First-of-its-kind study reveals predictive factors for outcomes of advanced stage AL amyloidosis

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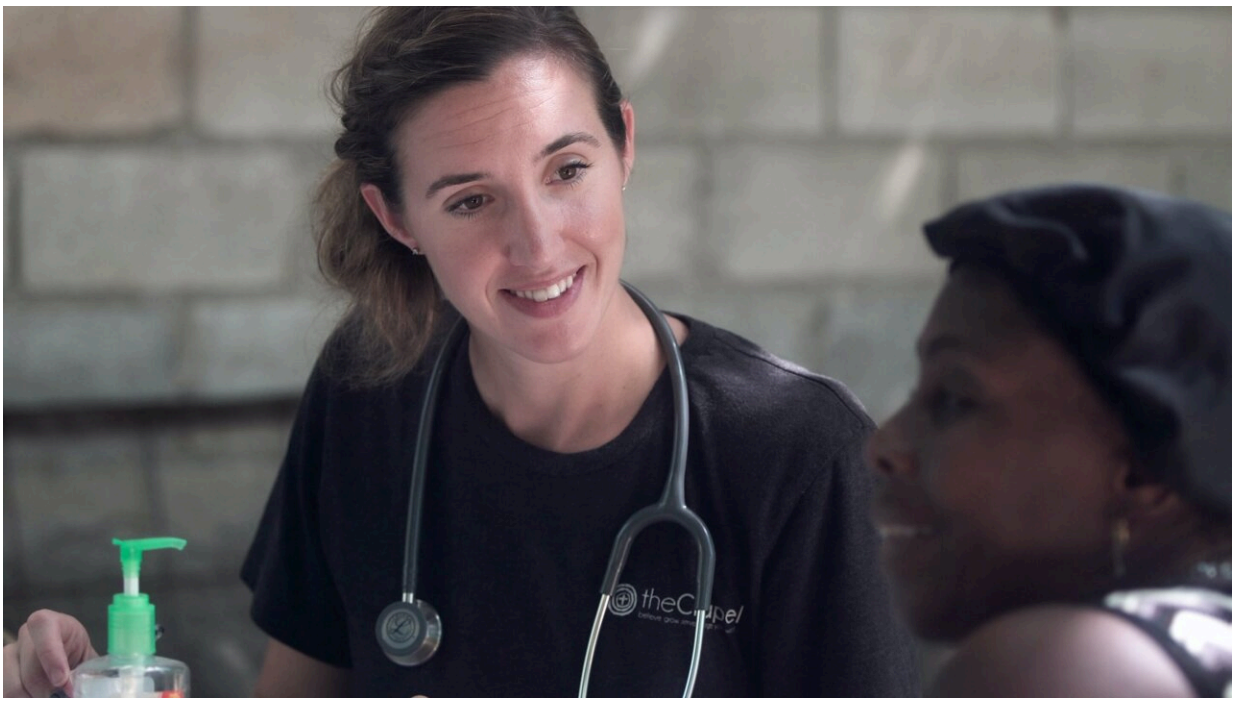

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Early improvements in cardiac and hematologic parameters may predict better survival outcomes for patients being treated for stage IIIb AL amyloidosis, a deadly disease with a median survival of four to six months caused by abnormal protein buildup, according to research

published in *Blood Advances*.

Amyloidosis occurs when normal proteins in the body misfold and form amyloid deposits in vital organs and tissues, which can lead to organ dysfunction, failure, and death. The prognosis for patients with advanced cardiac amyloidosis is extremely poor, but clinicians have little data to turn to to guide treatment regimens. This study reveals how early responses to treatment may predict survival outcomes.

"While AL amyloidosis is a rare condition, symptoms can mimic those of many other more common diseases, like diabetes, hypertension, autoimmune disorders, and connective tissue diseases, making it difficult for clinicians to diagnose it early," explained Vaishali Sanchorawala, MD, Director of the Amyloidosis Center at Boston University Chobanian & Avedisian School of Medicine and Boston Medical Center, and senior author of the study.

"By understanding the significance of early treatment responses at one and three months after beginning treatment, we can better guide our approaches to therapy and improve patient outcomes."

Researchers reviewed data from 142 patients with newly diagnosed stage IIIb AL amyloidosis with advanced cardiac involvement from 2007 until 2022. Patients were included after 2007 when bortezomib-based treatment regimens became available for the treatment of AL amyloidosis, after their success in treating myeloma.

After 60 months of follow-up, patients exhibited a median overall survival of nine months. Several baseline factors were predictors of worse survival including the onset of symptoms to diagnosis of greater than six months, bone marrow plasma cell count of more than 10%, higher troponin I levels (a marker of heart damage), and walking a distance of less than 200 meters in six minutes. Treatment with

bortezomib and daratumumab-based regimens led to improved survival.

Patients who experienced early hematologic (within one month) and cardiac (within three months) response had significantly longer overall survival outcomes. Patients achieving a very good partial hematologic response at one month following treatment had a prolonged survival of 47 months.

"Stage IIIb AL amyloidosis with advanced cardiac involvement does not have to be a death sentence," emphasized Dr. Sanchorawala. "We see patients who live four to five years after being diagnosed, and studies like this one helps us understand what factors may indicate a better prognosis. These findings help us to appropriately tailor our treatment regimens to achieve optimal outcomes."

Dr. Sanchorawala reiterated that to advance treatment and improve overall survival for patients living with this condition, we must prioritize their involvement in clinical research.

"Our ability to advance science and improve patient outcomes depends on rare disease-focused clinical trials," she said.

The American Society of Hematology is currently in the process of developing evidence-based clinical practice guidelines on the diagnosis of amyloidosis (both types, AL and ATTR) that reflect the latest evidence about the disease to help ensure the medical community can better diagnose and treat amyloidosis and people with amyloidosis can make the best decisions for their care. Learn more about all ASH Clinical Practice Guidelines.

Provided by American Society of Hematology