MYELOPROLIFERATIVE NEOPLASMS (MPNs) LANDMARK SURVEY

Essential Thrombocythemia (ET) Executive Summary Report

TABLE OF CONTENTS

TABLE OF CONTENTS (CONT.)

INTRODUCTION TO THE MPN LANDMARK SURVEY

MPN LANDMARK SURVEY Background

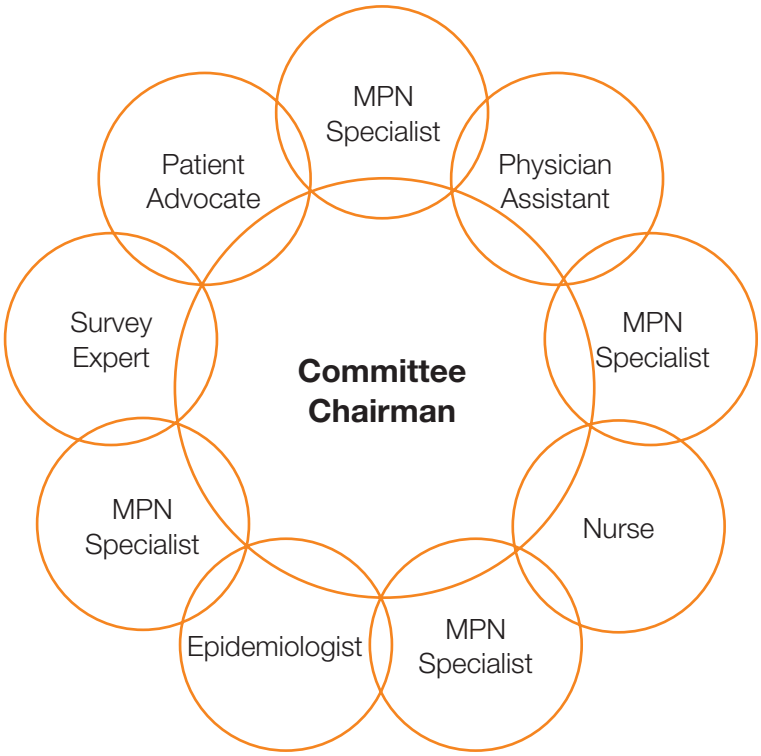
The LANDMARK SURVEY OF MYELOPROLIFERATIVE NEOPLASMS (MPNs) was developed to provide the first detailed, comprehensive picture of patient and physician experiences related to MPNs, including myelofibrosis (MF), polycythemia vera (PV), and essential thrombocythemia (ET), in the United States.

This large survey was developed to evaluate the patient's disease burden and patient–physician communication in MPN disease setting. It specifically examines the domains of symptomatology, disease burden, quality of life (QOL), timing to diagnosis, symptom recognition, activities of daily living, productivity, symptom assessment, prognostic assessment, treatment management attitudes, and physician–patient communication. To enhance patient care, it is important to have a current and clear understanding of how MPNs affect the health and daily lives of patients.

Steering Committee

The MPN LANDMARK SURVEY was developed in collaboration with an external steering committee that consisted of diverse MPN experts/thought leaders including MPN specialists, allied health providers, patient advocacy group members, epidemiologists, and survey experts. Serving as members of the Steering Committee were:

- Ruben Mesa, MD—*Committee Chairman*
- John Boyle, PhD
- Salman Fazal, MD
- Christopher C. Gayer, PhD
- Sara Goldberger, BA, MS
- Xiaomei Ma, PhD
- James Mangan, MD, PhD
- John O. Mascarenhas, MD, MS
- Carole B. Miller, MD
- Maureen Thyne, PA
- Wendy Wilson, RN, MSN, OCN



MPN LANDMARK SURVEY DESIGN

Patient Survey

A national sample of patients diagnosed with MF, PV, or ET was recruited from multiple sources to participate in an online survey conducted from May through July of 2014. Invitations were delivered by direct mail to consumers who opted in to receive health care related information. A digital recruiting campaign for the survey was conducted online during the 6 weeks of survey fielding. To supplement the online campaign, a newspaper campaign was conducted in 5 major markets. As the multichannel recruitment campaign was taking place, 1500 patient recruitment invitations were distributed through specialists treating patients with MPNs. Patient data were collected using a web-based survey tool. The survey was conducted only in English and averaged approximately 22 minutes in length. Patients received no remunerations for their participation. A total of 813 patients participated, including 207 surveys with MF patients, 380 surveys with PV patients, and 226 surveys with ET patients.

Population	Interview Dates	Completed Sample
Men and women aged ≥18 y	May 16, 2014–July 13, 2014	▪ 207 MF patients ▪ 380 PV patients ▪ 226 ET patients

Physician Survey

The physician survey was designed as a national probability sample of specialists treating patients with MPNs, using double-blind selection. A series of random samples were drawn from the American Medical Association/American Osteopathic Association listings of physicians in direct patient care with a primary medical specialty of hematology, oncology, or hematology/oncology. Medical residents and interns were excluded. The initial specialists were sent advance letters explaining the survey's purpose, survey length, participation requirements, and remuneration for completing the survey. Subsequent specialists were sent e-mail and fax invitations. The physician data were collected online using a web-based survey instrument designed in parallel with the patient questionnaire. The requirement for physician participation was to have at least 2 patients with MF, 5 patients with PV, or 5 patients with ET in the past 12 months. Consequently, 1 national sample of physicians yielded 3 independent subsamples of physicians treating each of the 3 targeted conditions. The physician survey was conducted only in English and averaged approximately 22 minutes in length. Physicians received remuneration of $185 for their participation. A total of 457 physician surveys were completed, including 156 surveys with physicians treating MF patients, 250 surveys with physicians treating PV patients, and 51 surveys with physicians treating ET patients.

Population	Interview Dates	Completed Sample
Physicians ▪ Hermatologists ▪ Medical oncologists ▪ Hematologists/Oncologists	May 21, 2014–July 13, 2014	▪ 156 MF physicians ▪ 250 PV physicians ▪ 51 ET physicians

ET LANDMARK SURVEY RESULTS

ET Background

Essential thrombocythemia (ET) is a specific type of MPN. ET is a a disease of the bone marrow and also known as a bone marrow cancer or a blood cancer. ET causes the bone marrow to make too many platelets and is considered to be a serious, chronic condition that develops slowly over time; however, with proper treatment it can be successfully managed long-term.[1] Approximately 71,000 to 88,000 people in the United States have been diagnosed with ET.[2] It may occur at any age in life, but it typically presents later in life, around 60 years of age and older.[1-3]

Many people with ET have no signs or symptoms. ET begins with 1 or more acquired changes (mutations) to the DNA of a single blood-forming cell, which results in the overproduction of blood cells, especially platelets, in the bone marrow. Signs and symptoms of ET may include:[1]

- Headache
- Dizziness or lightheadedness
- Burning or throbbing pain in hands and feet
- Weakness
- Fatigue
- Abnormal clotting (thrombosis)
- Numbness or tingling in hands/feet
- Mildly enlarged spleen

It should be noted, this executive summary does not include a full report of the data collected from the ET LANDMARK SURVEY. A complete list of patient survey questions and physician survey questions can be found at the end of this report in the Appendix. On request, data can be provided for any question that was not included within the context of this report. Throughout the report, individual values are rounded and may not total 100%.

Key Insights from the ET LANDMARK SURVEY

Most ET patients have significant symptom burden from their disease, and most patients agreed that symptoms reduced their QOL and have had an impact on employment.

- 96% of patients surveyed reported having experienced >1 symptom. The most common symptoms reported were fatigue or tiredness, bruising, and numbness or tingling in the hands or feet (Figure 1).
- The most severe symptoms reported by patients were fatigue or tiredness, problems with sexual desire, inactivity, weakness, and muscle aches (Figure 2).
- Half of ET patients (51%) surveyed reported that ET caused them at least some physical, emotional, or financial hardship in the past month (Figure 7).
- Most patients reported that they have felt “anxious or worried” (74%) and “depressed or discouraged” (59%) about ET in the past month (Figure 5).
- Nearly a third (30%) of those who were working after their diagnosis reported that they had to reduce hours at work as a result of their ET, and 25% of patients said they voluntarily terminated their job or took early retirement (Figure 10).
- Most patients (57%) strongly agreed or somewhat agreed that ET symptoms reduced their QOL (Figure 15).
- Prevention of vascular/thrombotic events was the most common treatment management goal selected by more than one third (34%) of ET patients (Figure 28).
- Of those patients who relied on a caregiver (15%) to help with their condition, most (71%) said that their main caregiver was a spouse or partner (Figures 69 and 70).
- Nearly all of the ET patients in the survey (99%) reported that they had some sort of health insurance to cover their health care costs (Figure 74).

Most patients reported being very satisfied with their communication and relationship with health care providers (HCPs).

- Most ET patients (84%) were satisfied with their current physician’s communication about their condition (Figure 56).
- A Nurse Practitioner or Physician Assistant (30%) were the most commonly reported HCPs aside from their current physician in their care (Figure 68).
- Patients preferred to receive educational materials from the Internet (88%), online email discussion groups (54%), and their doctor’s office (20%) (Figure 71). Patients most frequently went to MPN Advocacy Group web sites (74%), hospital web sites (65%), and health web sites (55%) on the Internet (Figure 72).

Although most patients reported a very positive relationship with their physician, there were some gaps in the comparisons.

- Most physicians (84%) agreed even mild to moderate symptoms can have a significant impact on an ET patient’s QOL (Figure 34). Physicians recommended drug treatment in more than half of patients (54%) with moderate symptoms; however, they rarely (22%) recommended drug treatment to patients with mild symptoms (Figure 35).
- When comparing symptom assessment completion in the office, nearly half of ET patients surveyed (44%) stated that physicians just proactively asked how they were feeling, whereas most physicians (55%) reported that they asked about the most important symptoms (Figures 39 and 40).
- Among ET patients, 35% disagreed that their physician created a treatment plan despite 94% of physicians reporting that they had created a plan or established goals for their patients (Figures 62 and 63).

ET LANDMARK SURVEY DEMOGRAPHICS

Patient Demographics

The mean age of ET patients in the survey was 61 years of age. Almost half of patients (46%) in this sample were aged 60–74 years. A little more than a third of the patients (33%) were aged 45–59 years. Only 12% of ET patients surveyed were aged 75 years and older and another 10% were under the age of 45 years. Most patients in the survey were women (72%).

Surveyed patients diagnosed with ET were less likely to be minority, lower income, or lower educational attainment than the general population. Almost all ET patients (98%) reported their race as white. More than half of patients had a college degree or higher (58%). Additionally, 56% of patients had a household income of more than $75,000, and the reported median yearly income was $87,500.

Please note, although the survey respondents were more highly educated and had a higher income than the general population, because there were no data describing income and educational attainment among people with MPNs, we cannot say whether the respondent sample for these domains is representative of all people with MPNs. Responses across the education and income categories were compared, and no significant differences or trends were seen in either group.

Demographics of Survey Respondents

Demographic Characteristic	% of Respondents
Age Range, y	
<18 or >90	**0**
18–44	**10**
45–59	**33**
60–74	**46**
75–90	**12**
Sex	
Men	**28**
Women	**72**
Education Level	
Some high school	**0**
High school graduate	**9**
Technical post-secondary	**5**
Some college	**28**
4-year college graduate	**26**
Postgraduate degree	**32**
Household Income, US $	
$15,000 or less	**4**
$15,001–$25,000	**4**
$25,001–$35,000	**5**
$35,001–$50,000	**10**
$50,001–$75,000	**16**
$75,001–$100,000	**26**
>$100,000	**30**
Don't know	**6**

Table 1. Note: Individual values are rounded and may not total 100%

Physician Demographics

An overwhelming majority of physicians within this sample identified themselves as hematologists/oncologists (90%) and reported that hematology cases comprise 26% to 75% of their practice.

Most ET-treating physicians (73%) surveyed graduated from medical school in 1990 or later. Only 14% graduated from medical school between 1980–1989, while another 14% graduated before 1980. Overall, the mean length of time since graduation for physicians in this survey was 20 years.

Physicians most often saw their patients in a single specialty group setting (43%) or within academic hospitals (33%). Less commonly, physicians saw their patients in multispecialty group settings (16%), and even smaller proportions of ET physicians saw patients in community hospitals (6%) or solo practice (2%).

Most physicians surveyed saw more than 200 outpatients per month (53%), with a mean number of 252 outpatients seen per month. Specific to ET, more than a third of the physicians (37%) surveyed reported more than 16 ET patients currently under their care. The average number of ET patients seen by physicians surveyed represented about 8% of their total reported outpatient visits. Physicians reported seeing an average of 11 newly diagnosed ET patients within the last 12 months.

Physician Demographics

Demographic Characteristic	% of Respondents
Year of Graduation From Medical School	
1960–1969	**6**
1970–1979	**8**
1980–1989	**14**
1990–1999	**25**
2000–2009	**47**
2010+	**0**
Percentage of Hematology Cases in Practice	
0–25%	**6**
26–50%	**49**
51–75%	**33**
76–100%	**12**
Practice Setting Physicians Spend at Patient Care	
Community hospital-inpatient	**0**
Academic hospital-inpatient	**0**
Other	**0**
Single specialty group	**43**
Academic hospital-outpatient	**33**
Community hospital-outpatient	**6**
Multispecialty group/HMO	**16**
Solo practice	**2**
Total No. of ET Patients Under Physician's Care	
1–3	**0**
4–6	**16**
7–10	**24**
11–20	**39**
>20	**22**

Table 2. Note: Individual values are rounded and may not total 100%

Symptom Burden

Survey findings showed that ET had a high symptom burden among patients.

Patients surveyed were asked whether they had ever experienced each of 27 symptoms associated with ET. This question was based on the MPN Symptom Assessment Form (MPN-SAF), a validated instrument used to assess patient-reported MPN symptoms.[4] On average, patients reported 8 symptoms with some reporting as many as 23 symptoms. Overall, 96% of patients surveyed reported having experienced at least 1 symptom.

The most common symptoms reported by ET patients were fatigue or tiredness (71%), bruising (52%), and numbness or tingling in the hands or feet (50%) (Figure 1).

