Light-chain amyloidosis patients treated with high-dose chemo and stem cell transplantation have long-term survival

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Patients with Light-chain (AL) amyloidosis who are treated with high-dose chemotherapy (melphalan) and autologous (one's own) stem cell transplantation (HDM/SCT) have the greatest success for long-term survival.

These findings, which appear as a "Letter" in the journal *Blood*, (*Journal of the American Society of Hematology*), report on the largest number of patients in the world receiving high dose chemotherapy and stem cell transplantation for this rare disease.

Light chain amyloidosis (AL) is the most common form of systemic amyloidosis in the U.S. The disease is caused when a person's antibody producing plasma cells produce abnormal light chains (components of antibodies). These light chains misfold and form amyloid deposits (a starch like protein) that accumulate in the liver, kidneys, spleen, or other tissues which can cause serious damage to these organs.

HDM/SCT was considered an innovative treatment approach when it was first developed at Boston University School of Medicine (BUSM) and Boston Medical Center (BMC) in 1994. Since that time, 629 patients with AL amyloidosis have undergone this treatment at BMC.

Hematologic responses (the first indicator that treatment is beginning to work) were assessed in 543 patients at 6-12 months following HDM/SCT treatment. Forty percent achieved a hematologic complete response (CR) post stem cell transplantation. Hematologic relapse occurred in 18.2 percent at a median of 3.97 years post treatment. Long-term survival, up to 20 years, was achieved in nearly one-third of patients.

"While survival is strongly dependent upon achieving hematologic complete response (CR), the survival of patients who did not achieve a CR and of those who relapsed after CR is notable, suggesting a benefit of aggressive treatment," explained corresponding and lead author Vaishali Sanchorawala, MD, professor of medicine and associate director of the Amyloidosis Center at BUSM.

"Strategies to better understand which patients may benefit the most from this treatment and reducing treatment-related mortality, as well as using combination therapies with novel agents to increase the CR rate, will likely improve outcomes in the future for patients who just a few years ago were considered to have a rapidly fatal diagnosis," added Sanchorawala, MD, director of the stem cell transplant program in the section of hematology and oncology at BMC.

Source:
Boston University Medical Center
