Conference Abstracts

Pacritinib in Patients with Myelodysplastic/Myeloproliferative Neoplasms

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Background

Myelodysplastic/myeloproliferative neoplasms (MDS/MPN) are a rare group of myeloid malignancies characterized by a combination of cytopenias and cytoses, divided into the following subtypes: chronic myelomonocytic leukemia (CMML), MDS/MPN with neutrophilia, MDS/MPN with SF3B1 mutation and thrombocytosis (MDS/MPN-SF3B1-T), and MDS/MPN, not otherwise specified (NOS). The use of JAK inhibitors in patients with MDS/MPN is being investigated in ongoing early phase clinical trials. While work in this space has focused on the therapeutic efficacy of ruxolitinib, there is evidence to suggest that pacritinib may represent a promising alternative, with its beneficial effect on targets such as CSF1R, IRAK1, and ACVR1, along with JAK2. This case series provides preliminary evidence of the therapeutic role of pacritinib in patients with MDS/MPN overlap syndrome.

Case Discussion

We report five patients (n=3; female) with MDS/MPN treated with pacritinib at Vanderbilt University Medical Center. The median age at diagnosis was 73 [range, 62-83] years. Diagnoses included dysplastic CMML (n=1), proliferative CMML (n=1), MDS/MPN,NOS (n=2), and MDS/MPN-SF3B1-T (n=1). Our cohort included 2 cases with higher-risk disease (moderate high per IPSS-M/Intermediate-2 by CPSS-Mol). The median treatment duration was 15 [range, 5-16] months, with four patients receiving pacritinib for relapsed/refractory disease. All patients with palpable splenomegaly (n=3) had sustained resolution following therapy. Decreased symptom burden ranging from fatigue, loss of appetite, and abdominal tenderness was experienced by three patients. Pre-therapy leukocytosis (n=2) normalized following therapy. Erythroid response (IWG 2015) was observed in two patients, one of whom had sustained post-therapy response for 10 months and platelet response for 5 months.

Conclusion

Our case series highlights the preliminary activity of pacritinib in select patients of MDS/MPN with advanced age and significant comorbidities. While we observed clinical benefit in a majority of cases in this small cohort, future clinical trials are needed to identify biologically effective doses and clinicomolecular predictors of response to therapy.