Symptoms Ever Experienced by ET Patients

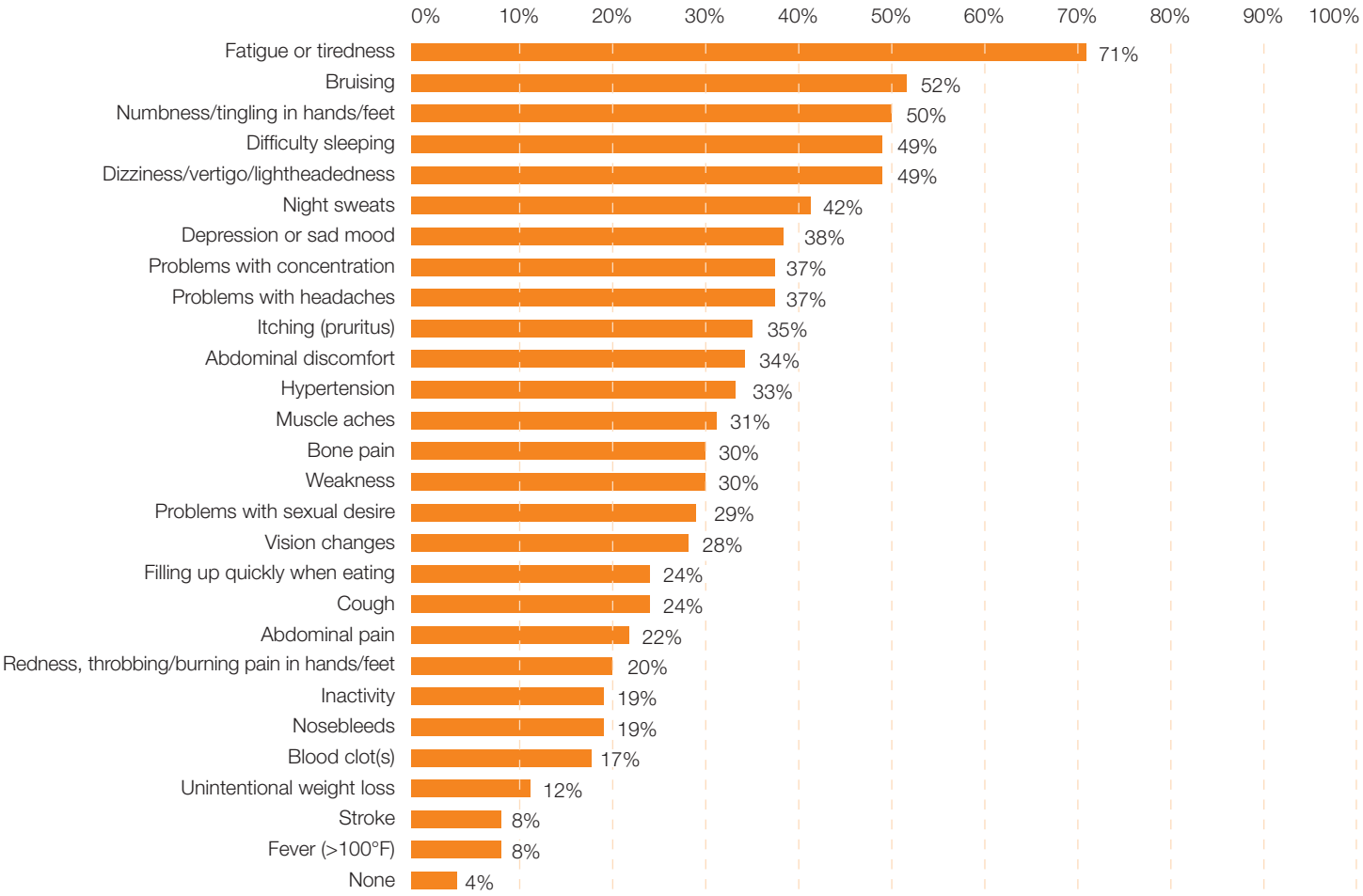


Figure 1. Question 8: Have you ever had any of the following symptoms? (n = 226)

Symptom Severity

Patients were asked to rate the severity of each symptom they had ever experienced on a scale from 0 (absent) to 10 (worst imaginable) in the past 12 months. This question was based on the MPN-SAF. An overall mean severity score was calculated for each symptom from the individual patient rankings (Figure 2).

The most severe symptoms reported by patients were fatigue or tiredness (6.0), problems with sexual desire (5.9), inactivity (5.4), weakness (5.4), and muscle aches (5.3). Although problems with sexual desire, inactivity, and weakness were among the most severe symptoms, they were not among the most common of symptoms, with less than a third of ET patients having experienced those symptoms (Figure 1).

Patient-Reported MPN-SAF Mean Severity Score

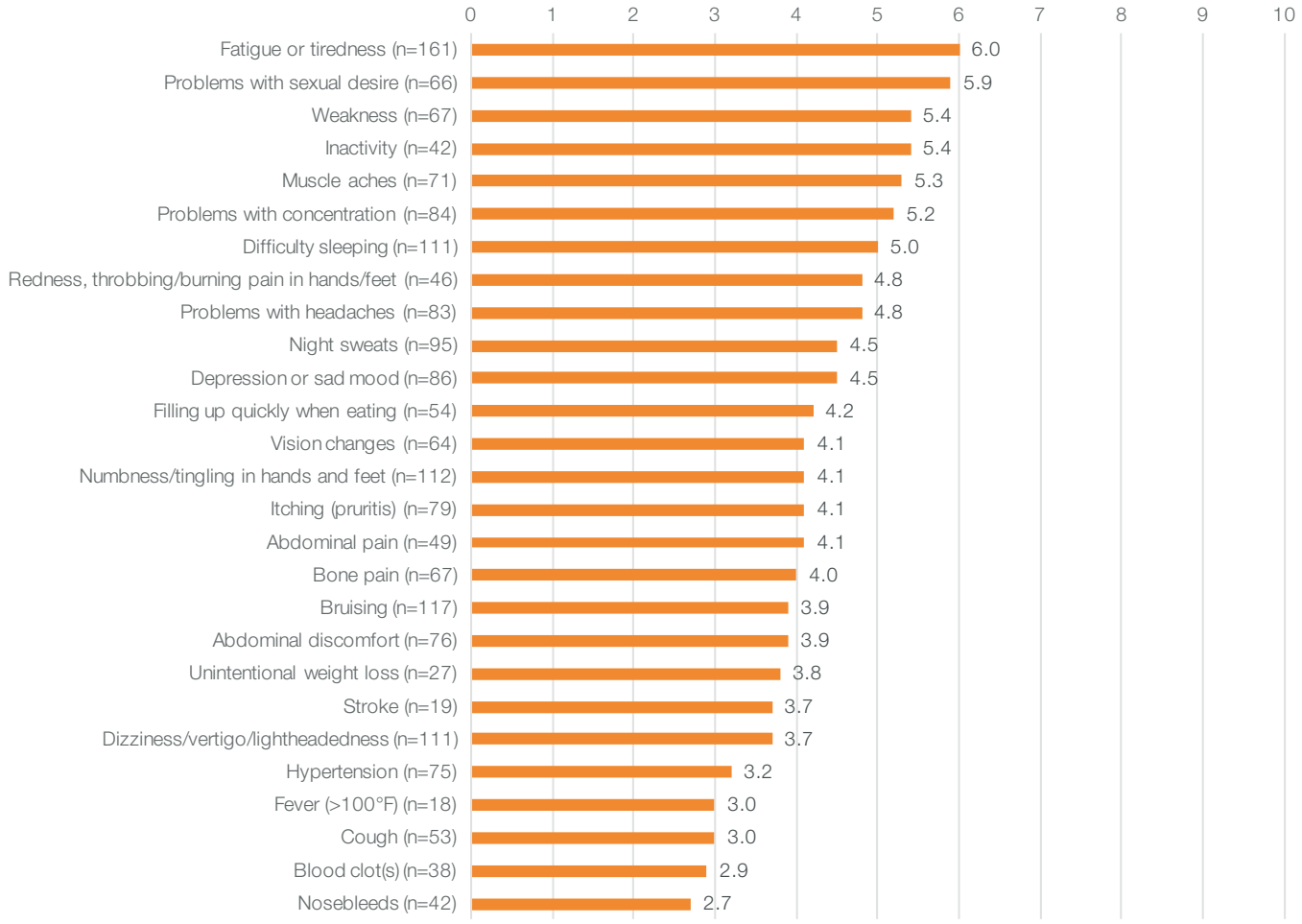


Figure 2. Question 13: How severe is [symptom]?

Bleeding-Related Symptoms

In ET, because blood volume expands and hyperviscosity develops, patients are more prone to thrombosis or bleeding events such as stroke, transient ischemic attacks, deep vein thrombosis, and heart attack.[3] Eighty-four percent of patients who reported having blood clots felt that the clots were the result of ET. Additionally, 79% of patients who reported the symptom of stroke felt it was related to their condition. In addition to major bleeding events, ET patients may be more prone to bleeding resulting in bruising and or nosebleeds.[3] Of patients who reported bruising or nosebleeds, 76% and 69%, respectively, felt these symptoms were the result of their ET.

Symptom Resolution

Patients surveyed were asked to select the symptom they would most like to resolve from the symptoms they were currently experiencing. A third of patients reported fatigue (33%) as the symptom they would most like to resolve followed by hypertension (7%) and numbness in hands and feet (6%) (Figure 3).

First Symptom ET Patients Would Like to Resolve as Reported by Patients

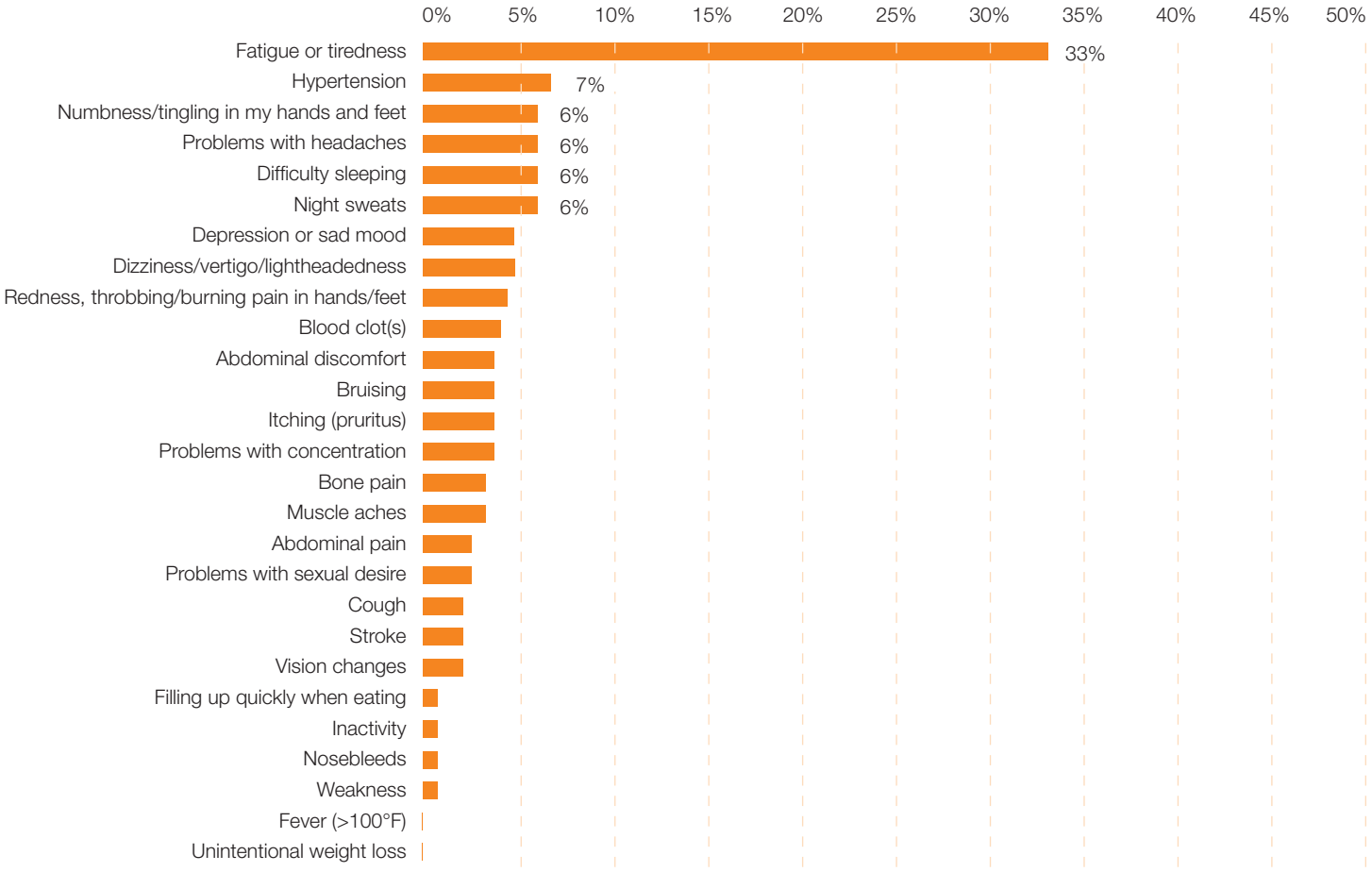


Figure 3. Question 14: Of the symptoms that you are currently experiencing, which one would you most like to resolve? (n = 218)
Note: Labels for data under 5% are not displayed

Symptom Recognition

More than half of the 27 ET symptoms listed were recognized as being the result of ET by most patients. Redness and throbbing/burning pain in hands or feet (85%), blood clot(s) (84%), fatigue or tiredness (81%), and stroke (79%) were the most recognized symptoms associated with ET by patients who reported experiencing that symptom in a previous question (Figure 4). Problems with sexual desire, one of the most severe symptoms experienced by patients, was recognized as being the result of ET by only 18% of patients who reported that symptom (Figures 2 and 4).

Symptoms Associated With ET Recognized by Patients

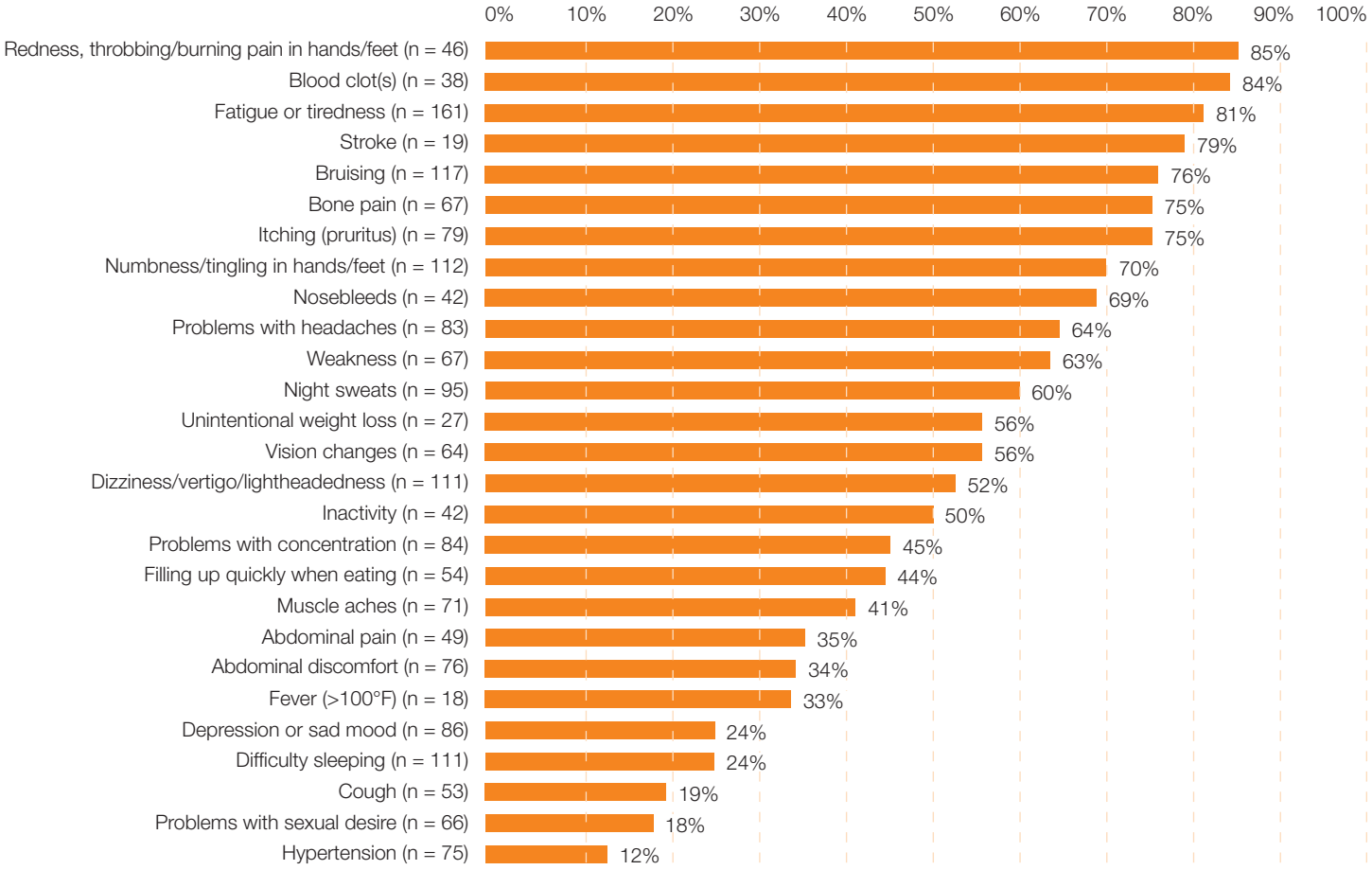


Figure 4. Question 12: Which of these symptoms do you feel are the result of your diagnosis?

Emotional Burden

The survey results showed that ET carries an emotional burden for many patients. Survey participants were asked to rate the extent to which ET has had an impact on them in the past month in a number of emotional or psychosocial areas.

