality of life. WM-related inclusions to the CDF include Bendamustine + Rituximab at 1st line; Bendamustine at relapse if unable to receive CHOP-R, FCR or high dose-therapy; Bortezomib at relapse following prior alkylating agents and purine analogues.

Three clinical trials are open for patients with WM in the UK. The R2W trial is an upfront trial of BortCR vs FCR in WM; preliminary results will be presented at this meeting. The Gilead 0125 trial evaluates BR +/- Idelalisib in at relapse, and a phase II study of Ixazomib for relapsed and/or refractory indolent lymphoma (for the lead-in dose-finding phase) after at least 1 prior therapy. Both trials require clinically or radiologically measurable disease.

Two UK-wide surveys have been carried out by the patient-doctor organisation, WMUK: a survey of UK clinicians who treat WM patients (43 respondents) and WM sufferers (120 respondents). The results of these surveys will be presented at the meeting to provide an insight into the WM landscape in the UK.