

Patient-Reported Outcomes Data From REVEAL at the Time of Enrollment (Baseline): A Prospective Observational Study of Patients With Polycythemia Vera in the United States

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Abstract

Data from REVEAL (Prospective Observational Study of Patients With Polycythemia Vera in US Clinical Practices; n = 2309), the first study of its kind, confirm that many patients experience quality of life and work productivity impairments that might negatively affect their lives. In the future, longitudinal data from REVEAL will be important for evaluating how such burdens change over time.

Background: Patients with polycythemia vera (PV) often experience symptoms that adversely affect their quality of life (QoL). The ongoing, prospective, observational REVEAL (Prospective Observational Study of Patients With Polycythemia Vera in US Clinical Practices) study was designed to collect contemporary data regarding burden of disease, clinical management, patient-reported outcomes (PROs), and health care resource utilization from adult patients with PV in the United States. **Patients and Methods:** Data on PROs were collected at enrollment using the Myeloproliferative Neoplasm Symptom Assessment Form Total Symptom Score (MPN-SAF TSS; range, 0-100); the European Organization for Research and Treatment of Cancer—Core Quality of Life Questionnaire, version 3.0 (EORTC QLQ-C30; range, 0-100); and the Work Productivity and Activity Impairment Questionnaire—Specific Health Problem (WPAI-SHP; range, 0%-100%). **Results:** Among 2309 patients, mean (SD) disease duration was 5.8 (6.1) years and Charlson Comorbidity Index was 3.4 (0.8); 54.0% (1247/2309) were male. Mean (SD) MPN-SAF TSS was 18.8 (15.5). The most common symptoms were fatigue (80.1% [1844/2302]), early satiety (60.9% [1402/2302]), and inactivity (57.6% [1324/2302]). The most common severe symptoms were fatigue (16.8% [387/2302]), itching (13.4% [308/2302]), and inactivity (11.8% [271/2302]). The mean (SD) EORTC QLQ-C30 global health status/QoL score was 73.1 (23.2); mean functional subscale scores ranged from 80.5 (23.9) for cognitive functioning to 85.7 (24.6) for social functioning. The mean WPAI-SHP activity impairment score was 19.7% (n = 2300). Employed patients had mean WPAI-SHP scores for absenteeism, presenteeism, and overall work impairment of 3.2% (n = 810), 12.1% (n = 807), and 13.4% (n = 802), respectively. **Conclusion:** These data confirm that many patients with PV experience symptoms, QoL impairments, and work productivity impairments that negatively affect their lives. Longitudinal data from REVEAL will be important for evaluating how PROs change over time in these patients.

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Keywords: Activity impairment, Myeloproliferative neoplasm, Quality of life, Symptoms, Work productivity

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REVEAL Baseline PROs

Introduction

Polycythemia vera (PV) is a myeloproliferative neoplasm (MPN) primarily characterized by erythrocytosis and activating mutations in the Janus kinase 2 gene.¹ Patients with PV have an increased risk of mortality compared with age- and sex-matched individuals in the general population² and experience a broad range of symptoms that negatively affect their quality of life (QoL) and productivity.³⁻⁶ In the recent MPN Landmark survey of US patients with MPNs, fatigue was the most frequently reported symptom among patients with PV (73%), and 49% of the patients described their fatigue as “very severe.”⁵ Many patients also reported itching (55%), night sweats (45%), and concentration problems (36%). In addition to the various symptoms associated with PV, approximately 40% of patients experience splenomegaly that resulted in occasional discomfort and pain.⁷ Findings from an international prospective study of 1334 patients with PV (mean disease duration, 6.9 years)⁸ suggest that many patients experience suboptimal symptom control with traditional treatments.

