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First interim analysis of the German prospective, non-interventional study PaVe: first results on the clinical routine of ruxolitinib in polycythemia vera

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Introduction: Ruxolitinib (RUX) is an oral, selective JAK1/2 inhibitor (JAKi) approved for the treatment of adult patients (pts) with polycythemia vera (PV) who are resistant or intolerant to hydroxyurea. The prospective, noninterventional study (NIS) PaVe (CINC424BDE12) is designed to document the current use and outcome of RUX therapy in daily clinical practice in Germany.

Methods: A population of 184 pts (81 JAKi-naive and 103 JAKi-pretreated pts (all RUX and 1 pts additionally momelotinib)) documented between 08/2015 and 01/2017 in 70 German centers was analyzed. RUX was administered according to the SmPC. Drug utilization, efficacy, safety, tolerability and quality of life were documented for each patient.

Results: At baseline, median age was 71 years and gender was balanced (50.5% male). 28.3% of pts had a previous thromboembolic event. The median time since diagnosis was 81.9 months, with abnormal blood counts as the most common reason for diagnosis. Only 14.7% of pts had a palpable spleen. 96.1% were positive for the JAK2V617F mutation. The majority of pts (90.9%) had an ECOG performance status of either 0 or 1. Baseline differences between JAKi-naive and -pretreated pts were the median time since last phlebotomy (5.1 vs 12.6 months respectively), the number of pts with constitutional symptoms (61.7% vs 42.7%) and the median hematocrit (Hct) (42.6% vs 38.0%). The median follow-up was 5.6 months and RUX was generally well tolerated, with 90.8% of pts still on treatment at data cut-off. After the start of RUX therapy, the Hct values of JAKi-naive pts decreased rapidly and reached levels similar to those of pretreated pts at month 2, remaining stable thereafter. Similar effects were seen for leukocyte and platelet counts. The mean number of phlebotomies per year while on study was 0.46. The MPN10 total symptom score (TSS) decreased in more than 50% of pts at all analyzed time points. 48.9% of pts experienced at least one adverse event (AE) and 12.0% a serious AE (SAE) (242 and 37 events in total). All SAEs were judged by the treating physician to be unrelated to RUX. The most frequent AEs were anemia (14.7%), fatigue (4.3%), pruritus (3.8%), hypertension (3.3%) and decreased hemoglobin (3.3%). More detailed data will be presented at the conference.

Conclusions: This interim analysis of the PaVe NIS depicts a representative group of PV pts treated with RUX and confirms the safety and efficacy profile of the two RESPONSE trials in a real world setting.