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# Characteristics, Blood Counts, Treatments, and Clinical Outcomes of 3022 Patients With Polycythemia Vera Treated in US Community Practices

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## Background

- Polycythemia vera (PV) is a myeloproliferative neoplasm characterized by elevated hematocrit, trilineage myeloproliferation in the bone marrow, and presence of Janus kinase 2 (*JAK2*) activating mutations,[1] and is associated with reduced survival[2,3]
- Treatment goals are to reduce blood counts (hematocrit [HCT], <45%; white blood cell [WBC] count, <10 × $10^9$/L; platelet count, <400 × $10^9$/L) and manage cardiovascular risk factors to reduce risk of thromboembolic events[4-6]
- Cytoreduction with hydroxyurea (HU) is the recommended first-line treatment for high-risk PV (age ≥60 years or history of thrombosis), but many patients have a suboptimal response to HU[7-9]

## Objective

- To describe characteristics, blood counts, treatments, and clinical outcomes among patients with PV, including those with suboptimal response to HU, in a large network of US community oncology practices

## Methods

### *Study Design and Patients*

- Retrospective observational study conducted using The US Oncology Network practice data in the iKnowMed electronic health record database
- Adult patients newly diagnosed with PV during the study identification period (January 1, 2008–January 31, 2020) and with ≥2 postdiagnosis visits during the study observation period (January 1, 2008–January 31, 2021) were eligible to participate
- Suboptimal response to HU was defined as meeting ≥1 of the following criteria after ≥3 months of treatment: (1) WBC count >10 × $10^9$/L, (2) platelet count >400 × $10^9$/L, and/or (3) HCT >45%

### *Statistical Analyses*

- Data on demographic/clinical characteristics and prior treatment were collected and summarized using descriptive statistics
- Duration of therapy and overall survival were assessed via the Kaplan-Meier method; patients without subsequent treatment discontinuation/death records at the end of the study observation period were censored

## Results

### *Patient Characteristics*

- A total of 3022 patients with PV were included in this analysis (**Table 1**)
  - Mean age was 65.8 years, 54.4% were male, and 85.3% were White
  - Elevated WBC (>10 × $10^9$/L), platelets (>400 × $10^9$/L), or HCT (>45%) were observed in 36.7%, 32.3%, and 66.9%, respectively

**Table 1. Patient Demographics and Baseline Characteristics**

| | N=3022 |
|---|---|
| Age at first diagnosis, mean (SD), y | 65.8 (13.9) |
| ≥60 y, n (%) | 2089 (69.1) |
| Male | 1645 (54.4) |
| White, n/N (%) | 2163/2535 (85.3) |
| Tobacco use, n (%) | n=2303 |
| Never used tobacco | 1184 (51.4) |
| Former tobacco use | 782 (34.0) |
| Current tobacco use | 337 (14.6) |
| *JAK2* mutation–positive* during study period, n/N (%) | 43/85 (50.6) |
| History of TE, n (%) | 208 (6.9) |
| Elevated HCT (>45%), n/N (%) | 1617/2418 (66.9) |
| Elevated hemoglobin (>15 g/dL), n/N (%) | 1410/2411 (58.5) |
| Elevated WBC count (>10 × $10^9$/L), n/N (%) | 886/2413 (36.7) |
| Elevated PLT count (>400 × $10^9$/L), n/N (%) | 778/2411 (32.3) |
| High-risk† status, n (%) | 2153 (71.2) |

*JAK2*, Janus kinase 2; HCT, hematocrit; N, number of evaluable patients; PLT, platelet; TE, thromboembolic event; WBC, white blood cell.
* Includes *JAK2*V617F and *JAK2* exon 12-15 mutations.
† Age ≥60 years or history of thrombosis.

