Ask ACCC's Community Resource Centers: *Myelofibrosis*

Myeloproliferative neoplasms (MPNs) are a cluster of chronic myleoproliferative diseases that are technically classified as malignancies wherein the bone marrow produces cells that are in some way abnormal. Myelofibrosis can arise on its own (as in primary myelofibrosis, PMF), or as a progression of polycythemia vera (post-PV-MF) or essential thrombocythemia (post-ET-MF). According to Timothy Tyler, PharmD, FCSHP, director of Pharmacy, Lab and Oncology Supportive Care Services at Desert Regional Medical Center, Palm Springs, Calif., MF is not a hematologic cancer in the classic sense, and certainly not a big attention-getter, like an acute leukemia or even a chronic leukemia, but MF does generate a great deal of symptomatology and is best managed by a hematologist with a comprehensive supportive care team.

MF is a chronic blood cancer in which excessive scar tissue forms in the bone marrow and impairs its ability to produce normal blood cells. It is thought to be caused by abnormal blood stem cells in the bone marrow. The abnormal stem cells produce more mature cells that grow quickly and take over the bone marrow, causing fibrosis (scar tissue formation) that results in chronic inflammation. As a byproduct of this scarring, the bone marrow loses the ability to generate normal blood cells, and other organs, such as the spleen, may become the primary producers of blood cells. MF is generally a disease of the elderly, with average patients typically in their 60s and 70s. Younger patients can develop MF—especially if there has been environmental exposure—but, overall, MF is a disease of old age.

Limited Options

For many years, MPNs were a discouraging cauldron of indolent disease; clinicians had very few options with regard to active therapy and certainly nothing that was in any sense a "targeted" therapy. Drugs that might help to manage MF symptoms were few and far between—with no clear coverage guidelines outside of the compendia. In essence, providers and payers tried their best to manage this group of very fatigued (feeling tired, weak, or short of breath are among the symptoms of MF) patients. While transfusions of blood products can be indicated for this patient population, after taking into account indications and even cautions from blood provider agencies, clinicians often used transfusions as agents of last resort.

The manifestations of PMF, post-PV-MF, and post-ET-MF are virtually identical and treatment is generally the same for all three. Until recently, supportive care focused on fatigue management and use of hydroxyurea (Hydrea®), a drug that has been around since the 1960s. While some clinicians attempted to treat MF symptoms with erythropoietin stimulating agents (ESAs), the lack of a medical indication often resulted in reimbursement challenges. There has been some success in a limited pool of patients who are eligible and desire transplant, but as the vast majority of patients are elderly with a loss of functioning protoplasm, this therapy is not widely done. Simply put, for many years the challenges caused by MF and its hematologic cousins—primarily fatigue and a lack of energy—did not have a targeted therapy and so symptom management was key.

Our Supportive Care Model

During this period of limited treatment and management options for patients with MF, The Comprehensive Cancer Center at the Desert Regional Medical Center looked to its strong oncology supportive care services to augment the paucity of drug therapy options. For example, our psychologist is available by appointment to counsel MF patients one-on-one about energy conservation, pursed lip breathing in patients with obstructive pulmonary complications, and other strategies to manage general fatigue and lack of energy. Dietitian consults help ensure our MF patients receive education about proper nutrition; social workers can intervene regarding living situations that no longer work for elderly MF patients—many of whom are used to being self-sufficient. Bottom line: our oncology supportive care team plays an active role in managing our MF patients as compared with their role with our more "traditional" solid tumor patients.

New Hope

With the introduction of JAK inhibitors, improvement in MPN disease-related symptoms has emerged as a realistic expectation of therapy and an integral measure of clinical efficacy.[1] At the end of 2011, the U.S. Food and Drug Administration (FDA) approved ruxolitinib (Jakafi®) for the treatment of myelofibrosis; late last year, the FDA expanded this indication to include polycythemia vera. This targeted agent has breathed new hope in the treatment of these diseases, demonstrating significant reductions in symptom burden, with consequent improvements in QoL (quality of life) measures.[1]

Positive data from stage I of an adaptive two-stage Phase II trial of PRM-151, a novel anti-fibrotic immunotherapy, demonstrated reduction of bone marrow fibrosis by at least one grade observed in 42 percent of patients, which was associated in most patients with improvements in anemia and/or thrombocytopenia and, in some patients, by transfusion independence lasting at least 24 weeks.[2] These study results were presented in an oral presentation by principal investigator Srdan Verstovsek, MD, PhD, at the American Society of Hematology (ASH) 2014 Annual Meeting, Dec. 8, 2014.

In the end, while these new and emerging therapies begin to increase our treatment options for patients with MF, supportive care—an experienced clinician talking one-on-one with these often elderly patients and ensuring they receive comprehensive symptom management education—remains key. OI

References

1. Mesa RA, Scherber RM, Geyer HL. Reducing symptom burden in patients with myeloproliferative neoplasms in the era of JAK inhibitors. *Leuk Lymphoma*. 2015 Feb 3:1-39. [Epub ahead of print]

2. Stein BL, Verstovsek S. Abstract 634. Myeloproliferative Syndromes: Clinical: JAK Inhibitors and Their Combinations. Oral and Poster Abstract. ASH 2014.

Case Study

B.Q. is a 58-year-old male with a long-standing history of thrombocytopenia since his late twenties. Originally diagnosed with essential thrombocythemia, B.Q. was put on the clinical research trial for anegralide. At some point after completion of the clinical trial, B.Q. was unable to be maintained on anagrelide and was switched to hydroxyurea. While on hydroxyurea, B.Q. developed profound anemia and became quite symptomatic. He was treated with blood transfusions; epoetin alfa was successfully used for symptom management. B.Q. also met regularly with our psychologist to work on supportive care measures. (In fact, over the past six years, this patient has logged almost 100 sessions with our psychologist, working on supportive care issues directly related to his myelofibrosis.)

Over the last few years, B.Q.'s disease has transformed into a myeloproliferative disease. The patient did benefit from red-cell growth factors initially, but is presently unable to afford his co-pay due to a change in his primary insurance. In researching assistance options, the patient improved to the point where he was satisfied that growth factors could be used if necessary. For the past year, the patient's hemoglobin and hematocrit are stable and his platelet count is adequate. B.Q. has not taken hydroxyurea for the past year and his last flow cytometry revealed 1 percent blasts. The patient has been intermittently transfused and is concerned that his disease is progressive. B.Q. is considering our recommendation to initiate therapy with a JAK2 inhibitor. His physician's clinical opinion is that the disease is stable, but would likely benefit from a trial of a JAK2 inhibitor, such as ruxolitinib.

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