

High-dose melphalan and autologous stem cell transplantation increases survival

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A team of researchers led by Boston University School of Medicine (BUSM), has found treatment of selected immunoglobulin light chain (AL) amyloidosis patients with high-dose melphalan and autologous stem cell transplantation (HDM/SCT) resulted in a high organ response rate and increased overall survival (OS), even for those patients who did not achieve a hematologic complete response (CR). These findings appear in the current issue of *Blood*.

AL amyloidosis is the most common form of systemic amyloidosis, with an incidence of five to 12 persons per million per year. In AL amyloidosis, clonal bone marrow [plasma cells](#) produce monoclonal light chains that misfold and deposit in tissues and organs as amyloid [fibrils](#), resulting in progressive system and [organ failure](#) and ultimately in death.

Untreated patients with this disease have a dismal outcome, with a median survival of 10-14 months from diagnosis. Moreover, fewer than five percent of patients survived for 10 years before the introduction of HDM/SCT.

The BUSM researchers analyzed a series of 421 patients treated with HDM/SCT and compared outcomes for patients with and without CR. Treatment related mortality was 11.4 percent overall (5.6 percent in the last 5 years). The CR rate was 34 percent and the median event-free survival (EFS) and OS were 2.6 and 6.3 years, respectively.

Eighty-one patients died within the first year after HDM/SCT and were

not evaluable for hematologic and organ response. Of 340 evaluable patients, 43 percent achieved CR and 78 percent of them experienced an organ response. For CR patients, median EFS and OS were 8.3 and 13.2 years, respectively. Among the 195 patients who did not obtain CR, 52 percent achieved an organ response, and their median EFS and OS were 2 and 5.9 years, respectively.

"This study provides the longest outcome data on AL patients treated with HDM/SCT, including OS, EFS, and long-term mortality," explained senior author Martha Skinner, MD, former director of BUSM's Amyloid Treatment and Research Program as well as a professor of medicine at BUSM. "Our results demonstrate that, with careful patient selection and experienced management, low rates of treatment related mortality can be achieved.

According to the authors, despite the importance of attaining CR, achievement of partial response often results in a long period of stable or improved diseases. "We pioneered HDM/SCT treatment for AL [amyloidosis](#) in 1994. This long term review shows durable hematologic and organ responses for most [patients](#) with an overall survival greatly exceeding what it was prior to HDM/SCT treatment," she added.

Provided by Boston University Medical Center

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