Hematocrit control, symptom burden and time toxicity among phlebotomy-treated patients with polycythemia vera in the United States

MPN-718

Dr Lindsay Rein[1], Angela Fan[2], Chloe Hammond[3], Alicia Cerretani[4] Amber Simpson[3], Katie Lewis[3], Luis Hernandez[2]

1 Duke University Hospital, Durham, NC, USA; 2 Takeda Development Center Americas, Inc., Cambridge, MA, USA; 3 Adelphi Real World, Bollington, UK; 4 Takeda Pharmaceuticals America, Inc., MA, USA

Background

- Polycythemia vera (PV) is a myeloproliferative neoplasm characterized by excessive red blood cell production (erythrocytosis) which increases hematocrit (HCT) levels, putting patients at risk for associated symptoms and thromboembolic events.
- Symptoms associated with PV include fatigue, itchiness, headaches, dizziness, and concentration problems and treatments for PV may also exacerbate symptom burden. [1].
- PV management often includes phlebotomy to reduce HCT levels to <45%, the threshold for reduced thrombotic risk [3].
- Despite widespread utilization, the burden associated with phlebotomy treatment remains poorly understood, especially in the real-world.
- Hence, it is important to understand HCT control within phlebotomy-treated patients, as well as characterizing phlebotomy-associated burden, including patient symptoms and health-related quality of life (HRQoL).

Objective

- This study aimed to characterize real-world HCT control, symptom burden, time toxicity and quality of life in phlebotomy-treated patients with PV.

Methods

- Data were drawn from the Adelphi PV Disease Specific Programme™ (DSP), a cross-sectional survey with retrospective medical chart review, of hematologists/hematologist-oncologists and their patients in the United States between August 2025 and February 2026 (**Figure 1**).
- The DSP methodology has been previously described and validated [4-7].
- Physicians reported symptoms, HCT levels, and treatment history for ~5 of their next patients with PV that they saw. These patients were invited to voluntarily complete a patient self-completion form (PSC) containing their experiences of phlebotomy and validated patient-reported outcome measures such as the Patient-Reported Outcomes Measurement Information System (PROMIS) Fatigue questionnaire, EQ-5D-5L and EuroQol visual analogue scale (EQ-VAS).
- From the PROMIS Fatigue questions, a T-score was calculated, where a score of 50 represents average fatigue in the general US population, and higher numbers indicate greater fatigue [8].
- A utility score representing overall HRQoL was calculated from EQ-5D-5L domain scores using the US tariff [9], ranging from less than 0 (death) to 1 (full health).
- The EQ-VAS is a patient's rating of overall current health, ranging from 0 (worst health imaginable) to 100 (best health imaginable).

Results

Demographics

- Overall, 39 physicians provided data for 160 patients, of whom 61% (n=97) had received phlebotomy in the 12 months prior to survey (**Table 1**).

Table 1. Patient demographics and clinical characteristics

	All phlebotomy-treated patients (n=97)	Phlebotomy alone* (n=47)	Phlebotomy* + CRT** (n=50)
Patient age at time of survey			
Median (IQR)	65.0 (52.0, 70.0)	59.0 (45.0, 69.0)	67.0 (58.8, 70.0)
Patient sex, n (%)			
Male	70 (72)	35 (74)	35 (70)
Female	27 (28)	12 (26)	15 (30)
Months from PV diagnosis to time of survey			
Median (IQR)	11.0 (4.0, 37.0)	9.0 (3.0, 19.0)	15.5 (5.0, 50.0)
ECOG score at time of survey†, n (%)			
0-1	80 (82)	42 (89)	38 (76)
2-4	16 (16)	5 (11)	11 (22)
Risk status (high risk: aged ≥60 years and/or prior thrombosis history), n (%)			
High risk	38 (39)	25 (53)	13 (26)
Low risk	59 (61)	22 (47)	37 (74)

CRT: cytoreductive therapy; ECOG, Eastern Cooperative Oncology Group; IQR, interquartile range; PV, polycythemia vera

* In the 12 months prior to survey. **At the time of survey. †ECOG score was not known for 1 patient in the phlebotomy + CRT group.

Study design

Figure 1. Study methodology

Hematologists/ hem-oncologists were recruited to the Adelphi Real World PV **Disease Specific Programme™** in the **US**.

Physician inclusion criteria: Physicians involved in the treatment and management of patients with PV; with access to their medical records; who saw 5 or more patients in the year prior to survey; and agreed to all rules and regulations, were included.

After screening into the survey, physicians completed patient record forms **(PRFs)** for their next ~5 **consecutively consulting** patients with **PV**.

Patient inclusion criteria: Patients were eligible if they were over the age of 18; had a physician-confirmed PV diagnosis; and were not participating in a clinical trial at data collection.

