Safety and Efficacy of Ruxolitinib in Patients With Low Platelets
Enrolled in a Phase 3bExpanded-Access Study in Myelofibrosis

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**INTRODUCTION**

Ruxolitinib is a potent Janus kinase (JAK)1/JAK2 inhibitor that has demonstrated durable reductions in spleen size and myelofibrosis (MF)–related symptoms and improved survival compared with placebo and best available therapy in the 2 large phase 3 COMFORT studies.1-3 The COMFORT studies used baseline platelet count (PLT) criteria to identify patients with severe MF, classified as high-risk, intermediate-risk, or intermediate-risk 1+ with a palpable spleen ≥ 5 cm from the costal margin.

**RESULTS**

**METHODS**

- **Inclusion criteria**
  - Age of patients at study entry: ≥ 18 years
  - Platelet count ≥ 50 × 10⁹/L
  - Available bone marrow aspirate
  - Meet one or more of the following IPSS criteria: ≥ 65 years, n (%) 33 (66)
  - Hemoglobin ≤ 10 g/dL, n (%) 18 (36)
  - White blood cell count ≥ 1000/µL, n (%) 18 (36)
  - Platelet count ≥ 50 to < 100 × 10⁹/L, n (%) 48 (96)

- **Exclusion criteria**
  - Presence of active bleeding
  - Patients who required platelet transfusions within 30 days before baseline

- **Study design**
  - Phase 3b expanded-access study
  - Treatment duration: up to 2 years

- **Follow-up**
  - Up to 2 years after completion of study treatment

**RESULTS**

**CONCLUSIONS**

- **Efficacy**
  - The most common adverse event (AE) related to ruxolitinib treatment was edema peripheral (38%) followed by headache (19%), anemia (16%), and dyspnea (12%).
  - Of evaluable patients at week 24, 38.2% (13/34) achieved a ≥ 50% reduction from baseline in the Trial Outcome Index, and 29.6% (10/34) achieved a ≥ 25% reduction from baseline in spleen length.

- **Nonhematologic AEs**
  - Among the nonhematologic AEs regardless of drug relationship, the most frequently reported included dyspnea (35%) and fatigue (31%).

- **Implications**
  - Lower-dose ruxolitinib is generally safe and efficacious in patients with MF who have low baseline PLTs.

**REFERENCES**

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