Most ET patients (74%) reported that their condition had at least some impact on them (a rating between 2 and 5 on a scale of 1–5) in the past month, as it caused them to feel anxious or worried about their condition (Figure 5).

Nearly two thirds of patients (59%) felt depressed or discouraged to at least some extent. Close to half of patients surveyed (43%) reported having trouble coping with the stress of living with ET.

Emotional Impact of ET Reported by Patients

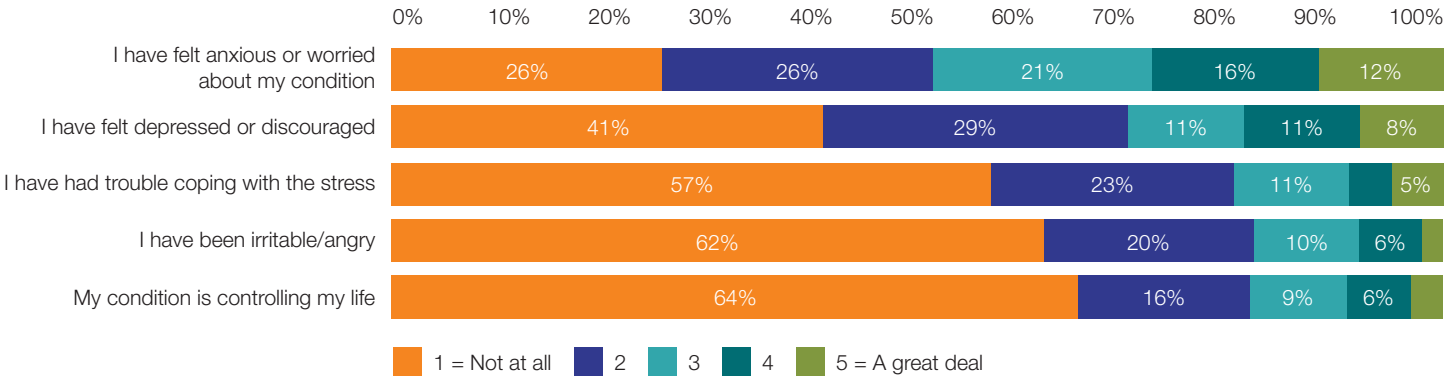


Figure 5. Question 24: Rank the following statements as they have occurred during the past month, as a result of your ET (n = 226)
Note: Labels for data under 5% are not displayed

Physical Burden

Most ET patients report that their ET condition had a physical impact on their life in the past month. Nearly half of patients surveyed reported their condition caused trouble focusing on things (48%) or caused their sleeping habits to change (47%) to some extent in the past month. Additionally, 35% of patients reported that ET caused a changed in appetite, and nearly a third (32%) reported that ET caused a change in their appearance (Figure 6).

Physical Impact of ET Reported by Patients

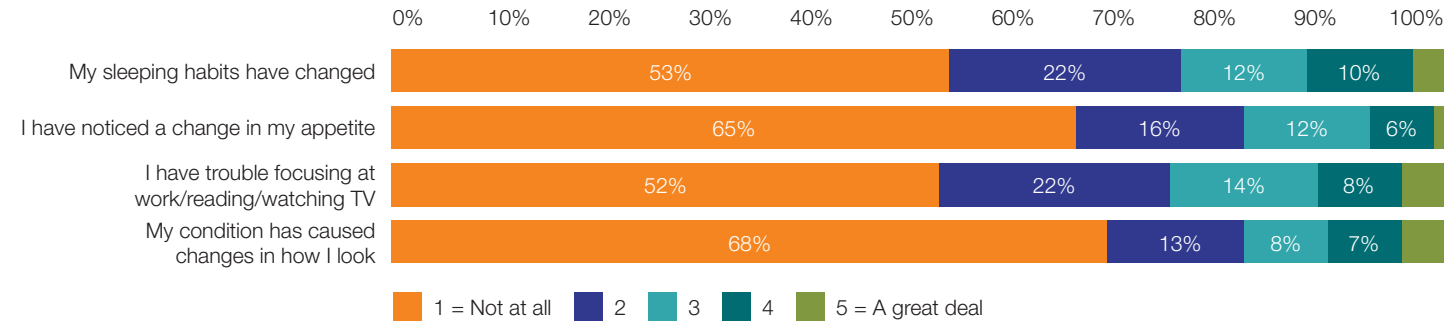


Figure 6. Question 24: Rank the following statements as they have occurred during the past month, as a result of your ET (n = 226)
Note: Labels for data under 5% are not displayed

Hardship Caused by ET

Half of patients (51%) surveyed reported that ET caused them at least some physical, emotional, and financial hardship in the past month (Figure 7), and 9% of patients reported that their condition caused a great deal of hardship in the past month.

Physical, Emotional, and Financial Hardship Caused by ET as Reported by Patients

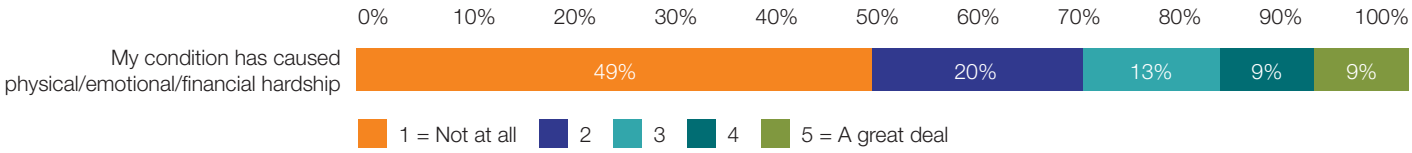


Figure 7. Question 24: Rank the following statements as they have occurred during the past month, as a result of your ET (n = 226)

ET Interference With Patient Daily Activities

Survey findings showed that ET interferes with a patient's daily activities. Most patients reported that their condition caused at least some interference in their family or social life (55%) and that pain and discomfort were limiting their activities (45%) and sex life (42%) (Figure 8).

Some ET patients reported that their condition caused a great deal of interference in their family or social life (8%) and their daily activities (7%) (Figure 8).

ET Interference With Patient Daily Activities

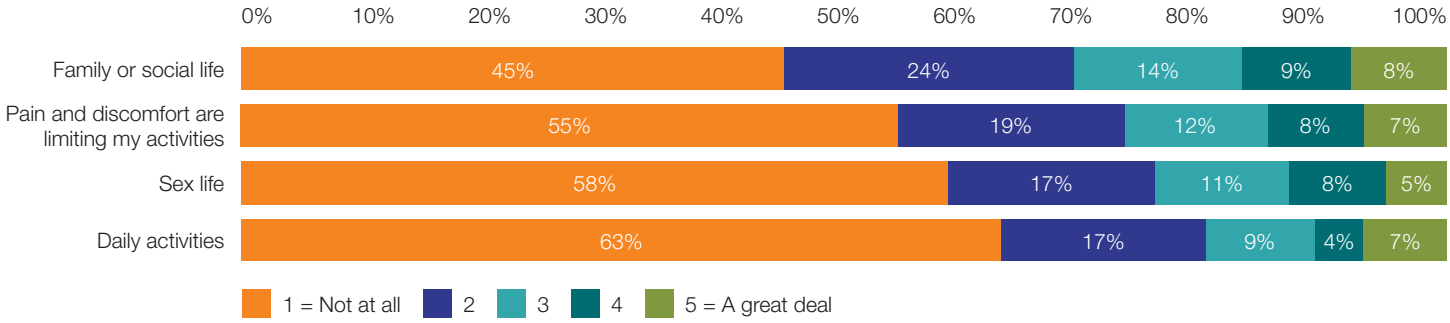


Figure 8. Question 25: To what extent does ET interfere with the following (n = 226)

Impact on Employment

The survey findings showed that ET had an impact on the employment and productivity of patients when they were in the workforce.

Close to one third of patients (30%) were working full-time, 9% were working part-time, and 8% were self-employed. More than a third of patients (39%) reported that they were retired (Figure 9).

Patients were also asked questions regarding the impact of ET on their employment status. Please note that those patients who reported "not applicable" to question 21 were excluded in the following percentages and chart below (Figure 10).

Close to a third of those who were working after their diagnosis reported that they reduced hours at work (30%) as a result of their ET. Additionally, 14% of patients said that they took early retirement as a result of their condition (Figure 10).

Patient Current Employment Status

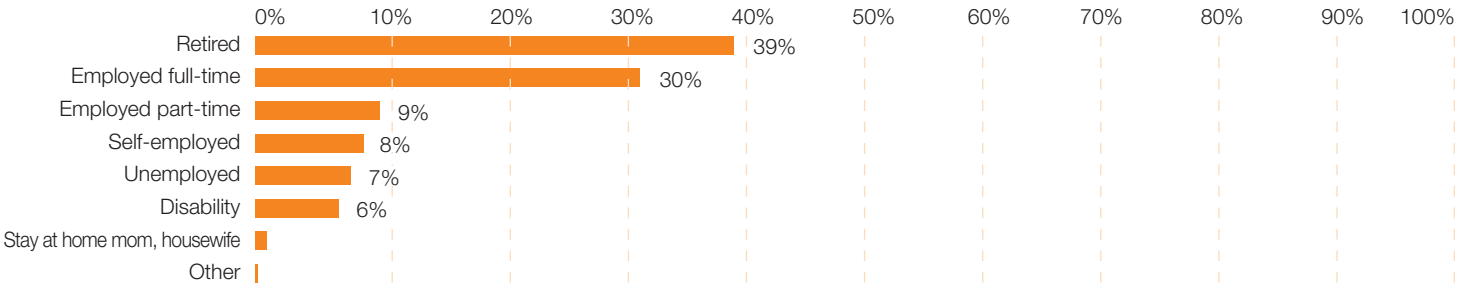


Figure 9. Question 22: What is your current employment status? (n = 226)
Note: Labels for data under 5% are not displayed

ET Impact on Employment Status

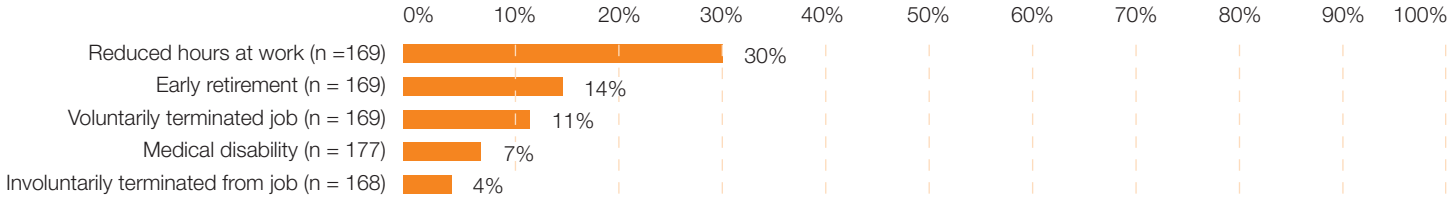


Figure 10. Question 21: As a result of your diagnosis have you ever…?
Note: Data excludes "Not applicable" responses

Impact on Productivity

ET not only had an impact on productivity for those in the workforce, it also had an impact on a patient's planned activities outside of work. To better understand the impact of ET on a patient's social life, patients were asked how many days of the last 30 days had they missed work or cancelled any scheduled activities. They were also asked how many days were spent in bed most or all of the day because of ET.

Most patients did not report any sick days, missed or canceled activities, or days spent in bed. However, 23% of patients reported taking 1 to 7 sick days from work, and 28% of patients reported canceling a planned activity at least once in that last month because of ET (Figures 11 and 12). Twenty-five percent of patients reported spending at least a day or most of the day in bed in the last 30 days as a result of ET (Figure 13). Of those ET patients who missed at least 1 day in the last 30 days, the average was 2 sick days, 4 days of canceled activities, and 6 days spent in bed.

Impact on Productivity

Sick Days From Work

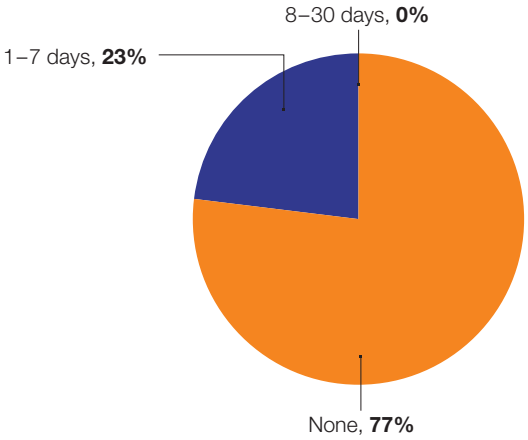


Figure 11. Question 23: In the last 30 days, how many days did you have to call in sick as a result of your diagnosis symptoms? (n = 88)
Note: Individual values are rounded and may not total 100%

Canceled Activities

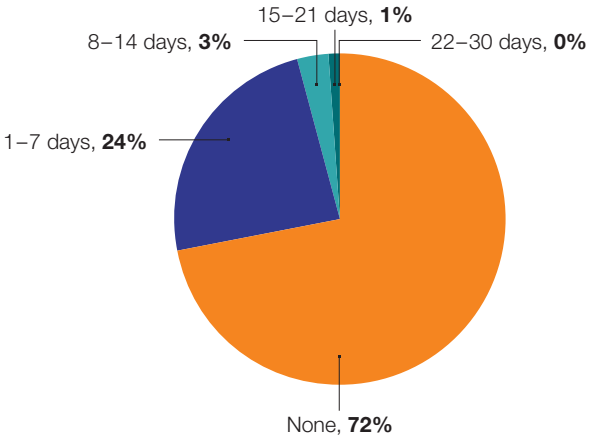


Figure 12. Question 17: In the last 30 days, how many days did you have to cancel planned or scheduled activities as a result of your diagnosis? (n = 226)
Note: Individual values are rounded and may not total 100%

Days Spent in Bed

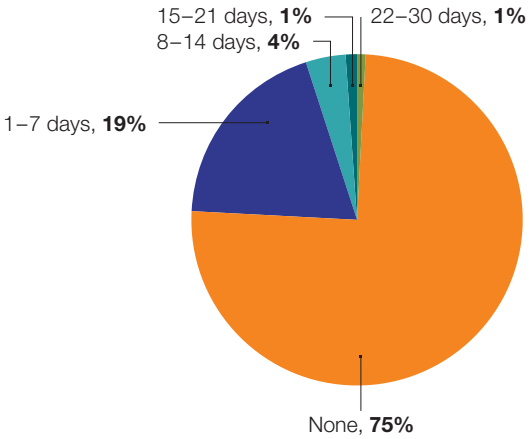


Figure 13. Question 18: In the last 30 days, how many days did you stay in bed all or most of the day as a result of your diagnosis? (n = 226)
Note: Individual values are rounded and may not total 100%

Current State of Health

Patients surveyed were asked to describe their current state of health at the time of survey administration. More than a third of patients described their current state of health as "Good" (34%); 32% of patients described their current state of health as "Very Good," and another 12% reported it as "Excellent." (Figure 14)

On the opposite end of the spectrum, 18% of patients reported their current state of health as "Fair." A small portion of patients (4%) reported their current state of health as "Poor".

Current State of Health as Reported by ET Patients

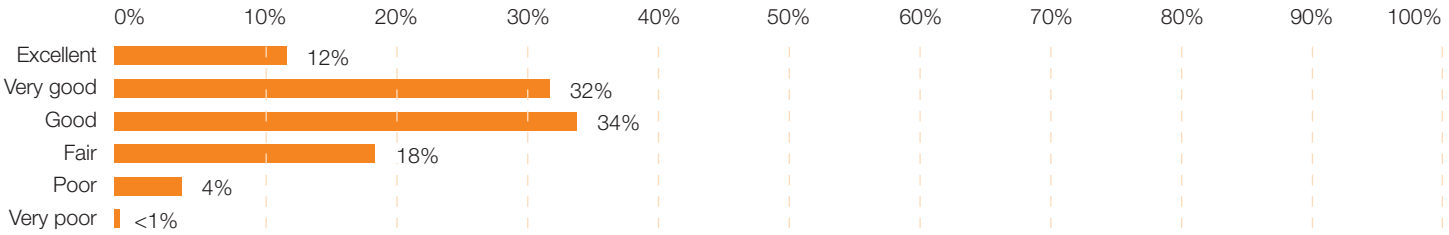


Figure 14. Question 7: How would you describe your current state of health? (n = 226)

Impact on QOL

Most patients agreed that ET symptoms reduced their QOL (57%). In fact, one fifth of ET patients (20%) strongly agreed that ET symptoms reduced their QOL, and another 36% of patients somewhat agreed with this statement (Figure 15).