PRFs collected data including **patient demographics, clinical characteristics, and treatment history.**

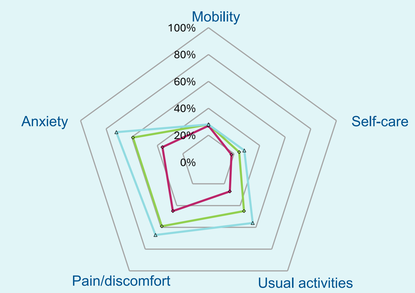
Completed at a single point in time, with retrospective data also captured from medical history

Patients were invited to **voluntarily** complete **patient self-completion** forms.

Completed at a single point in time, capturing patient experiences with phlebotomy and validated patient-reported outcome measures.

Results

Figure 2. Proportion of patients reporting any level of problem in the individual EQ-5D-5L domains



* In the 12 months prior to survey. **At the time of survey.

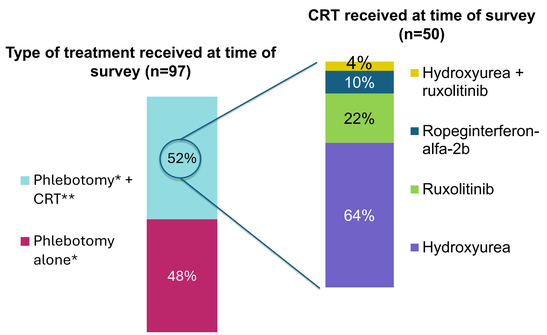
Health-related Quality of Life

- At time of survey, mean (SD) EQ-5D-5L utility scores were 0.81 (0.17) for phlebotomy-treated patients (n=29 who completed all EQ-5D-5L domains), 0.88 (0.12) for phlebotomy-alone patients (n=11), and 0.76 (0.19) for patients receiving phlebotomy + CRT (n=18). Of all phlebotomy-treated patients, 59% reported experiencing pain/discomfort to some extent, and 59% anxiety to some extent (**Figure 2**).
- Mean (SD) EQ VAS scores were 83.0 (11.7) for phlebotomy-treated patients (n=30 who completed the EQ-VAS), 86.3 (8.8) for phlebotomy-alone patients (n=12), and 80.8 (13.1) for phlebotomy + CRT patients (n=18).
- The mean (SD) PROMIS Fatigue T-score was 54.2 (8.3) for phlebotomy-treated patients (n=28), 49.4 (9.1) for phlebotomy-alone patients (n=9), and 56.5 (7.0) for phlebotomy + CRT patients (n=19).
- Patients reported missing a mean (SD) of 3.7 (4.6) hours from their day per phlebotomy procedure (n=27); this was 3.1 (2.6) hours for phlebotomy-alone patients (n=10) and 4.1 (5.5) hours for phlebotomy + CRT patients (n=17).
- Phlebotomy was reported as time-consuming by 77% of all phlebotomy-treated patients (n=26); this was 56% of phlebotomy-alone patients (n=9) and 88% of phlebotomy + CRT patients (n=17).
- When asked how burdensome the frequency of their phlebotomy treatment was, 81% of phlebotomy-treated patients (n=27), 64% of phlebotomy-alone patients (n=11), and 81% of phlebotomy + CRT patients (n=16) reported burden to some degree.

Key takeaways

In this real-world US cohort, patients receiving phlebotomy alone or in combination with CRT had poor HCT control and experienced multifactorial burden and impact on HRQoL. This highlights a need for new treatments that offer HCT control as well as reducing patient burden.

Figure 3. Treatments received at the time of survey



* In the 12 months prior to survey. **At the time of survey.

Treatments Received Alongside Phlebotomy at Time of Survey

- Around half (48%, n=47) of patients were receiving phlebotomy alone at time of survey, and 52% (n=50) were receiving phlebotomy plus cytoreductive therapy (CRT) (**Figure 3**).
- Of the 50 patients who were receiving phlebotomy + CRT, 64% were receiving hydroxyurea, 22% were receiving ruxolitinib, 10% were receiving ropeginterferon-alfa-2b, and 4% were receiving a hydroxyurea plus ruxolitinib combination, at the time of survey (**Figure 3**).
- In the 12 months prior to survey, phlebotomy-alone patients had received a mean (standard deviation; SD) of 3.0 (2.2) phlebotomies, and phlebotomy + CRT patients (n=49) had received a mean of 2.8 (2.0) phlebotomies.

HCT Control

- Of phlebotomy-alone patients (n=46), 87% had uncontrolled HCT levels (defined as at least one HCT result ≥45% in the 12 months prior to survey) (**Figure 4a**). Of patients with uncontrolled HCT levels (n=40), 70% had 2 or more HCT results ≥45%. In the phlebotomy + CRT group (n=48), 96% had uncontrolled HCT (**Figure 4a**). Of patients with uncontrolled HCT levels (n=46), 83% had 2 or more HCT results ≥45%.
- In the phlebotomy-alone group, patients had a median (IQR) of 3.0 (3.0, 5.0) HCT tests in the 12 months prior to survey, and this was 4.0 (2.8, 6.2) in the phlebotomy + CRT group (**Figure 4b**).

