CLINICAL FEATURES AND SURVIVAL OUTCOME OF YOUNG PATIENTS WITH WALDENSTROM MACROGLOBULINEMIA

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Background: Data in patients with Waldenstrom Macroglobulinemia (WM) who are 50 years or younger at diagnosis are sparse. We present outcomes of a large cohort of young WM patients who were evaluated at Mayo Clinic, Rochester, MN, USA between January, 2000 and December, 2013. Methods: Sixty-nine (10.7%) of 640 consecutive patients were ≤ 50 years at diagnosis. Overall survival (OS) was calculated with Kaplan-Meier method and years of life lost were computed based on expected mortality for the age group. Results: Median age at diagnosis in this cohort was 45 years (range: 31-50 years). Males constituted 65% of patients. Nine (14%) patients had familial WM. Waldenstrom Macroglobulinemia was at smoldering stage in approximately 21% of the cases at diagnosis. The most common clinical symptoms at presentation were constitutional (43%), hyperviscosity-related (23%) and paresthesia (17%). Splenomegaly and lymphadenopathy were present in 14% and 29% of the patients, respectively. At diagnosis, median hemoglobin was 10 g/dL (range: 5.4-14.6), platelet count 220 x10^9/L (80-501), β2 Microglobulin 2.7 mcg/ml (1.3-7.8) and IgM 4501 mg/dL (68-14400). Stem cells were successfully harvested from 10 of the 12 attempted cases. Only 5 (8%) patients underwent autologous stem cell transplantation (ASCT), one of whom had ASCT post transformation to a higher grade lymphoma. In the non-transformed patients, ASCT was used as salvage therapy after a median of 6.5 regimens (range: 4-10). Among these patients, median FU was 4.6 years from ASCT and median OS was not reached. Of 67 patients treated, the most common initial management involved the use of rituximab monotherapy (24%), dexamethasone, rituximab, cyclophosphamide (DRC) regimen (15%), nucleoside analogs (NA)-based (22%), chlorambucil-based (10%) and other therapies (28%). Sixty-five patients (97%) received rituximab during their disease course. Overall, 6/25 (24%) who received NA developed therapy related myelodysplastic syndrome (t-MDS) or transformed lymphoma compared to 1/42 (2%) who received non NA based therapy (p=0.009). These events occurred at a median of 7.6 years from NA therapy. Median follow up (FU) was 8 years from diagnosis and 7.8 years from initial therapy. Eight-year OS was 84% from frontline therapy (median 14.8 years). Of all deaths (n=18), only 1 was non WM related. Over 30 years of FU, the average years of life lost were 10.8 years. Conclusions: Despite its indolent course, WM remains a major cause of morbidity and mortality in young patients with this cancer. NA-based therapy is best avoided in this population due to high risk of developing t-MDS or transformation. Although effective, ASCT appears to be an underutilized approach (This abstract has been selected for presentation at ASCO Annual Meeting June 2014).