ET Symptoms Reduce QOL Reported by Patients

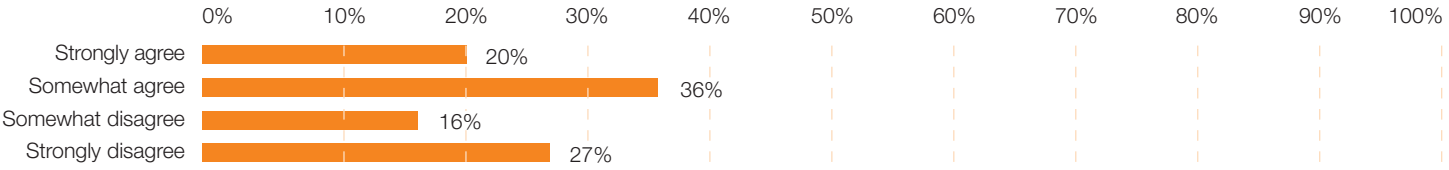


Figure 15. Question 35a: Please indicate whether you agree/disagree with the following statement... (n = 226)

Summary of Findings

- Overall, 96% of patients surveyed reported having experienced at least 1 symptom (Figure 1).
- The most common symptoms reported by ET patients were fatigue or tiredness (71%), bruising (52%), and numbness or tingling in the hands or feet (50%) (Figure 1).
- The most severe symptoms reported by patients were fatigue or tiredness (6.0), problems with sexual desire (5.9), inactivity (5.4), weakness (5.4), and muscle aches (5.3) (Figure 2).
- A third of patients reported fatigue (33%) as the symptom they would most like to resolve followed by hypertension (7%) and numbness/tingling in hands and feet (6%) (Figure 3).
- Redness and throbbing/burning pain in hands or feet (85%), blood clot(s) (84%), fatigue or tiredness (81%), and stroke (79%) were the most recognized symptoms associated with ET by patients who reported experiencing that symptom (Figure 4).
- Most patients reported that they have felt “anxious or worried” (74%) and “depressed or discouraged” (59%) about ET in the past month (Figure 5).
- Most patients reported that their condition caused them trouble focusing (48%) or caused their sleeping habits to change (47%) to some extent in the past month (Figure 6).
- Half of ET patients (51%) surveyed reported that ET caused them at least some physical, emotional, and financial hardship in the past month (Figure 7).
- Most patients reported that ET caused at least some interference in their family or social life (55%) and that pain and discomfort were limiting their activities (45%) (Figure 8).
- Nearly one third (30%) were employed full-time, 9% were employed part-time, and 8% were self-employed (Figure 9).
- Nearly a third (30%) of those who were working after their diagnosis reported that they had to reduce hours at work as a result of their ET, and 25% of patients said they voluntarily terminated their job or took early retirement (Figure 10).
- Nearly a third of ET patients (28%) reported canceling a planned activity as a result of ET; 25% of patients reported spending all or most of the day in bed, and 23% called in sick to work at least 1 day of the last 30 days (Figures 11–13).
- More than a third (34%) of patients described their current state of health as “Good” (Figure 14).
- Most patients (57%) agreed that ET symptoms reduced their QOL (Figure 15).

Timing of Diagnosis

ET patients were asked to recall the age and year in which they were first diagnosed. Most patients had been diagnosed for as many as 10 years (70%), and 40% of patients were diagnosed between the ages of 45–59 years (Figures 16 and 17). The median number of years since diagnosis for patients surveyed was 7 years, and the average age at diagnosis was 51 years.

Number of Years Since Diagnosis

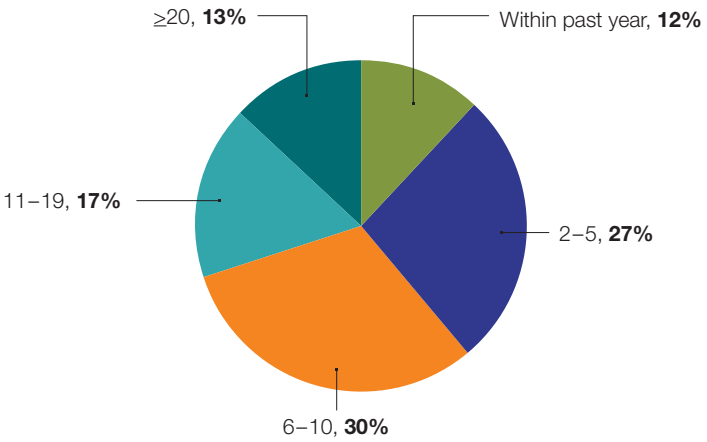


Figure 16. Question 3B_YEAR: In what year were you first diagnosed with essential thrombocythemia? (n = 187)
Note: Individual values are rounded and may not total 100%

Age Range at Time of Diagnosis

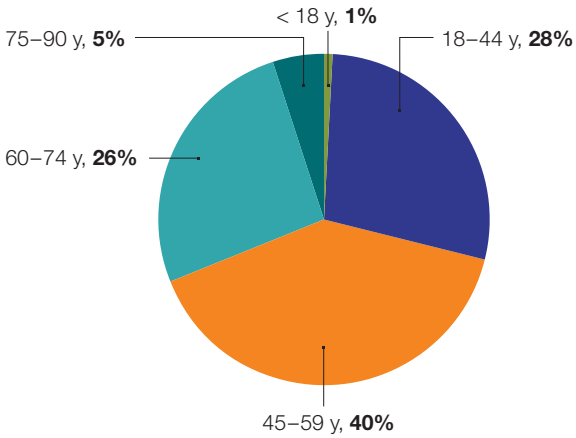


Figure 17. Question 3B: At what age were you first diagnosed with essential thrombocythemia? (n = 184)
Note: Individual values are rounded and may not total 100%

Time from Symptoms to Diagnosis

Patients were asked how long before their ET diagnosis, had they experienced each of their reported symptoms. The time from specific symptoms to diagnosis varied by symptom. However, when looking at all the symptoms, there was a span of as many as 2 years between onset and diagnosis in 31% of ET patients. Furthermore, 61% of patients experienced symptoms for more than 2 years before they received a formal diagnosis (Figure 18).

Time From Symptoms Overall to ET Diagnosis

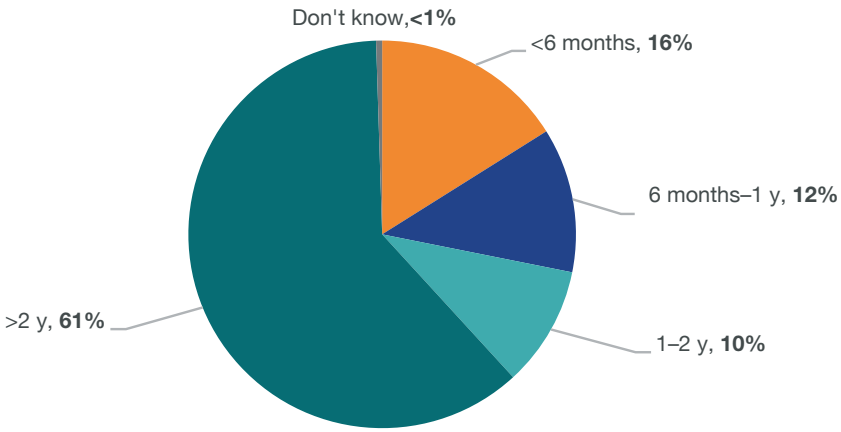


Figure 18. Question 10a-dd: How long before you were diagnosed did you first experience [symptom]? (n = 226)
Note: Individual values are rounded and may not total 100%

Symptoms at Time of Diagnosis

Fatigue was the most common symptom at the time of diagnosis, reported by 38% of ET patients who reported symptoms (Figure 19). Patients also mentioned they experienced problems with headaches (23%), dizziness/vertigo/lightheadedness (20%), numbness/tingling in their hands and feet (19%), and hypertension (17%). Sixteen percent of patients reported no symptoms at time of diagnosis. Regarding spleen-related symptoms experienced by patients at time of diagnosis, 6% of patients reported abdominal pain, 9% reported abdominal discomfort, and filling up quickly when eating was reported by 6% of patients.

Symptoms at Time of Diagnosis

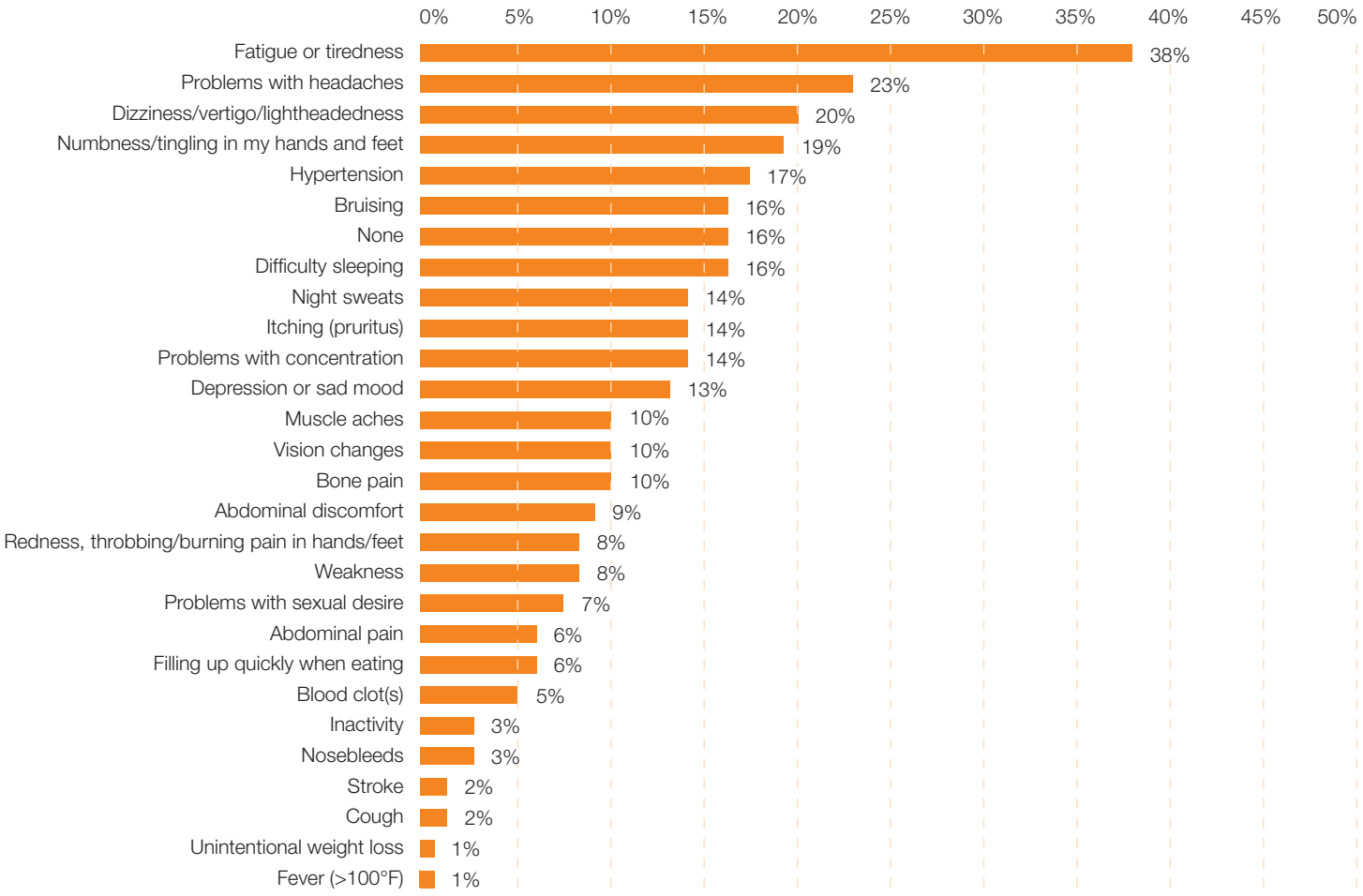


Figure 19. Question 9: Which of these symptoms were you experiencing at time of diagnosis? (n = 218)

Other Diagnosed Conditions

ET patients were asked to select other conditions they were currently managing in addition to ET, also known as comorbid conditions. Most patients (63%) reported no other conditions (Figure 20). Thirty-seven percent of patients reported conditions that included moderate to severe kidney disease (5%) and heart attack (myocardial infarction) (4%).

Comorbidities as Reported by ET Patients

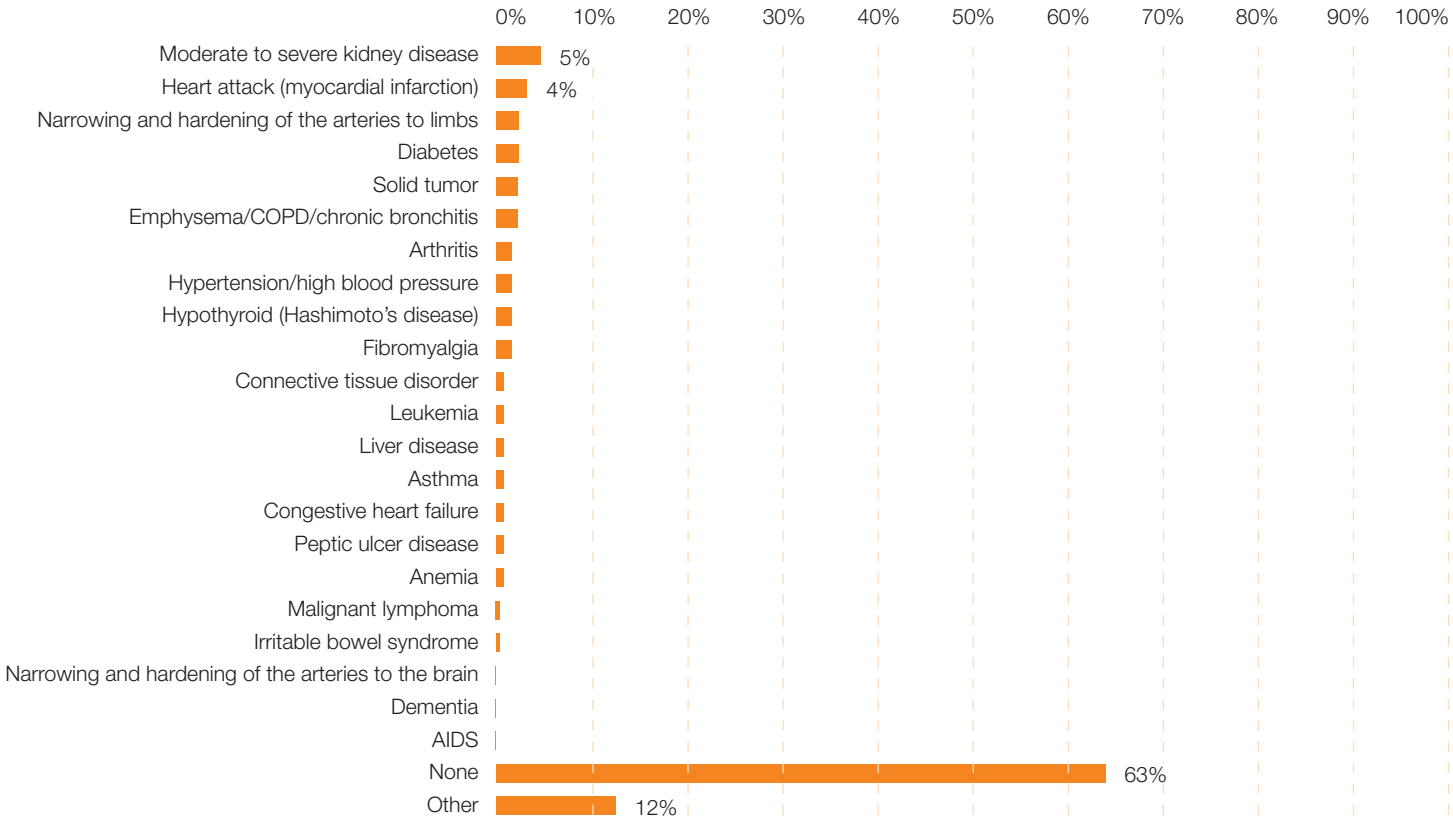


Figure 20. Question 19: Do you have any of the following conditions? (n = 226)
Note: Labels for data under 4% are not displayed

Patient Bleeding History

ET is associated with an increased risk of thrombotic events.[2] Most surveyed patients (65%) reported a history of bleeding before being diagnosed with ET (Figure 21).

