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Preliminary safety and efficacy of ruxolitinib in patients (pts) with primary and secondary myelofibrosis (MF) with platelet counts (PC) of 50–100x10^9/L.

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Abstract Disclosures

Abstract

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Background: Ruxolitinib (RUX) has demonstrated clinical benefit as therapy for MF. This study explores the safety and efficacy of RUX in pts with MF and low PC, where clinical data are limited. **Methods:** In this phase II study, pts with intermediate-1 to high-risk MF, and PC of 50–100x10^9/L started RUX at 5 mg BID. Doses could be increased in 5 mg QD increments every 4 weeks (wks) beginning at wk 4. Doses were decreased or held for PC <35x10^9/L and <25x10^9/L respectively. Assessments: Total Symptom Score (TSS, using the modified MF Symptom Assessment Form v2.0, and comprised of scores from 0=absent to 10=worst imaginable for night sweats, itching, bone/muscle pain, early satiety, abdominal discomfort and pain under left ribs); Patient Global Impression of Change (PGIC, a 7-point scale ranging from "very much improved" to "very much worse"); spleen size by palpation and by MRI (data not yet available); and safety. **Results:** At the time of analysis, 23 pts had completed ≥4 wks on study. At baseline: mean PC=72x10^9/L; high (4%), intermediate-2 (52%) and intermediate-1 (44%) risk group; mean spleen length=16.6 cm; mean TSS=25.5. At the time of analysis, 4%, 40%, 26%, 26% and 4% of pts were receiving RUX 5 mg QD, 5 mg BID, 5 mg AM/10 mg PM, 10 mg BID, and 10 mg AM/15 mg PM, respectively. At wk 4 (all pts on 5 mg BID), mean TSS improved 14%; 13% had ≥50% improvement. At wk 8 (52% on 5 mg AM/10 mg PM), mean TSS improved 23%; 30% had ≥50% improvement. Mean spleen length reduction of 22% (wk 4) and 27% (wk 8) was observed, and PGIC scores of "much improved" or "very much improved" were reported in 35% (wk 4) and 39% (wk 8) of pts. There were no Grade 4 PC, no dose holds for AEs and no discontinuations; 1 pRBC transfusion-dependent pt had Grade 4 anemia. Three pts experienced a total of 4 SAEs (fever; hypnagogic dreams; and spleen pain/pneumonitis) which resolved while on treatment. **Conclusions:** Effects on spleen size, PGIC, and TSS, even over the first weeks at low doses, are superior to those observed in the placebo group in the COMFORT-I study. These preliminary findings suggest a dosing strategy starting with 5 mg BID RUX with subsequent dose optimization may be efficacious and well tolerated in MF pts who have low platelets.

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