Telomerase targeting drug demonstrates benefit in myelofibrosis treatment

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Imetelstat, a novel drug that targets telomerase, has demonstrated potential value in treating patients with myelofibrosis, according to the results of a study published today in the *New England Journal of Medicine*.

"We observed that Imetelstat was active and induced morphologic and molecular remissions in some patients with myelofibrosis," says Ayalew Tefferi, M.D., a hematologist at Mayo Clinic and lead author of the study. "We also observed that Imtelstat demonstrated selective anti-clonal activity, inhibiting the growth of cancer cells, which we had not previously documented with other drugs."

Myelofibrosis is a chronic myeloid cancer in which bone marrow cells that produce blood cells develop and function abnormally. The result is the formation of scar tissue in the bone marrow (fibrosis), severe anemia that often requires transfusion, weakness, fatigue, and an enlarged spleen and liver. Patients with myelofibrosis harbor one of several genetic mutations in their blood stem cells,including JAK2, MPL, CALR, ASXL1 and spliceosome pathway mutations."Typically, myelofibrosis is characterized by marrow scarring, and, although patients may derive symptomatic relief from other treatments, such as ruxolitinib, they usually do not revert back to normal bone marrow," Dr. Tefferi says. "Some patients treated with Imetelstat have reverted back to normal bone marrow." Imetelstat works by inhibiting telomerase activity in tumor cells, which leads to cell death.

Researchers studied 33 patients with a median age of 67 years. Nearly half of those patients had received prior treatment with a JAK inhibitor. Researchers noted a complete or partial remission in seven patients. The median duration of complete response was 18 months. Bone marrow fibrosis was reversed in all four patients who experienced a complete response, and a molecular response was documented in three of the four patients.

"We noted a difference in response rates, especially in complete remission rates, in patients with and without certain specific gene mutations, such as ASXL1, SF3B1 and U2AF1, says Dr. Tefferi. "This underscores the need for laboratory correlative studies in future clinical trials."

Treatment-related adverse events included thrombocytopenia, neutropenia, anemia and elevation in liver function enzyme levels.

Provided by Mayo Clinic

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