Easy bruising was most commonly experienced by more than one third of patients (34%) followed by heavy menstrual periods (28%) and nosebleeds (14%).

Patient-Reported Bleeding History Prior to ET Diagnosis

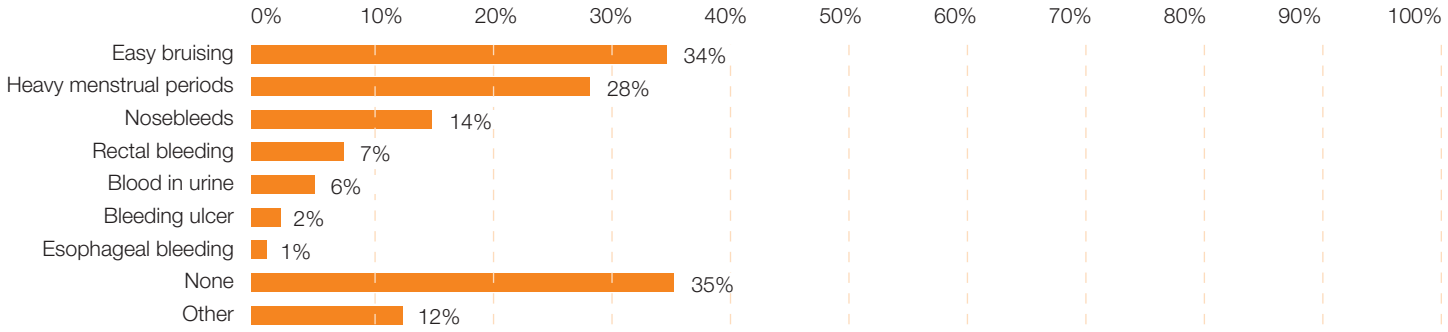


Figure 21. Question 26: Did you ever suffer from any of the following prior to being diagnosed with ET? (n = 226)

Diagnosed Bleeding Event History

In addition to a bleeding history, patients were asked if they had ever been diagnosed with a more serious bleeding event. Most patients (72%) reported they had never been diagnosed with a serious bleeding event (Figure 22).

Although individually the serious bleeding events were relatively uncommon, collectively 28% of ET patients had been diagnosed with ≥1 of these conditions before their ET diagnosis.

History of Diagnosed Bleeding Event Prior to ET Diagnosis

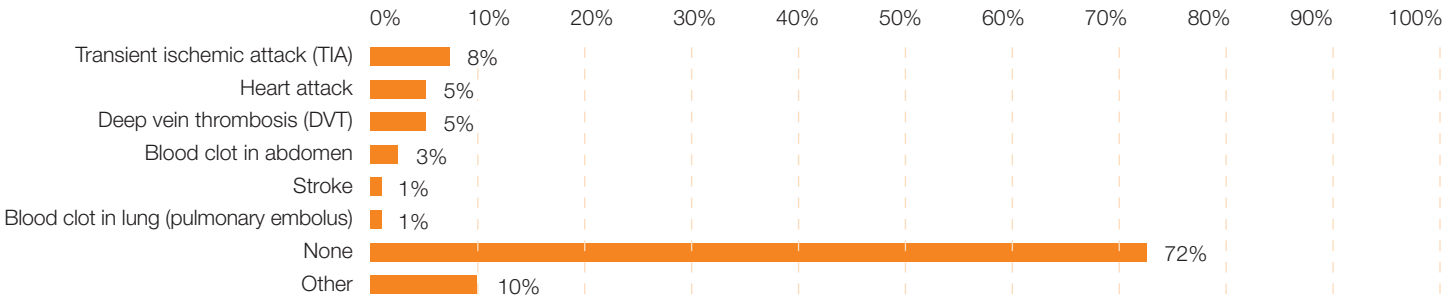


Figure 22. Question 27: Were you ever diagnosed with any of the following prior to being diagnosed with ET? (n = 226)

Summary of Findings

- The median number of years since diagnosis for patients surveyed was 7 years, and the mean age at diagnosis was 51 years of age (Figure 17).
- There was a span of as many as 2 years between onset and diagnosis in 31% of ET patients. Furthermore, 61% of patients experienced symptoms for more than 2 years before they received a formal diagnosis (Figure 18).
- Fatigue was the most common symptom at the time of diagnosis, reported by 38% of ET patients in the survey (Figure 19).
- Nearly two thirds (63%) of patients reported that they had no other diagnosed conditions in addition to ET; of the 37% who reported a concomitant condition, it was either kidney disease or heart attack (Figure 20).
- Most patients (65%) surveyed reported having a bleeding history before being diagnosed with ET (Figure 21).
- Most patients (72%) reported they had never been diagnosed with a serious bleeding event, whereas 28% had been (Figure 22).

Changing Physicians

ET patients were asked whether they had ever changed their ET physician and, if so, why they had made that change. The survey results found that more than half of ET patients (56%) had changed their physician (Figure 23). The most common reason for making the change was that the patient was unhappy with the care received from the physician (32%) (Figure 24). One fifth of patients (20%) said they changed physicians because the patient had relocated to a different city or state, and an additional 18% reported their physician had retired. Although most ET patients had switched physicians, most patients reported they were highly satisfied with their current physician.

Change in ET Physician Reported by Patients

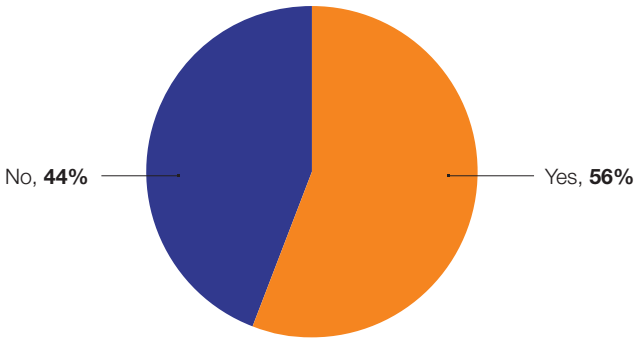


Figure 23. Question 39: Have you ever changed your ET doctor? (n = 226)

Reasons for Changing ET Physician

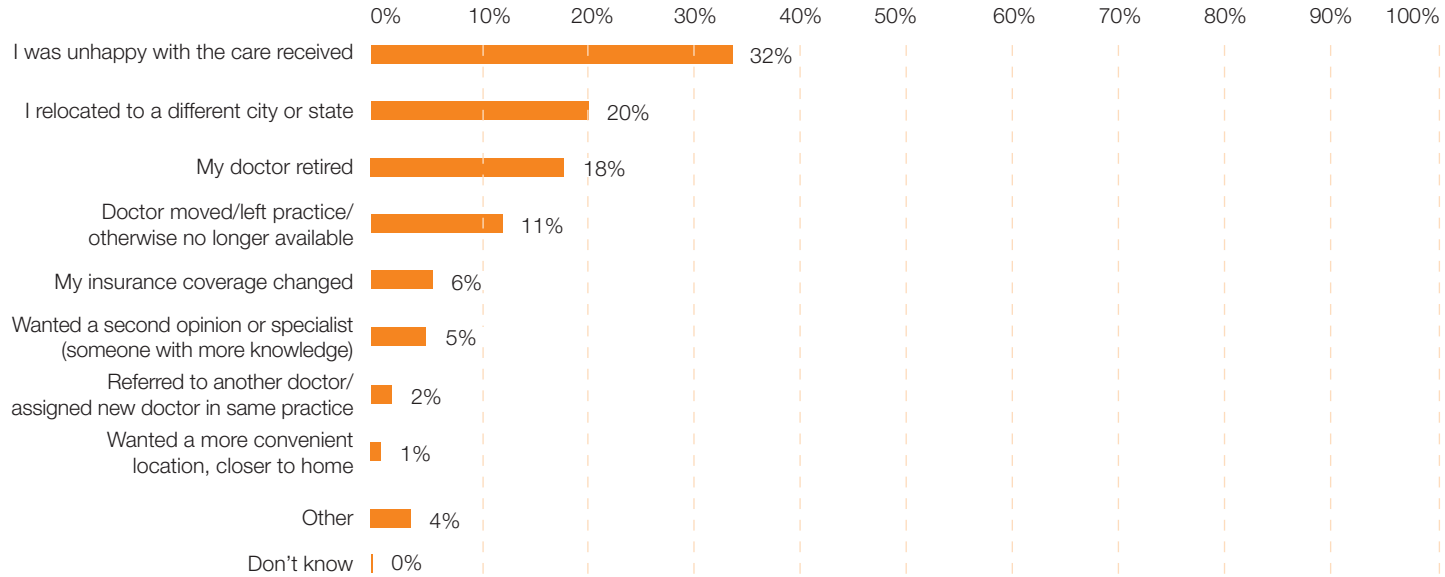


Figure 24. Question 39A: Why did you make a change? (n = 126)
Note: Individual values are rounded and may not total 100%

Number of ET Physician Visits Per Year

ET patients saw their ET physician an average of 5 times in the last year. More than two thirds of ET patients reported 4 or fewer ET physician visits in the past year (69%), and the remainder reported 5 or more visits in the past year (Figure 25).

Number of ET Physician Visits in the Past 12 Months

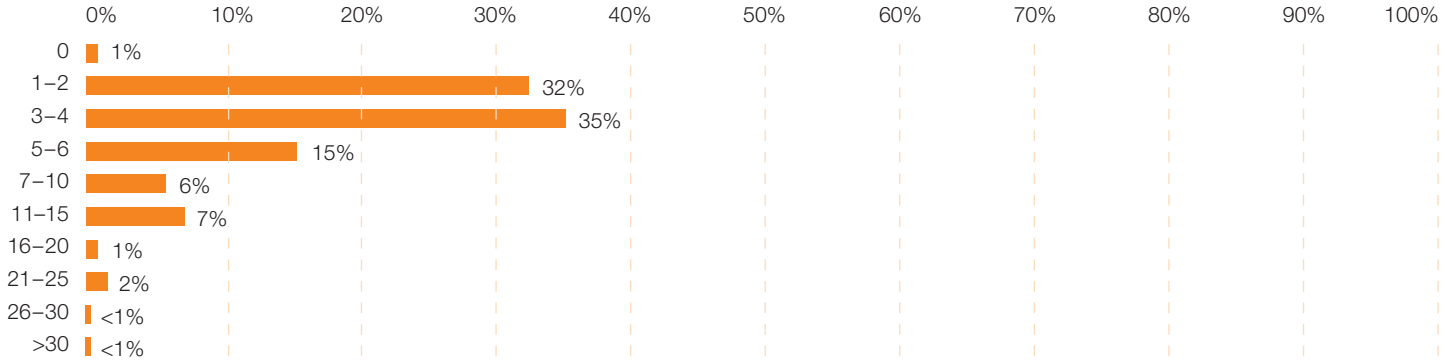


Figure 25. Question 38: How many times have you seen your doctor in the past 12 months? (n = 226)

Treatment Management Ever Received Reported by Patients

Patients were asked to select the treatments that they had ever received to help manage their ET. It is important to note that patients may not have been receiving reported treatment(s) at time of survey completion.

The most common treatments ET patients had ever received to manage their condition were antiplatelet therapy (92%) and chemotherapy (69%) (Figure 26). More than a third of patients (36%) reported receiving Agrylin® (anagrelide). Much less common, but still reported by ET patients, Pegasys® (interferon) (11%), anticoagulant therapy (10%), and antidepressants (10%) were taken at some point during their disease.

Treatment Management Ever Received Reported by ET Patients

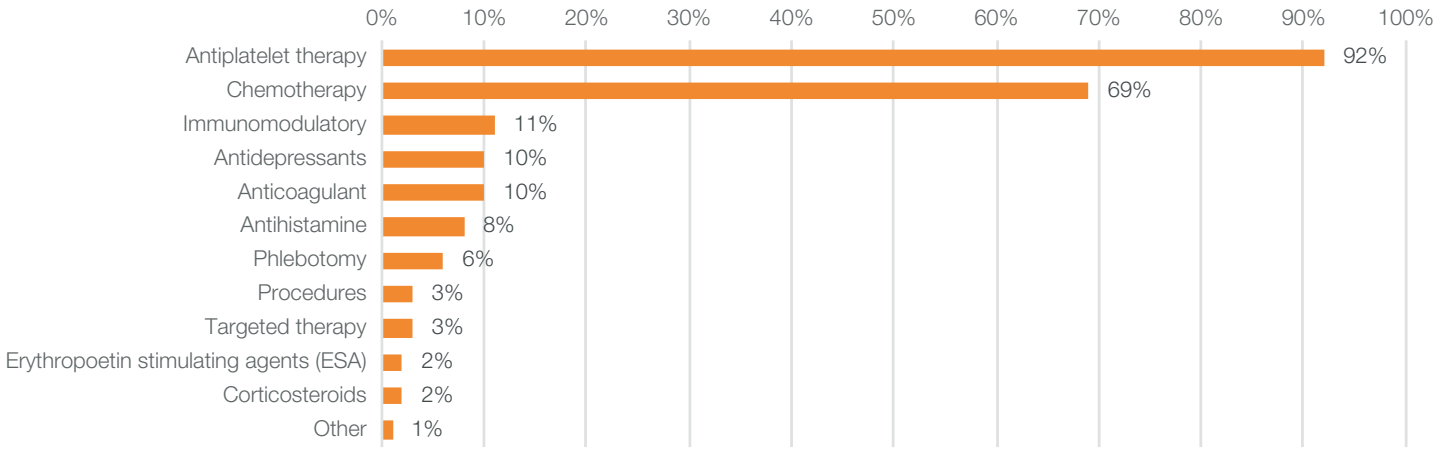


Figure 26. Question 28: Select any of the following treatments that you have ever received to help manage your diagnosis. (n = 226)

Burden of Treatment Side Effects

Patients surveyed who reported ever receiving specific treatments (bone marrow transplant, phlebotomy, removal of spleen, and radiation therapy) were asked to report the extent to which side effects from those specific treatments negatively effected their QOL. Of the 13 patients who received phlebotomy, 38% reported side effects that had at least some negative impact on their QOL. Of the 5 patients who had their spleen removed, nearly two thirds (60%) reported the side effects had at least some negative impact on their QOL (Figure 27).

Burden of Treatment Side Effects as Reported by ET Patients

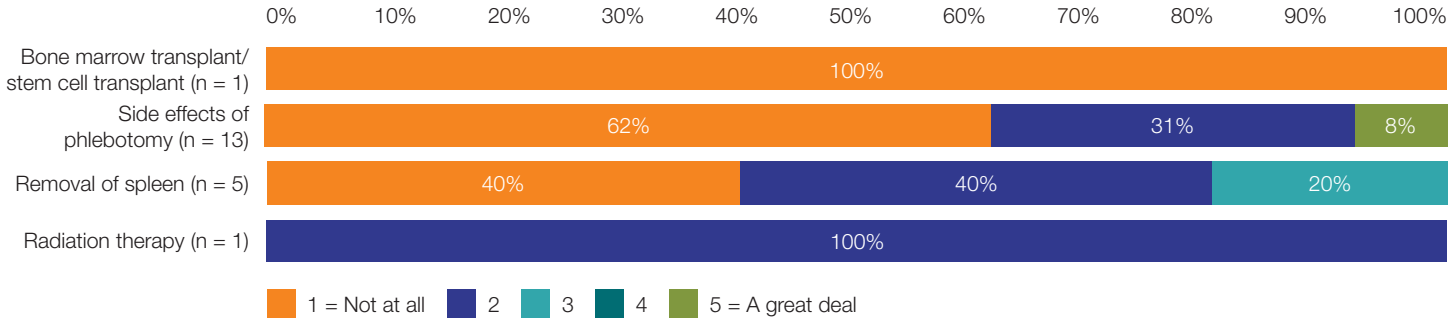


Figure 27. Question 28cc-rr: On a scale from 1 (not at all) to 5 (a great deal), to what extent do side effects from [treatment] have a negative impact on your quality of life?

