

Powerful myeloma treatment regimen shows promise for AL amyloidosis

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Two studies published today in *Blood*, the Journal of the American Society of Hematology (ASH), demonstrate preliminary success of an effective multiple myeloma (MM) regimen in patients with AL amyloidosis, a rare and devastating blood disease that results in deposition of damaging abnormal protein in critical organs of the body, including the kidneys, heart, liver, and intestines, and shares some characteristics with MM.

The combination of bortezomib, cyclophosphamide, and dexamethasone (CyBorD, or CVD) is a recently discovered but already widely used MM treatment regimen that produces rapid and profound responses. Because of its success in MM, recent investigations have explored the use of this regimen as a potential therapy for AL amyloidosis, since those with the disease have few treatment options. Currently, the most effective treatment for AL amyloidosis is high-dose melphalan and autologous stem cell transplant; however, most patients are ineligible for this aggressive and difficult to tolerate approach due to organ damage common in patients with the disease.

Fortunately for AL amyloidosis patients, there is hope for a better treatment. Each of the studies summarized below conclude that treatment with the novel CyBorD/CVD regimen can lead to complete hematologic response and may allow AL amyloidosis patients to become eligible for stem cell transplants and live longer, higher quality lives.

STUDY 1: Cyclophosphamide-Bortezomib-Dexamethasone



(CYBORD) Produces Rapid and Complete Hematological Response In Patients with AL Amyloidosis

First Author: Joseph Mikhael, MD, Mayo Clinic, Scottsdale, AZ

Background

Retrospective analysis of 17 patients with confirmed and symptomatic AL amyloidosis treated with CyBorD prior to autologous stem cell transplant (ASCT), as an alternative to high-dose therapy for those deemed ineligible, and as salvage for those with prior therapy Results

94% hematologic response rate observed in patients
Three patients originally not eligible for ASCT became eligible after treatment
Conclusion

CyBorD produces rapid and complete hematologic responses in the majority of patients with AL amyloidosis regardless of previous treatment or ASCT candidacy and is well tolerated with few side effects. In addition, results demonstrate that the regimen may also allow ASCT non-eligible patients to become eligible after treatment.

STUDY 2: Cyclophosphamide, Bortezomib and Dexamethasone Therapy in AL Amyloidosis is Associated with High Clonal Response Rates and Prolonged ProgressionFree Survival First Author: Christopher Venner, MD, National Amyloidosis Centre, University College London Medical School, London, United Kingdom

Background

Retrospective analysis of 43 patients with AL amyloidosis treated with CVD upfront or at relapse



Results

81.4% hematologic response rate observed in patients Conclusion

CVD is a highly effective combination for the treatment of AL <u>amyloidosis</u> with high hematologic responses. In addition, rapid improvement in organ function was observed and functional organ improvement may potentially allow deferred <u>stem cell transplant</u> in previously ineligible patients due to increased organ response.

More information: <u>bloodjournal.hematologylibrary ...</u> <u>blood-2011-11-390930</u> <u>bloodjournal.hematologylibrary ... blood-2011-10-388462</u>

Provided by American Society of Hematology

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