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Safety and efficacy findings from the open-label, multicenter, phase 3b, expanded treatment protocol study of ruxolitinib for treatment of patients with polycythemia vera who are resistant/intolerant to hydroxyurea and for whom no alternative treatments are available

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Abstract

Ruxolitinib was recently approved for the treatment of patients with polycythemia vera who are resistant/intolerant to hydroxyurea based on data from the RESPONSE studies. This phase 3b, Expanded Treatment Protocol study (NCT02292446) of ruxolitinib for hydroxyurea-resistant/intolerant patients with polycythemia vera (*N* =

161: median exposure = 25.1 weeks) further evaluated the safety of ruxolitinib. Adverse events (AEs) led to dose adjustment/interruption in 37.9% of patients and study drug discontinuation in 8.7% of patients. The most common hematologic AEs included anemia and thrombocytosis; while headache and diarrhea were the most frequent nonhematologic AEs. At week 24, 45.3% of patients achieved hematocrit control; hematologic remission was seen in 18% of patients. At least, 50% of reduction in spleen length was achieved in 86.7% of patients from baseline at any time. The observed safety profile of ruxolitinib was consistent and the efficacy results were similar to the observed values in the RESPONSE studies.

Keywords: Myeloproliferative neoplasms polycythemia vera ruxolitinib

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Disclosure statement

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Ethics approval and consent to participate

This study was approved by the institutional review board (IRB). The IRB for each investigator site approved the respective protocols. The study was conducted in accordance with the principles of the Declaration of Helsinki. Informed consent was obtained from all patients for

principles of the Declaration of Helsinki. Informed consent was obtained from all patients for being included in the study.

Data availability

The authors confirm that the data supporting the findings of this study are available within the article [and/or] its supplementary materials.

Novartis is committed to sharing with qualified external researchers, access to patient-level data and supporting clinical documents from eligible studies. These requests are reviewed and approved by an independent review panel on the basis of scientific merit. All data provided is anonymized to respect the privacy of patients who have participated in the trial in line with applicable laws and regulations. This trial data availability is according to the criteria and process described on www.clinicalstudydatarequest.com