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## Managing Myelofibrosis-Associated Thrombocytopenia

### Announcer:

Welcome to CME on ReachMD. This episode is part of our MinuteCE curriculum.

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### Dr. Hobbs:

Hello. This is CME on ReachMD, and I'm Dr. Gabriela Hobbs. Today, I'm emphasizing the importance of thrombocytopenia as a prognostic factor in myelofibrosis in reviewing the available treatment options.

So thrombocytopenia is a common complication of myelofibrosis, the incidence of which is 25% in newly diagnosed patients. The prevalence is much higher, at about 68% of patients with myelofibrosis.

Thrombocytopenia is a poor-prognosis indicator, as has been demonstrated in all of the risk stratification tools that we have for myelofibrosis. Patients with thrombocytopenia have a significantly reduced overall survival. They have a 2 times higher risk of transforming to leukemia. They also have high-grade marrow fibrosis, and in addition to having thrombocytopenia, they more frequently have other cytopenias such as anemia and leukopenia.

Unfortunately, there are limited treatment options for the management of myelofibrosis and severe thrombocytopenia. Many different agents have been utilized; however, few have been efficacious. Different strategies such as interferon, splenectomy, spleen radiation, other agents like immunomodulatory agents, have been tried, including thrombopoietin mimetics, but these really do not have much of a role for the management of patients with myelofibrosis. So this is definitely an area of unmet need.

Now pacritinib is the first agent that has been approved for the management of myelofibrosis in patients that have platelets of less than 50,000. Pacritinib was approved based on the PERSIST studies, and its FDA approval is in first line for patients with myelofibrosis that have splenomegaly and symptoms and platelets of less than 50. Patients treated with pacritinib have a significantly higher likelihood of achieving an SVR of 35% as well as to have a significant improvement in their symptoms when treated with full-dose pacritinib, as opposed to being managed with best available therapy. There was recent data presented at ASCO in 2024 that showed blood count stability, in addition to some trends towards improvement in not only hemoglobin, but improvement in platelet count in a small subset of patients as well. So pacritinib definitely demonstrates an important approach for the management of patients with thrombocytopenia.

Momelotinib is approved for patients with anemia and myelofibrosis, but the MOMENTUM study also included a small subset of patients with platelets of less than 50. An analysis of the MOMENTUM study in patients with thrombocytopenia demonstrates survival advantage to patients treated with momelotinib and platelets of less than 50 compared to those treated with danazol. In addition, the efficacy of momelotinib seems to be maintained in patients treated with momelotinib and low platelets compared to patients with momelotinib treated with high platelets.

So in summary, thrombocytopenia, unfortunately, is a common complication of myelofibrosis. Thrombocytopenia is an independent prognostic risk factor and is associated with a decreased overall survival. There are limited treatment options for the management of

thrombocytopenia in patients with myelofibrosis. Pacritinib has demonstrated efficacy and an ability to improve total symptom scores as well as spleen volume response in thrombocytopenic patients. Momelotinib has also been investigated in thrombocytopenic patients, and its use is associated with meaningful total symptom score and spleen volume responses as well as an overall survival benefit in these thrombocytopenic patients, albeit in a small number of patients.

Thank you so much for your attention. I hope you find this information as useful as it was brief.

**Announcer:**

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