**P1047 REAL-WORLD SAFETY OF RUXOLITINIB IN PATIENTS WITH INTERMEDIATE OR HIGH RISK OF PRIMARY MYELOFIBROSIS, POST-POLYCYTHEMIA VERA MYELOFIBROSIS OR POST-ESSENTIAL THROMBOCYTHEMIA MYELOFIBROSIS IN CHINA**

**Topic:** 16. Myeloproliferative neoplasms - Clinical

Zefeng Xu1, Minghui Duan2, Qian Jiang3, Qing Leng4, Na Xu5, Yin Zhang6, Chunting Zhao7, Wen Wu8, Qi Ke Zhang9, Jiaping Fu10, Jingyu Zhang11, Rong Fu12, Zhenyu Yan13, Jingdong Zhang14, Congmeng Lin15, Guifang Ouyang16, Zhi Wang17, Liping Ma18, Hongling Hao19, Xiaoming Li20, Siyang Ran21, Yongbing Chen22, Tao Li23, Zhijian Xiao1

1 State Key Laboratory of Experimental Hematology, National Clinical Research Center for Blood Diseases, Institute of Hematology and Blood Diseases Hospital, Chinese Academy of Medical Sciences & Peking Union Medical College, Tianjin, China; 2 Peking Union Medical College Hospital, Beijing, China; 3 Peking University People's Hospital, Beijing, China; 4 Anshan Central Hospital, Liaoning, China; 5 Nantong Hospital, Southern Medical University, Guangdong, China; 6 Henan Provincial People's Hospital, Henan, China; 7 Affiliated Hospital of Qingdao University, Shandong, China; 8 Ruijin Hospital, Shanghai Jiao Tong University, Shanghai, China; 9 Gansu Provincial People's Hospital, Gansu, China; 10 Shaoying People's Hospital, Zhejiang, China; 11 The Second Hospital of Hebei Medical University, Hebei, China; 12 Tianjin Medical University General Hospital, Tianjin, China; 13 North China University of Technology Affiliated Hospital, Hebei, China; 14 Nanchang University Affiliated Ganzhou Hospital, Jiangxi, China; 15 Zhangzhou Affiliated Hospital of Fujian Medical University, Fujian, China; 16 Ningbo First Hospital, Zhejiang, China; 17 Wuhan People's Hospital, Jiangsu, China; 18 Sun Yat Sen Memorial Hospital of Southern University of Science and Technology, Guangdong, China; 19 Shaoxing People's Hospital, Zhejiang, China; 20 Affiliated Hospital of Southwest Medical University, Sichuan, China; 21 Beijing Novartis Pharma Co., Ltd, Beijing, China; 22 China Novartis Institutes For Biomedical Research Co. Ltd., Shanghai, China

**Background:** Ruxolitinib, a potent and selective inhibitor of Janus kinase 1 (JAK1)/JAK2 tyrosine kinases, was approved for treatment of intermediate- and high-risk patients with myelofibrosis (MF) based on the findings from the pivotal studies, COMFORT I and II. Although an Asia regional study was conducted, the enrolled Chinese population was limited (N=63).

**Aims:** We aimed to evaluate the safety profile and the treatment of ruxolitinib in Chinese patients with MF under routine clinical practice.

**Methods:** This was a retrospective, non-interventional, multicenter, post-marketing surveillance study collecting data up to 48 weeks after ruxolitinib initiation. Patients aged ≥18 years with a confirmed diagnosis of intermediate or high-risk primary MF (PMF), post-polycythemia vera MF (PPV-MF) or post-essential thrombocytemia MF (PET-MF) who had received or were currently receiving ruxolitinib treatment per clinical judgment according to China approved label were included.

**Results:** In total, 480 patients from 20 sites were enrolled and 428 patients completed the study treatment. The most reported reasons for treatment discontinuation were death (17.3%), adverse events (AE, 7.7%) and abnormal laboratory value (5.8%). Gender distribution were balance (male 51.6% vs female 48.4%) with median age of 61.0 years and median BMI of 22.5 kg/m². Majority of the patients were diagnosed with PMF (69.8%), followed by PPV-MF (15.7%), and PET-MF (14.5%).

The information on dosing and safety is detailed in Table 1. Ruxolitinib dosing was according to the local approved label (median initial daily dose 30.0 mg). The median time from first diagnosis to medication was 1.5 months. Overall, patients received ruxolitinib for a median of 11.1 months with median dose intensity of 28.7 mg/day. The most prescribed dose was 15 mg BID across varying durations of exposure to ruxolitinib (up to 48 weeks). A total of 196 (40.8%) patients had dose modification.
On-treatment AE and serious AE (SAE) were reported in 361 (75.2%) and 57 (11.9%) patients, respectively. Of those, 60.2% and 6.0%, were related to study drug, respectively. The severity of majority of the AE were grade 2 and 3 (26.7 & 25.2%, respectively) while SAEs were grade 3 & 4 (3.5% & 4.4%, respectively). A total of 4.0% and 2.3% of patients reported AEs and SAEs leading to discontinuation. The most commonly reported AEs were anemia (3.8%), platelet count decreased (1.0%), infectious pneumonia and pulmonary inflammation (both 0.8%). On-treatment AESI was reported by 18 (3.8%) patients, consisted of bleeding events (2.3%), serious or opportunistic infections (0.8%), and second primary malignancies (SPM, 0.6%). The SPM were malignant tumors (n=2) and liver cancer (n=1). A total of 18 on-treatment deaths (MF progression=9, AE=4, and others=5) were reported.

During the study period, we found that the number of patients with efficacy data were decreasing with time (baseline=183&116, Week 16=126 & 96, Week 32=90 & 62, Week 48=38 & 19 for spleen size measurement and total symptom score, respectively). This reflects the need for improve compliance in disease management.

**Image:**

**Summary/Conclusion:** This was the largest ruxolitinib real-world study in China. The safety profile of ruxolitinib in patients with intermediate- or high-risk PMF, PPV-MF, or PET-MF was generally consistent with previous global and Chinese studies of MF. The incidence of SPM was lower than that reported previously. No new unexpected safety signals have been identified.