Griesshammer M, Kämpfe D, Weide R, Reichert D, Sauer A, Dörfel S, Koenigsmann M, Stauch M, Grosser S, Zander I – Vortrag V895

Interim analysis results from the prospective, non-interventional study of ruxolitinib in patients with polycythemia vera (PAVE): safety results were consistent with previous findings.

Introduction: In the clinical trial setting, ruxolitinib, a JAK1/2 inhibitor (JAKi 1/2), demonstrated efficacy in terms of hematocrit control, spleen volume reduction, and improved symptoms and quality of life in poly-cythemia vera (PV) patients, resistant/intolerant to hydroxyurea. Here, we present interim analysis data from a non-interventional study in patients with PV who received ruxolitinib in daily clinical practice in selected centers across Germany
Methods: Patients were monitored for safety, efficacy, and symptom scores for up to 36 months.
Results: Of the 365 patients (135 JAKi-naive, 230 JAKi-pretreated; data cut-off date, 19 Feb 2019), 48 completed the study, 230 were ongoing, and 87 dropped out. Adverse events (AEs) were reported in 259 (71.0%) patients, with anemia (20.0%), thrombocytosis (6.8%), fatigue (6.8%), and dizziness (5.8%) being the most frequent. One case of second primary malignancy was re-ported that was deemed as unrelated to the study treatment. No aggressive B-cell lymphoma incidences were seen. Infections/infestations were reported in 77 (21.1%) patients, including nasopharyngitis (4.4%), herpes zoster (3.0%), and urinary tract infection (2.7%). Serious AEs were re-ported in 98 (26.8%) patients, with pneumonia (1.6%), anemia (1.4%), and atrial fibrillation (1.4%) being some of the most common. At baseline, the median (range) spleen size was 11 cm (0 to 30) in JA-Ki-naive patients and 10 cm (0 to 20.4) in JAKi-pretreated patients, which changed by −2 cm (−13 to 2) and 0 cm (−13.9 to 9), respectively, at the last post-baseline follow-up. The median (range) hematocrit levels changed from baseline to last post-baseline visit by −5.3% (−27.5% to 48.1%) in JAKi-naive and −1.5% (−17.5% to 16.0) in JAKi-pretreated patients. Myeloproliferative neoplasm symptom assessment form derived total symptom scores (MPN-SAF TSS) have improved in the JAKi-naive patients, whereas JAKi-pretreated patients who benefited from prior ruxolitinib therapy maintained their scores; the median (range) MPN-SAF TSS scores were 28.5 (10.0 to 83.0) and 24.0 (6.0 to 65.0) at baseline, and 20.0 (8.0 to 61.0) and 24.5 (10.0 to 73.0) at the last post-baseline visit, respectively.
Conclusion: The safety profile of ruxolitinib in daily clinical practice was consistent with the previous findings, with anemia and diverse infections being the most frequent AEs. Ruxolitinib treatment showed a positive effect on symptom burden, spleen size and hematocrit levels.