Treatment Management Goal Attitudes Reported by Patients

Patients were asked, aside from a cure for ET, what their top 3 most important treatment goals were. Survey findings showed that ET patients consistently chose the same 3 goals as their top selections.

Prevention of vascular/thrombotic events, slowing or delaying progression, and healthy blood counts were the most common treatment goals selected by ET patients (Figure 28).

Most Important Patient Treatment Goal

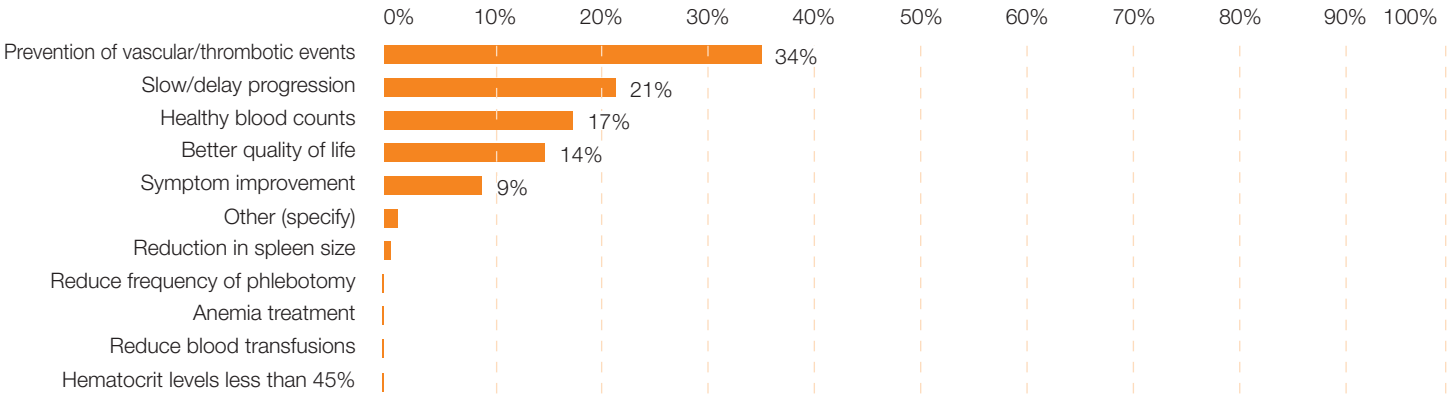


Figure 28. Question 32: Other than a cure for diagnosis, what is your most important treatment goal for therapy? (n = 226)
Note: Labels for data under 5% are not displayed

Other Therapies Beyond Prescription Drugs and Treatments

Patients surveyed were asked to report any other therapies, beyond prescription drugs and treatments, they used to manage ET symptoms. Most patients (69%) reported they exercised to help manage ET symptoms (Figure 29). Additionally, 46% reported taking nonprescription supplements to manage symptoms. A fifth of patients (20%) mentioned a change in their diet to help manage symptoms.

Managing Symptoms Beyond Prescription Drugs and Treatments

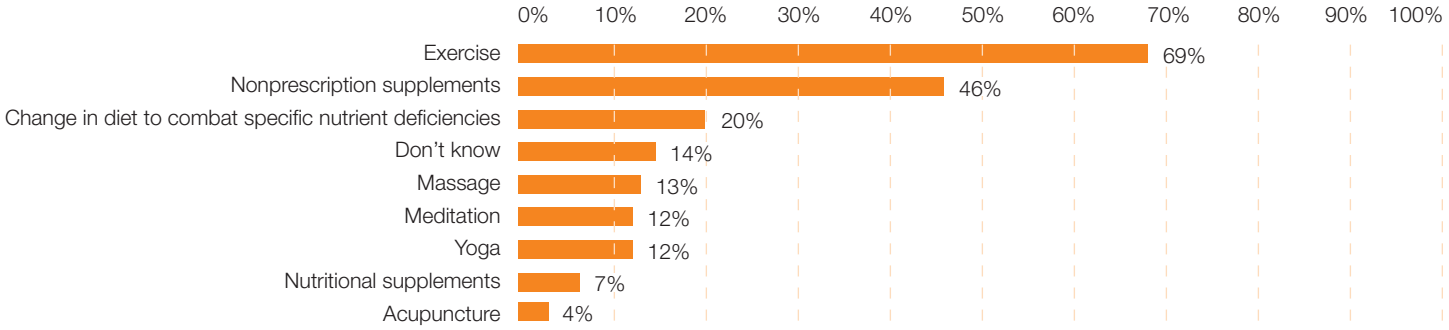


Figure 29. Question 31: Aside from prescription drugs and treatments, select any of the following things you do to manage your essential thrombocythemia symptoms? (n = 226)

Patient Measures of Treatment Success

In addition to their goals for treatment, patients were asked what measures they used to determine whether a treatment for ET was successful or not. Almost all patients (89%) selected lab results or blood counts as one of the measures they used to evaluate their treatment (Figure 30).

Feedback from the physician (74%) was the second most common measure of treatment success and 28% of patients reported overall symptom relief as a measure used to determine treatment success.

Measures of Treatment Success Reported by Patients

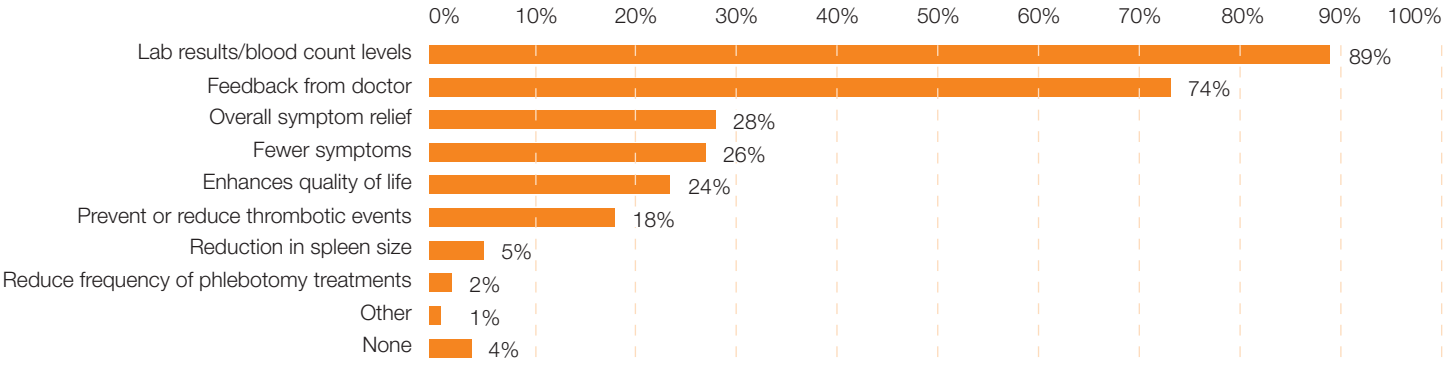


Figure 30. Question 30: What measures do you use to determine whether a diagnosis treatment is successful or not successful? (n = 226)

Summary of Findings

- Although more than half of ET patients (56%) had switched physicians (Figure 23), with the most common reason being that they were unhappy with the care (Figure 24), most patients reported that they were highly satisfied with their current physician.
- ET patients saw their physician an average of 5 times in the last year (Figure 25).
- The most common treatments ET patients had ever received to manage their condition were antiplatelet therapy (92%), followed by chemotherapy (69%) (Figure 26).
- Prevention of vascular/thrombotic events was the most common treatment management goal selected by more than one third (34%) of ET patients (Figure 28).
- Most patients (69%) reported they exercised to help manage their ET symptoms (Figure 29).
- The most common measure of treatment success reported by ET patients was lab results or blood counts (89%), followed by feedback from doctor (74%) (Figure 30).

Current Treatment Management Reported by Physicians

Physicians were provided with the same list of treatments as the patient survey and were asked to select the treatments they had ever recommended to their ET patients. An overwhelming majority of physicians (90%) reported they had recommended antiplatelet therapy to their ET patients (Figure 31). Also, 76% of physicians had recommended chemotherapy.

Percentage of Physicians Who Have Ever Recommended Treatment Management

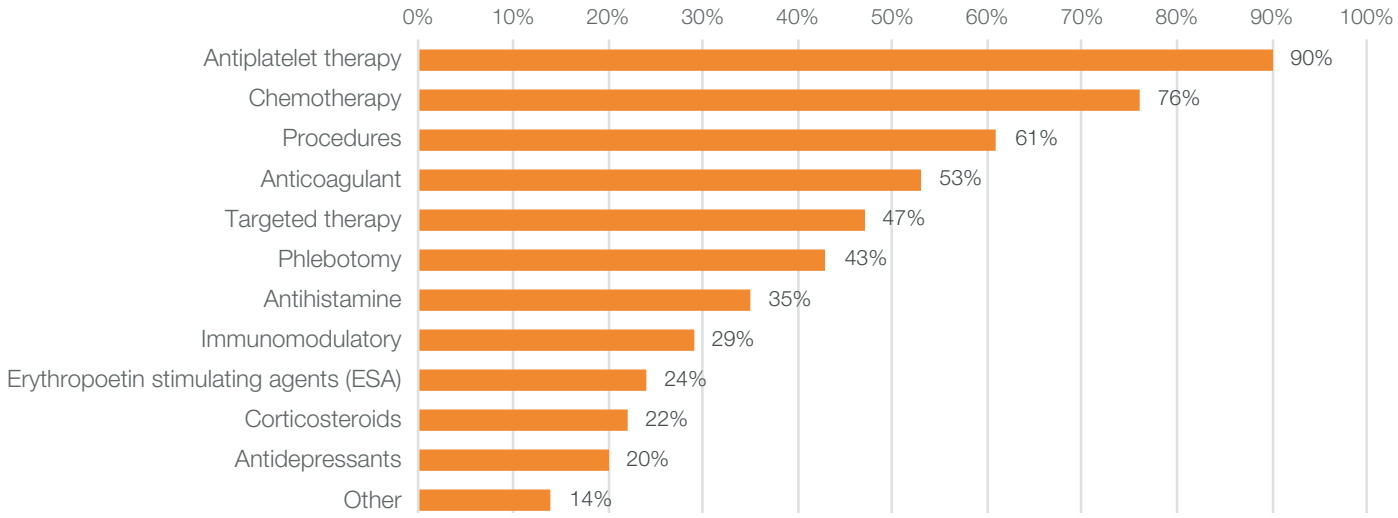


Figure 31. Question 34: Have you ever recommended any of the following treatments to your ET patients? (n = 51)

Decision to Observe Patient or Recommend Drug Treatment at Time of Diagnosis

Physicians surveyed were asked to estimate the proportion of patients they decided to observe at time of diagnosis. They were next asked to estimate the proportion of patients to whom they decided to recommend drug treatment at diagnosis. The estimated mean proportion of patients physicians decided to observe at diagnosis was 29% (Figure 32). The estimated mean proportion of patients who received a recommendation for drug treatment at time of diagnosis was 55% (Figure 33).

Physician-Reported Estimated Proportion of Patients Physicians Decided to Observe at Diagnosis

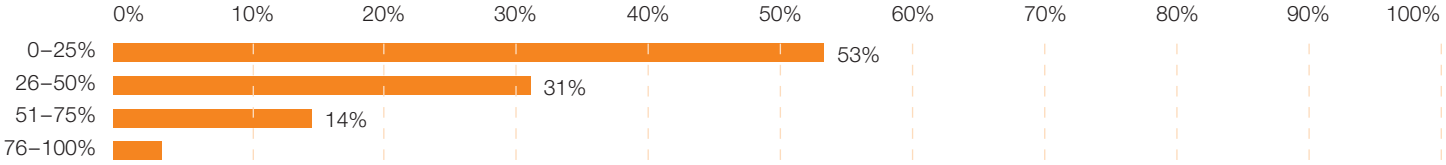


Figure 32. Question 11: What proportion of patients do you choose to observe instead of recommending drug treatment? (n = 51)
Note: Labels for data under 5% are not displayed

Physician-Reported Estimated Proportion of Patients for Whom Drug Treatment Was Recommended at Diagnosis

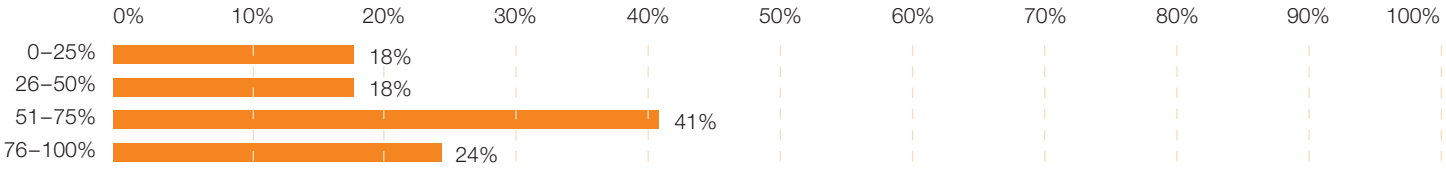


Figure 33. Question 12: What proportion of your newly diagnosed patients do you ever recommend drug treatment at time of diagnosis? (n = 51)

Physician Perception of the Impact Symptom Severity Has on Patient QOL

Most physicians (84%) agreed even mild to moderate symptoms can have a significant impact on a patient's QOL (Figure 34).

Physician-Reported Symptom Impact on QOL

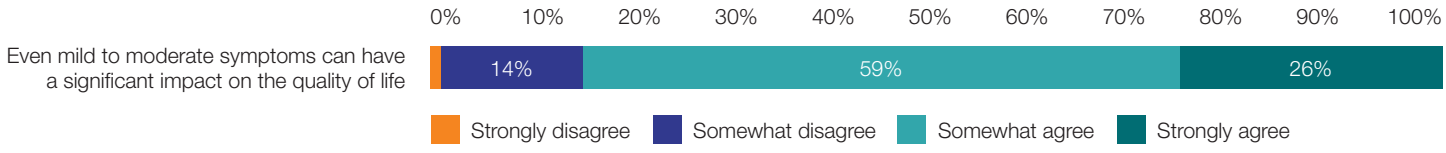


Figure 34. Question 22: Agree or disagree with the following statements: Even mild to moderate symptoms can have a significant impact on the quality of life (n = 51)
Note: Labels for data under 5% are not displayed

Treatment Recommendations by Symptom Severity

Physicians were asked about their likelihood to recommend drug treatment for ET patients based on the severity of their symptoms. ET-treating physicians surveyed recommended drug treatment for an average of 22% of patients with mild symptoms, 54% with moderate symptoms, and 74% with severe symptoms (Figure 35).

Physician-Reported Mean Proportion of Patients Recommended Drug Treatment Based on Symptom Severity

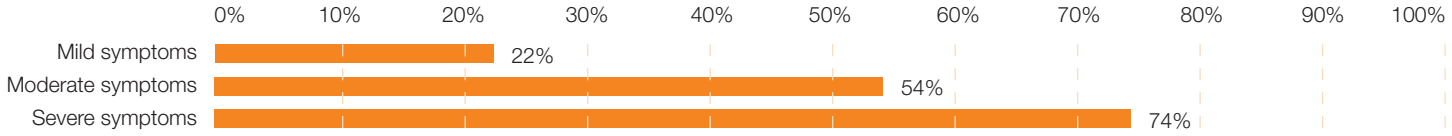


Figure 35. Question 33: What proportion of those ET patients who are experiencing [mild, moderate, or severe] symptoms alone, do you ever recommend drug treatment? (n = 51)

Treatment Management Goal Attitudes Reported by Physicians

Like patients, physicians were asked to report, aside from a cure for ET, their top 3 most important treatment goals for therapy. Patients and physicians were aligned with the most important treatment goal. Prevention of vascular events was selected as the most important goal for ET therapy by 57% of physicians (Figure 36).

