PACRITINIB VS BEST AVAILABLE THERAPY, INCLUDING RUXOLITINIB, IN PATIENTS WITH MYELOFIBROSIS AND BASELINE THROMBOCYTOPENIA: FOCUS ON ANEMIA IN THE PHASE 3 PERSIST-2 TRIAL

INTRODUCTION
- Myelofibrosis (MF) is a life-threatening hematologic malignancy characterized by splenomegaly, debilitating constitutional symptoms, and progressive cytopenias.
- Thrombocytopenia is an early precursor to myeloablation that is associated with disease progression and is highly correlated with symptom burden and shorter survival.
- Approved JAK1/2 inhibitor ruxolitinib reduces splenomegaly and symptoms in patients with MF and produces minimal myelosuppression.
- In the phase 3 PERSIST-2 trial of RUX vs best available therapy (BAT), myelosuppression (RUX) in patients with MF and baseline thrombocytopenia.
- PAC 200 mg twice daily (BID) had a more favorable adverse event profile compared with PAC QD and BAT and reduced the incidence of serious treatment-emergent anemia.

OBJECTIVE
- To evaluate the impact of baseline anemia or transfusion-dependence on efficacy outcome measures, as well as the effects of therapy on anemia in patients from the phase 3 PERSIST-2 study.

METHODS

RESULTS
- At baseline, 59% of patients in the ITT-Efficacy population had anemia (Table 1).
- Patients with anemia at baseline were more likely to have primary MF (17% vs 51%), a lower platelet count (median 39 vs 50 × 10^9/L), and a lower hemoglobin level (median 10.1 vs 10.9 g/dL) compared with patients without anemia.
- At baseline, 30% of patients had concomitant RBC transfusion needs.

CONCLUSIONS
- Patients with anemia at baseline were more likely to have primary MF (71% vs 30%) and to meet the definition of RBC-transfusion-dependence (TD).
- In PAC patients, SVR ≥35% and TSS reduction ≥50% at week 24 were observed regardless of baseline anemia or transfusion burden.
- Rates of any treatment-emergent grade 3/4 and serious AEs were higher in patients with baseline anemia on the PAC QD and BAT arms, but not on the PAC BID arm.

REFERENCES
3. University of Michigan, Comprehensive Cancer Center, Ann Arbor, MI, USA; Fisch Cancer Institute, Icahn School of Medicine at Mount Sinai, New York, NY, USA; Cleveland Clinic, Cleveland, OH, USA; Northwestern University, Feinberg School of Medicine, Chicago, IL, USA; Princess Margaret Cancer Center, University of Toronto, Ontario, Canada; Albert Szent-Györgyi Clinical Center, University of Szeged, Szeged, Hungary; Beaumont West of Scotland Cancer Centre, Glasgow, UK; Ryazan’s Clinical Hospital, Ryazan, Russia; CTI BioPharma Corp., Seattle, WA, USA; Stanford Cancer Institute, Stanford, CA, USA; University of California-San Diego, La Jolla, CA, USA; Guy’s and St Thomas’ NHS Foundation Trust, London, UK; Mayo Clinic, Scottsdale, AZ, USA; MD Anderson Cancer Center, Houston, TX, USA.

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ADDITIONAL TABLES AND FIGURES

Figure 1. Study Schema

Figure 2. Mean Hemoglobin Levels Through Week 24 (A) All Patients and (B) Patients With Baseline Anemia (Safety Population, Central Laboratory Assessment)

Figure 3. Improvement in Hemoglobin (A) and Transfusion Burden (B) at Week 24 (ITT-Efficacy Population)

Figure 4. RBC Transfusions Over Time in All Patients With 21 RBC Unit on Study (ITT Population)

Figure 5. Spleen Volume Reduction (35%) and Total Symptom Score Reduction ≥50% (B) at Week 24 (ITT-Efficacy Population)

Table 1. Key Baseline Characteristics (ITT-Efficacy Population)

Table 2. Summary of Anemia Adverse Events (Safety Population)

Table 3. Treatment-Emergent Adverse Events (Safety Population)