

RUBEN MESA, MD: My name is Ruben Mesa, and I'm one of the hematologists at the Mayo Clinic who focuses on myeloproliferative neoplasms. It was my pleasure to present at the American Society of Clinical Oncology meeting, in Chicago, the results of the PERSIST-1 study. The PERSIST-1 study is a randomized phase-three study of the JAK2 inhibitor, pacritinib, versus best alternative therapy in patients with intermediate and high-risk myelofibrosis and, in particular, in those individuals who had baseline anemia or thrombocytopenia. Myelofibrosis is a debilitating, chronic hematologic malignancy, a myeloproliferative neoplasm that can afflict people with difficult symptoms-- splenomegaly, cytopenias, the potential progression of acute leukemia, and potentially be a fatal disease.

One of the unmet needs for patients with myelofibrosis is the inability to receive effective therapy for their splenomegaly symptoms in their disease in the setting of where they have significant drop in their platelets and/or in their red blood cell, so anemia or thrombocytopenia. The PERSIST-1 study was an international study with over 300 patients who we identified and demonstrated that pacritinib was vastly superior to best alternative therapy for control of the enlarged spleen in the disease and that resulted in reduction in the size of the spleen as well significant improvement in the symptoms. What was unique about the study is that the drug was able to be given safely and well tolerated in individuals who had baseline anemia and thrombocytopenia. This makes it a unique drug for patients with myelofibrosis in that those individuals with advanced-disease features who have thrombocytopenia usually have no other treatment options.

These important and positive results, we feel, may well allow pacritinib to be an option for a broader group of patients with myelofibrosis around the world who currently struggle with difficult splenomegaly symptoms and progressive disease and have the aspect of having baseline anemia and thrombocytopenia. Indeed, we identified with the study that many individuals who had baseline anemia had improvement and became transfusion independent with the therapy. We also identified that the drug was well tolerated. There could be gastrointestinal toxicities that occur but, in general, they were self-met, they were self-limited, and could be very manageable by physicians.

So, on behalf of the PERSIST-1 investigators, we felt that this was a very positive step forward for patients with myelofibrosis, and there are other concurrent studies ongoing, including the PERSIST2 study. Thank you.