The second most important goal for therapy reported was better QOL (18%), followed by symptom improvement (14%) (Figure 36).

Most Important Goals for Therapy Reported by Physicians

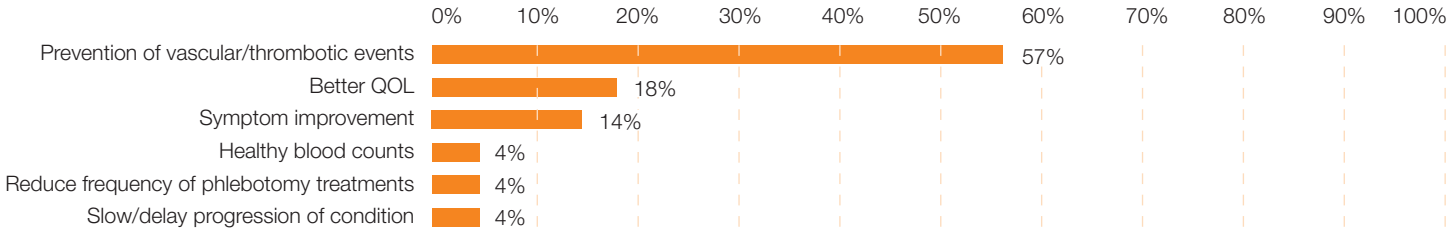


Figure 36. Question 36: Other than a cure for this diagnosis, what is your most important treatment goal for therapy? (n = 51)

Reasons to Change Treatment Reported by Physicians

As a counterpart to the patient question regarding measures of treatment success, physicians were asked under what conditions would they change drug therapy for their ET patients. Physicians could select more than one response. Most physicians surveyed responded that they would change a patient's drug therapy based on side effects (82%), lack of efficacy (80%), or disease progression (72%) (Figure 37).

Reasons to Change Treatment Reported by Physicians

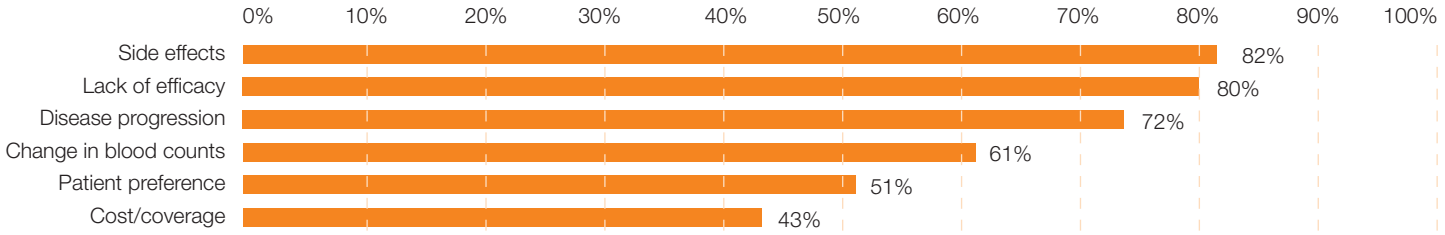


Figure 37. Question 39: Under what conditions would you change drug therapy for your ET patient? (n = 51)

Unmet Needs in Current Treatments as Reported by Physicians

Physicians were asked in an open-ended question, what they considered to be the most important unmet need in ET treatment. Their answers were coded and grouped into broad categories for the purposes of this report.

Effective drugs or therapies were mentioned by 43% of physicians as the most important unmet need in ET treatment. (Figure 38). A smaller group of physicians mentioned a need for a cure (20%), and 16% reported a need for symptom improvement (Figure 38).

Most Important Unmet Need in Current Treatments as Reported by Physicians

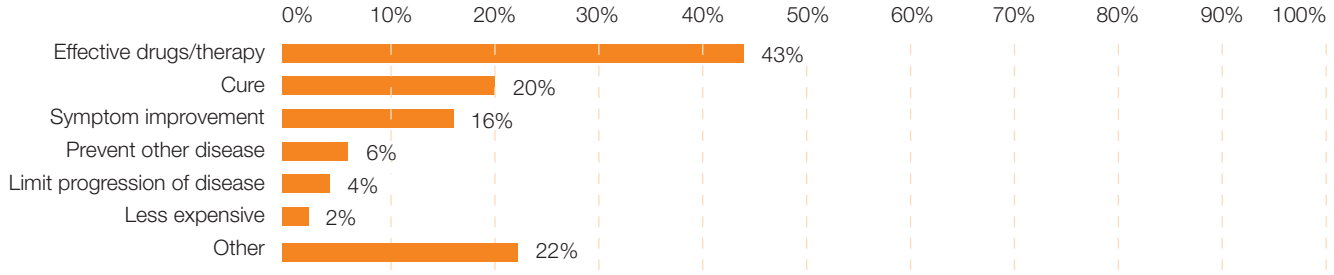


Figure 38. Question 54: (Open-Ended Response) What do you consider to be the most important unmet need in ET treatment? (n = 51)
Note: Open-ended responses were coded and grouped into broad categories displayed in chart

Summary of Findings

- An overwhelming majority of physicians (90%) reported that they recommended antiplatelet therapy to their ET patients (Figure 31).
- Most physicians (84%) agreed even mild to moderate symptoms can have an impact on patient's QOL (Figure 34).
- Physicians recommended drug therapy to an average of 22% of ET patients with mild symptoms, 54% of patients with moderate symptoms, and 74% of patients with severe symptoms (Figure 35).
- Prevention of vascular/thromobotic events was selected as the most important goal for ET therapy by 57% of physicians (Figure 36).
- Most physicians surveyed would change a patient's drug therapy based on lack of efficacy (80%), side effects (82%), or disease progression (72%) (Figure 37).
- Effective drugs or therapies were mentioned by 43% of physicians as the most important unmet need in ET treatment (Figure 38).

Patient-Reported vs Physician-Reported Symptom Assessment

The following section is a comparison of a set of questions that were asked of both patients and physicians in their respective surveys. The purpose of the comparison is to note any areas within the reported data for which concordance or discordance may exist in the perception of physicians and their patients. These areas demonstrated that, although a conversation is occurring between patients and their physicians, there is room for improvement on both ends that may lead to improved patient outcomes.

The survey provided an opportunity to compare patient and physician perspectives on how ET symptoms are assessed during physician visits. Patients were asked how the physician they see most often assesses ET symptoms they might be experiencing. Physicians were asked how they assess patient symptoms during an average patient visit. There was a gap in how patients reported that their physicians assess their symptoms and how physicians described their symptom assessment methods.

Most ET patients (44%) stated that physicians just proactively asked how they were feeling, whereas most physicians (55%) reported that they specifically asked about most important symptoms (Figure 39).

Patient-Reported Symptom Assessment

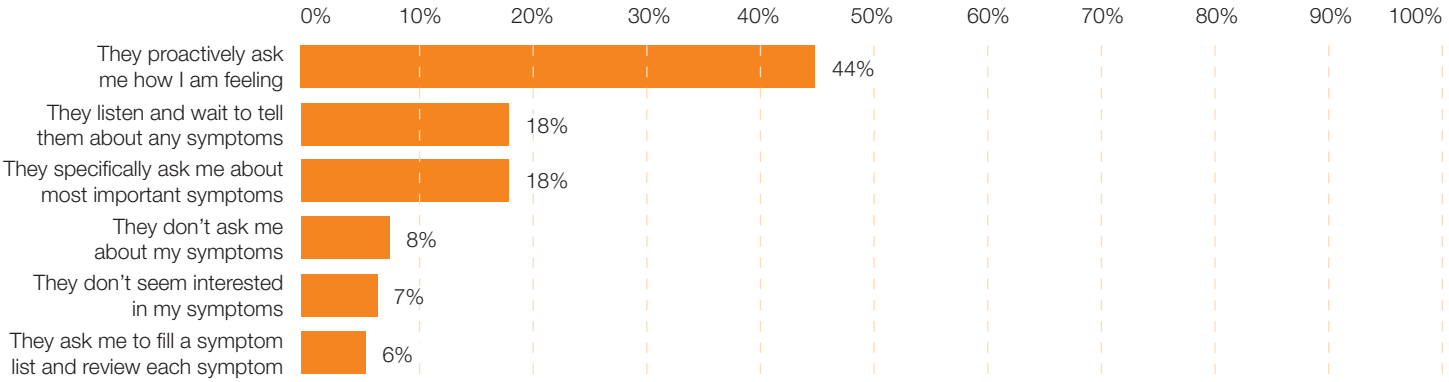


Figure 39. Question 41: How does the doctor you see most often for your diagnosis assess any diagnosis symptoms you may be experiencing? (n = 226)

Physician-Reported Symptom Assessment

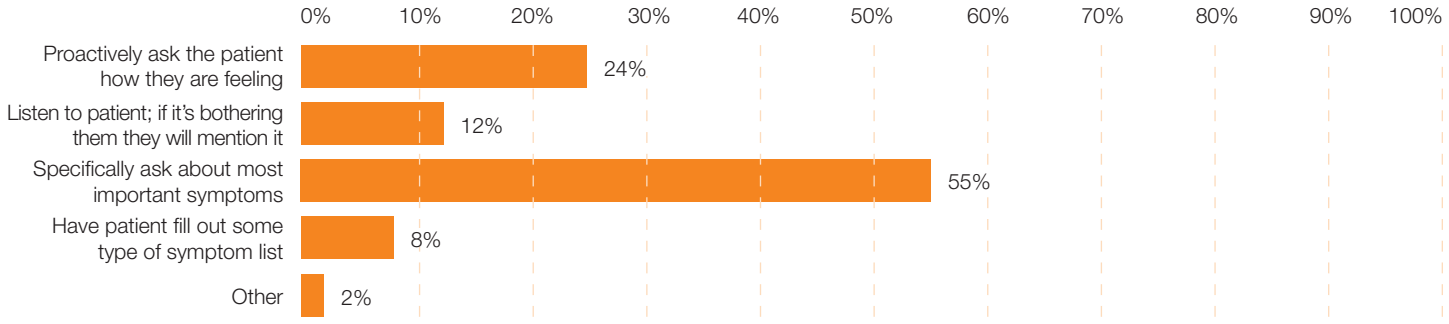


Figure 40. Question 27: During an average patient visit, how do you assess patient's symptoms? (n = 51)

Patient-Reported Symptoms vs Symptoms Heard by Physicians

ET patients were asked to report all the symptoms they had ever experienced from having ET and for comparison, physicians were asked for the 5 symptoms they heard the most from their ET patients. Findings showed that there is similar agreement between patients and physicians on the top symptoms. For instance, fatigue was the most commonly experienced symptom by patients (71%) and the symptom most commonly heard by physicians from ET patients (78%) (Figures 41 and 42). Bruising was the second most common reported symptom by patients (52%) and the third most commonly heard symptom among physicians (47%).

However, some gaps existed beyond the top symptoms. Half of ET patients surveyed (50%) experienced numbness or tingling in their hands or feet, whereas only 14% of ET-treating physicians reported it in the top 5 symptoms heard from patients. Furthermore, difficulty sleeping was the fourth most common symptom experienced by almost half of ET patients (49%); however, only 10% of physicians reported it as one of the top 5 symptoms heard most from their patients (Figures 41 and 42).

Symptoms Ever Experienced by ET Patients

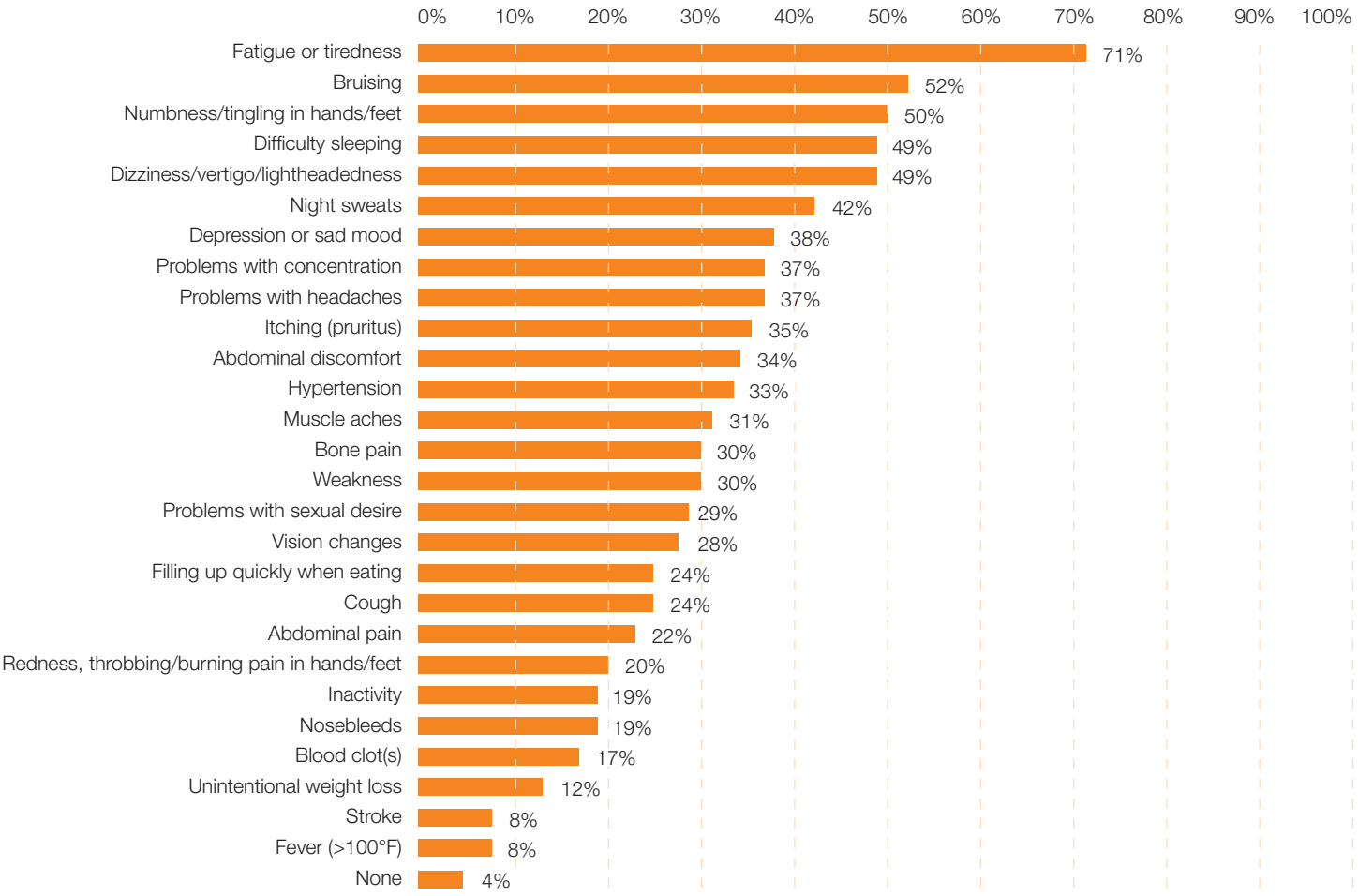


Figure 41. Question 8: Have you ever had any of the following symptoms? (n = 226)