Figure 4a. HCT control in the 12 months prior to survey

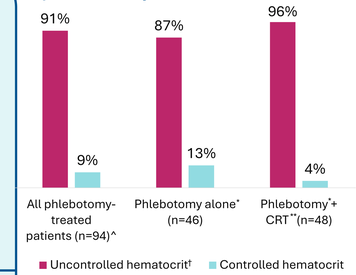
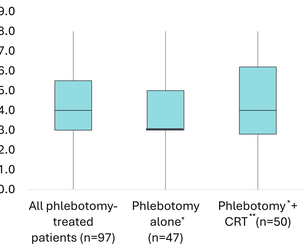


Figure 4b. Number of HCT tests received in the 12 months prior to survey



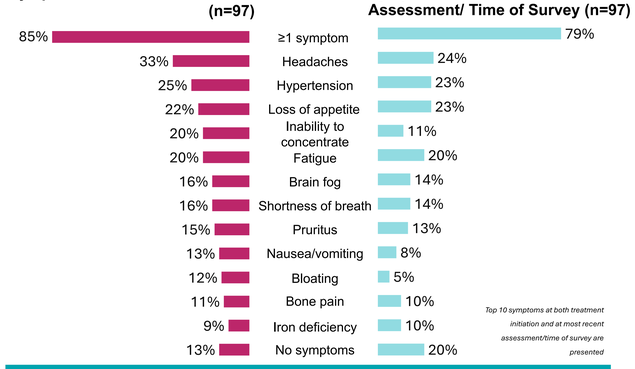
^Includes all patients with known HCT test results in the 12 months prior to survey

† Uncontrolled HCT defined as at least one HCT result ≥45% in the 12 months prior to survey. * In the 12 months prior to survey. **At the time of survey.

Symptoms Experienced

- At treatment initiation, 85% of phlebotomy-treated patients had at least one symptom, versus 79% when assessed most recently to the time of survey (**Figure 5**).
- Physicians most commonly reported that patients were experiencing headaches (33%), hypertension (25%), and loss of appetite (22%) at treatment initiation. The most common symptoms at the most recent assessment/time of survey were headaches (24%), loss of appetite (23%), and hypertension (23%) (**Figure 5**).
- Patients most commonly self-reported tiredness (58%), dizziness (48%), and shortness of breath (48%) at time of survey.

Figure 5. Symptoms experienced at treatment initiation and at most recent assessment/time of survey



Conclusions

- In this real-world US cohort, HCT control was poor, including 96% of patients receiving both CRT and phlebotomy, with patients experiencing symptom burden persistence.
- Patient-reported mild fatigue and time toxicity indicated multifactorial burden for phlebotomy-treated patients; most patients reported frequency of their phlebotomy to be burdensome by losing 3 to 4 hours out of their day per phlebotomy. Future research assessing the holistic burden of phlebotomy from the healthcare system and patient perspectives is warranted.
- Impact on all five domains of the EQ-5D-5L was notable, particularly pain/discomfort and anxiety, suggesting patient HRQoL to be a consideration in treatment decisions.
- These findings indicate a need for novel treatments that offer both HCT control and reduce burden for patients.

Limitations

- As physicians were asked to provide data on consecutively consulting patients who met the eligibility criteria, this study does not reflect a true random sample as patients were required to be alive and consulting with their physician at time of survey to be eligible for inclusion. Therefore, patients who consult with their physician more frequently were more likely to be included.
- Physicians and patients were requested to capture patient information retrospectively, which may have introduced recall bias (a common limitation of survey data). Despite this, physicians were able to refer to patient medical records during the survey, thus minimizing the possibility of recall bias.
- The timing of cytoreductive therapy initiation during the 12 months prior to the survey was unknown, limiting interpretation of treatment patterns.
- Due to the voluntary nature of the patient-reported survey, sample sizes are small, precluding statistical analysis from being conducted.

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Disclosures

LR reports advisory council or committee participation with Merck and Sobi; consulting fees from Blueprint Medicines, Cogent, Incyte, Merck, Novartis, Sobi, Takeda, PharmaEssentia, Sumitomo, and Dainippon; and institutional grants or other funding from Blueprint Medicines, Cogent, Incyte, Merck, Novartis, Protagonist, PharmaEssentia, Sumitomo, Disc Medicine, Telios, Kartos, Karyopharm, Geron, and Silence Therapeutics.

AF, AC, and LH are employees of Takeda. and report stock and other ownership interests in Takeda.

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Presented at Society of Hematologic Oncology (SOHO) Annual Meeting, Houston, Texas, USA, September 9-12, 2026. For questions or comments, please contact Dr Lindsay Rein: Lindsay.magura@duke.edu