### *Treatment Patterns*

- HU was the most common first-line treatment regardless of risk status; however, many patients did not receive any cytoreductive therapy (**Table 2**)
- Among patients who received first-line HU (n=1317), median (IQR) time from diagnosis to initiation was 19.0 (0.0–189.0) days, regardless of risk status; median (IQR) duration of treatment was 29.5 (9.5–58.5) months for high-risk PV and 35.0 (9.5–77.0) months for low-risk PV

**Table 2. First-Line Cytoreductive Treatment***

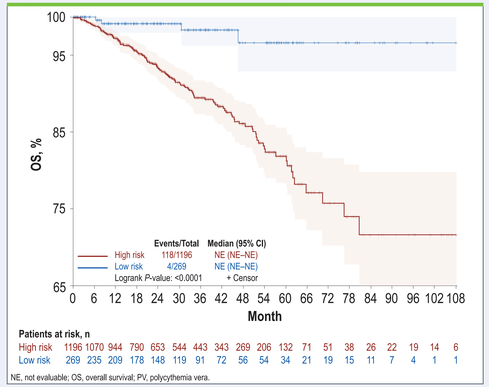
| Treatment, n (%) | High risk (n=2153) | Low risk (n=869) |
|---|---|---|
| Hydroxyurea | 1075 (49.9) | 242 (27.8) |
| Ruxolitinib | 93 (4.3) | 22 (2.5) |
| Anagrelide | 24 (1.1) | 4 (0.5) |
| Busulfan | 2 (0.1) | 0 |
| Interferon/PEGylated interferon | 2 (0.1) | 1 (0.1) |
| No treatment | 957 (44.4) | 600 (69.0) |

PEG, polyethylene glycol.
* Phlebotomy use is not available in the iKnowMed structured database and was therefore not examined.

### *Overall Survival*

- 5-year survival probability was 84.4% among all patients, 73.8% for high-risk patients, and 96.6% for low-risk patients (age <60 years and no history of thrombosis; **Figure 1**)

**Figure 1. Overall Survival in Patients With High-Risk and Low-Risk PV**

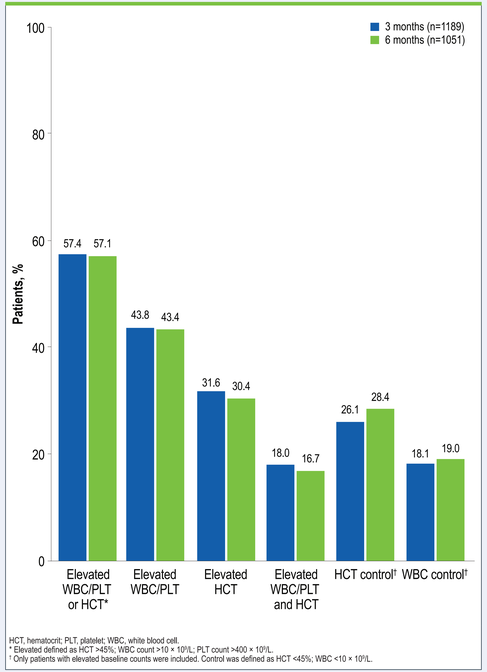



NE, not evaluable; OS, overall survival; PV, polycythemia vera.

### *Blood Count Control*

- At 3 months, 57.4% of patients had a suboptimal response to cytoreductive treatment, and this remained roughly the same at 6 months (**Figure 2**)

**Figure 2. Blood Count Control During Treatment**




HCT, hematocrit; PLT, platelet; WBC, white blood cell.
* Elevated defined as HCT >45%; WBC count >10 × $10^9$/L; PLT count >400 × $10^9$/L.
† Only patients with elevated baseline counts were included. Control was defined as HCT <45%; WBC <10 × $10^9$/L.

### *Patients With Suboptimal Response to First-Line HU*

- Among patients treated with first-line HU, 55.5% had a suboptimal response at 3 months, among whom 61.6% continued to have a suboptimal response at 6 months
  - Median duration of first-line HU treatment for patients with suboptimal response was 30.2 (95% CI, 25.4–35.7) months
  - 5-year survival probability was 86.7% for the suboptimal response group (high risk, 84%; low risk, 98.9%)

## Conclusions

- **The prevalence of elevated blood counts at diagnosis and at follow-up while on HU treatment is consistent with previous real-world experience[8,10-12]**
- **Despite suboptimal response after 3 months of first-line HU treatment in a real-world setting, nearly two-thirds of patients remained on HU with continuing suboptimal response at 6 months**
- **About 1 in 8 patients with a suboptimal response to HU died within 5 years**
- **Future research should examine whether HU dose adjustments or alternative treatments may be needed for patients with suboptimal response to HU**

### Disclosures

RML provided research consulting services to Incyte Corporation. KMA is an employee and shareholder of McKesson Corporation and provided research consulting services to Incyte. LS, DV, JN, and IZ are employees of McKesson Corporation and provided research consulting services to Incyte. JY is an employee and shareholder of Incyte.

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