Most patients with PV in the MPN Landmark cross-sectional survey reported feeling anxious or worried about their condition (78%) and that their PV symptoms reduced their QoL (66%).⁵ Many patients also reported interference with family or social life (63%), daily activities (48%), and normal work hours (37%). Most of the observational studies of patients with PV have been reported out of Europe^{3,6,9-11} or have been limited to single centers in the United States.^{12,13} Administrative claims databases and electronic medical records can be used to characterize patterns of care in clinical practice; however, these data sources frequently lack patient-reported outcomes (PROs) and can be limited in utility because of selection bias and missing values. Cross-sectional surveys such as the Landmark survey cannot characterize how PROs change over time.

With respect to the evolution of MPN-specific PROs, the Myeloproliferative Neoplasm Symptom Assessment Form (MPN-SAF) is a questionnaire that includes 17 MPN-related symptoms and a single question pertaining to overall QoL that was designed to assess the most impactful symptoms observed in patients with MPNs.^{3,6} Subsequently, on the basis of the evaluation of the MPN-SAF in clinical practice, it was concluded that an abbreviated version was necessary.³ Consequently, the MPN-SAF Total Symptom Score (TSS; also known as MPN10) was constructed and validated. This shorter questionnaire includes a question pertaining to the worst level of fatigue in the past 24 hours as well as the other 9 most characteristic and clinically significant MPN symptoms.³ Both instruments remain in use. For example, in the National Comprehensive Cancer Network’s clinical guidelines for MPNs, the MPN-SAF and the MPN-SAF TSS are currently recommended for the assessment of symptom burden at baseline and during the course of treatment, respectively.¹⁴

The ongoing REVEAL (Prospective Observational Study of Patients With Polycythemia Vera in US Clinical Practices; [ClinicalTrials.gov: NCT02252159](https://clinicaltrials.gov/ct2/show/study/NCT02252159)) was designed to collect contemporary data regarding the burden of disease, clinical management, PROs, and health care resource utilization of patients with PV in the United States. The objective of the present report was to characterize the PRO data from REVEAL at patient enrollment to assess the symptom burden, QoL impairments, and work productivity impairments associated with PV.

Table 1 Demographic and Clinical Characteristics at Enrollment

Characteristic	All Patients (n = 2309)
Mean (SD) Age, Years	66.1 (12.1)
Age Group, Years, n (%)	
18-34	28 (1.2)
35-59	618 (26.8)
60-74	1073 (46.5)
≥75	590 (25.6)
Sex, n (%)	
Male	1247 (54.0)
Female	1062 (46.0)
Disease Duration, Years	
Mean (SD)	5.8 (6.1)
Median (range)	4.1 (0-39.2)
Primary Employment Status at PV Diagnosis, n (%)	
Full-time	1121 (48.5)
Part-time	148 (6.4)
Retired	746 (32.3)
Homemaker	116 (5.0)
Unable to work/disabled	86 (3.7)
Other	46 (2.0)
Student	13 (0.6)
Unknown/missing	33 (1.4)
Employment Status at Enrollment, n (%)	
Full-time	669 (29.0)
Part-time	110 (4.8)
Retired	1176 (50.9)
Homemaker	80 (3.5)
Unable to work/disabled	101 (4.4)
Other	70 (3.0)
Student	6 (0.3)
Unknown/missing	97 (4.2)
PV Risk Status, n (%)	
Low	528 (22.9)
High ^b	1781 (77.1)
Mean (SD) Charlson Comorbidity Index	3.4 (0.8)
Management of PV, n (%)	
Watchful Waiting	123 (5.3)
Phlebotomy Only (With or Without Aspirin)	787 (34.1)
Hydroxyurea (With or Without Aspirin)	661 (28.6)
Hydroxyurea With Phlebotomy (With or Without Aspirin)	550 (23.8)
Other	186 (8.1)
Missing	2 (0.1)

Abbreviation: PV = polycythemia vera.

^aAll patients with age and sex information enrolled on or before May 18, 2017, and with at least 1 nonmissing patient-reported outcome at enrollment.

^bHigh-risk PV defined as patients with a history of thrombotic events and/or patients 60 years of age or older.

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