Symptoms Most Heard by ET Physicians

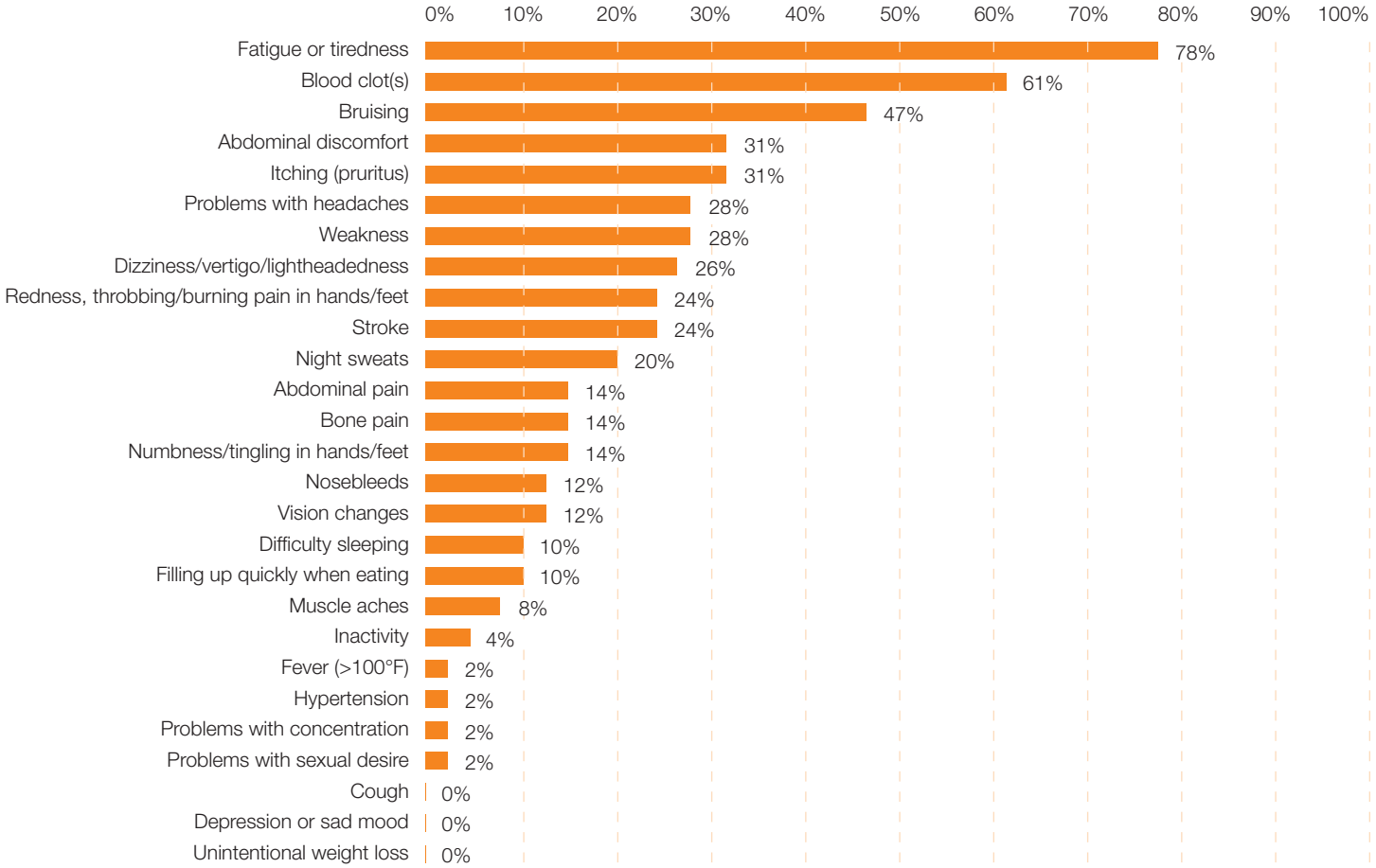


Figure 42. Question 15: What are the 5 symptoms of ET you most often hear about from your patients? (n = 51)

Patient-Reported vs Physician-Reported Symptoms at Diagnosis

Overall, 84% of patients reported symptoms at time of diagnosis. In comparison, physicians were asked to estimate the proportion of patients who presented with symptoms at time of diagnosis. Physicians estimated a mean of 51% of patients who presented with symptoms at time of diagnosis (Figure 43).

Percentage of Patients With Symptoms at Time of Diagnosis as Reported by Patients vs Physicians-Reported Estimate

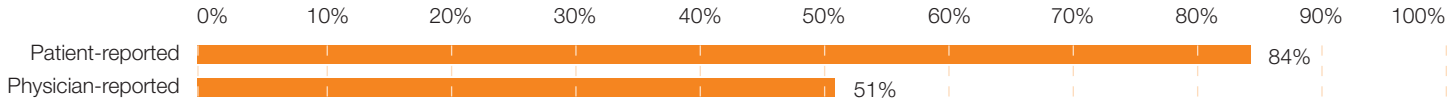


Figure 43. Question 9: Which of these symptoms were you experiencing at time of diagnosis? (n = 218) Question 10a-d: Out of 100%, what proportion of all newly diagnosed ET patients do you estimate have [no, mild, moderate or severe] symptoms? (n = 51)

Patient-Reported Symptom Resolution vs Physician-Reported Perception

Identical to the patient survey, physicians were asked to select the 1 symptom patients would most like to resolve. Almost one third of physicians selected stroke (29%) as the top symptom to resolve (Figure 44). Fatigue was selected by one third of ET patients (33%).

Patient-Reported First Symptom They Would Like to Resolve vs Physician-Reported Perception

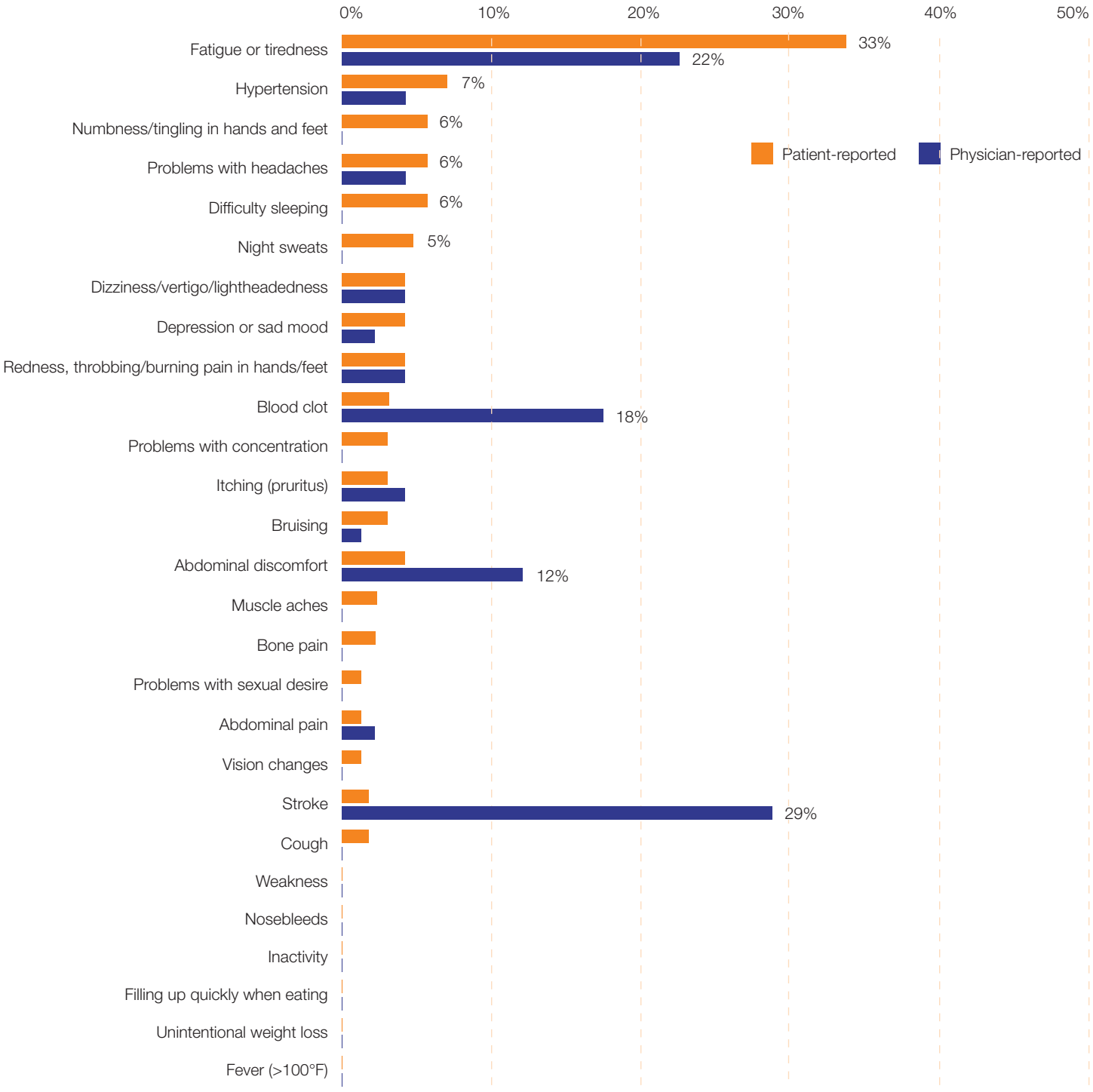


Figure 44. Question 14: Of the symptoms that you are currently experiencing, which one would you most like to resolve? (n = 218)
Question 17: Out of all the symptoms patients experience, which single symptom do you perceive they would most want to resolve? (n = 51)
Note: Labels for data under 5% are not displayed

Patient-Reported Symptom Recognition vs Physician-Reported Perception

As previously reported on Figure 4, most ET patients in this survey recognized the most common symptoms associated with ET. For comparison, physicians were asked to report the proportion of their ET patients they felt were able to recognize the symptoms associated with ET.

Most physicians (57%) reported that some of their patients were able to recognize the symptoms associated with ET (Figure 45). Twenty-six percent reported that almost all of their patients were able to recognize the symptoms associated with ET (Figure 45).

Physician's Perception of Patients' Ability to Recognize Symptoms

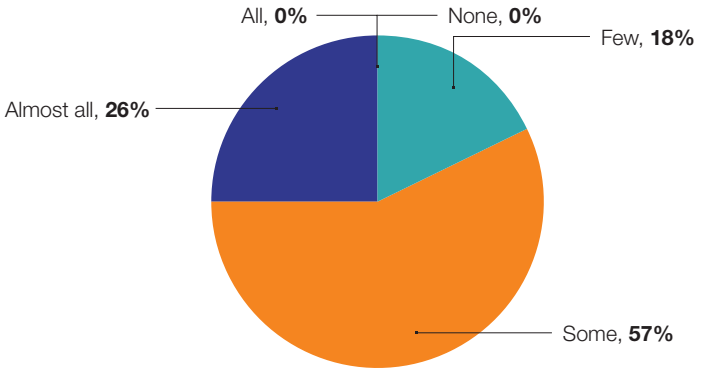
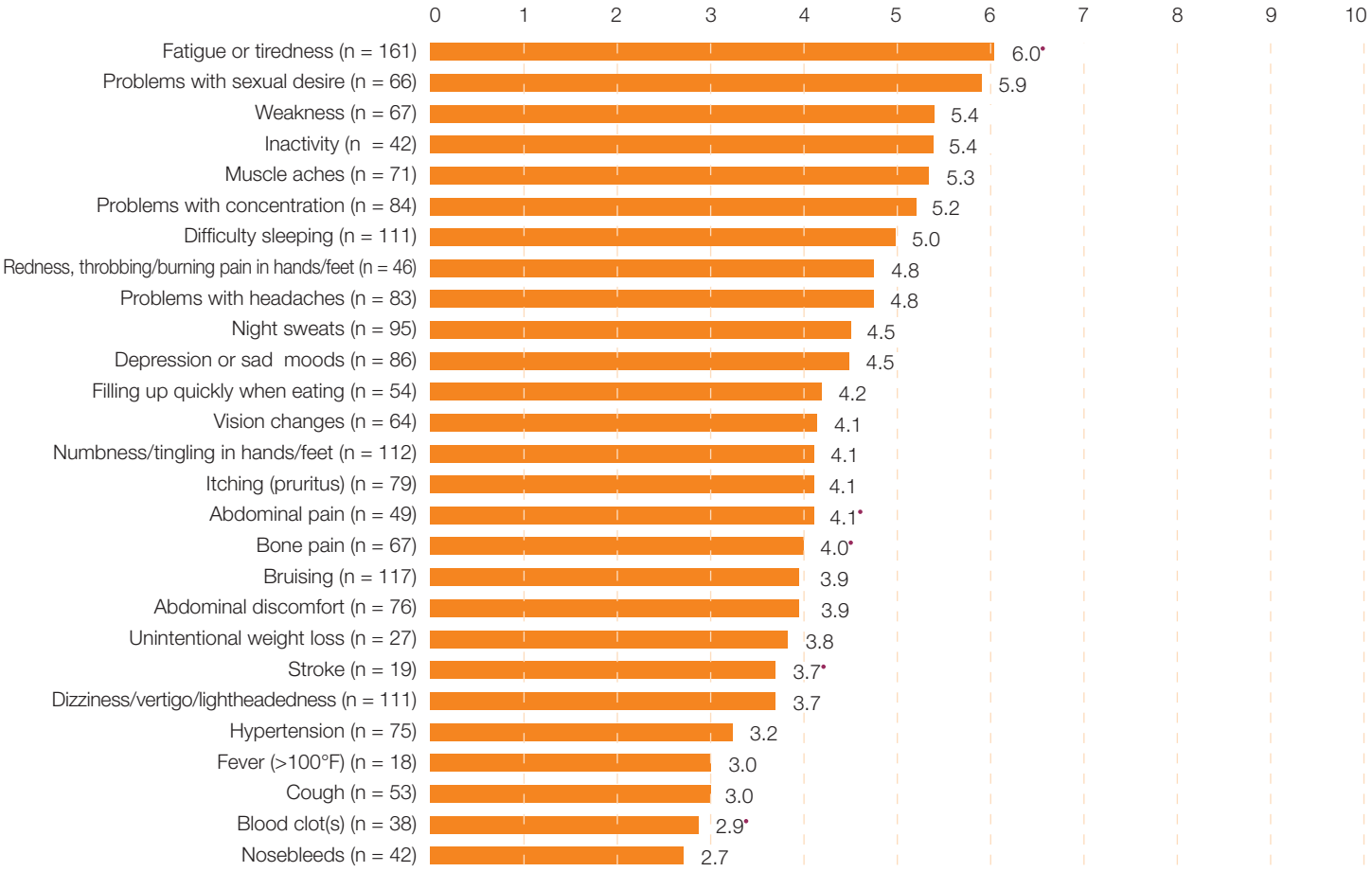


Figure 45. Question 31: What proportion of your ET patients recognize their symptoms as being related to ET? (n = 51)
Note: Individual values are rounded and may not total 100%

Patient-Reported Symptom Severity vs Physician Perception

There was less agreement between patients and physicians when it came to the severity of symptoms. Patients reported fatigue or tiredness, problems with sexual desire, weakness, and inactivity as the most severe symptoms, and physicians reported blood clot, stroke, fatigue or tiredness, bone pain, and abdominal pain as the top symptoms with the most negative impact on a patient's QOL. Figure 46 shows the discordance between patients and physicians with alignment on only 1 symptom, fatigue or tiredness, within each groups' top 5 rankings. Furthermore, the 2 top ranked symptoms by physicians, blood clot(s) and stroke, were among the lowest ranked symptoms as reported by patients.

Patient-Reported MPN-SAF Mean Severity Score (Physician-Reported Symptoms Included)



• Symptoms with most negative impact on QOL as reported by physicians.

Figure 46. Question 13a-dd: How severe is [symptom]?

Patient-Reported vs Physician-Reported History of Diagnosed Bleeding Event

Sixty-five percent of patients reported some type of bleeding history such as easy bruising and nosebleeds before their ET diagnosis, and more than a quarter of ET patients reported that, before their ET diagnosis, they had been diagnosed with a serious bleeding event (28%) before receiving their ET diagnosis. This percentage includes those patients who selected the "other" response option, which may have included conditions not related to bleeding. When physicians were asked what proportion of their patients had a previous history of a serious thrombotic/bleeding event, they reported a mean of 24%.

Patient-Reported vs Physician-Reported Goals for Therapy

Patients and physicians were asked, aside from a cure for ET, what was their most important goal for therapy. Among the ET patients in the survey sample, prevention of vascular/thrombotic events (34%) was the single most important goal for therapy; similarly, most physicians (57%) selected this response (Figure 47).

Patient-Reported vs Physician-Reported Most Important Goal for Therapy

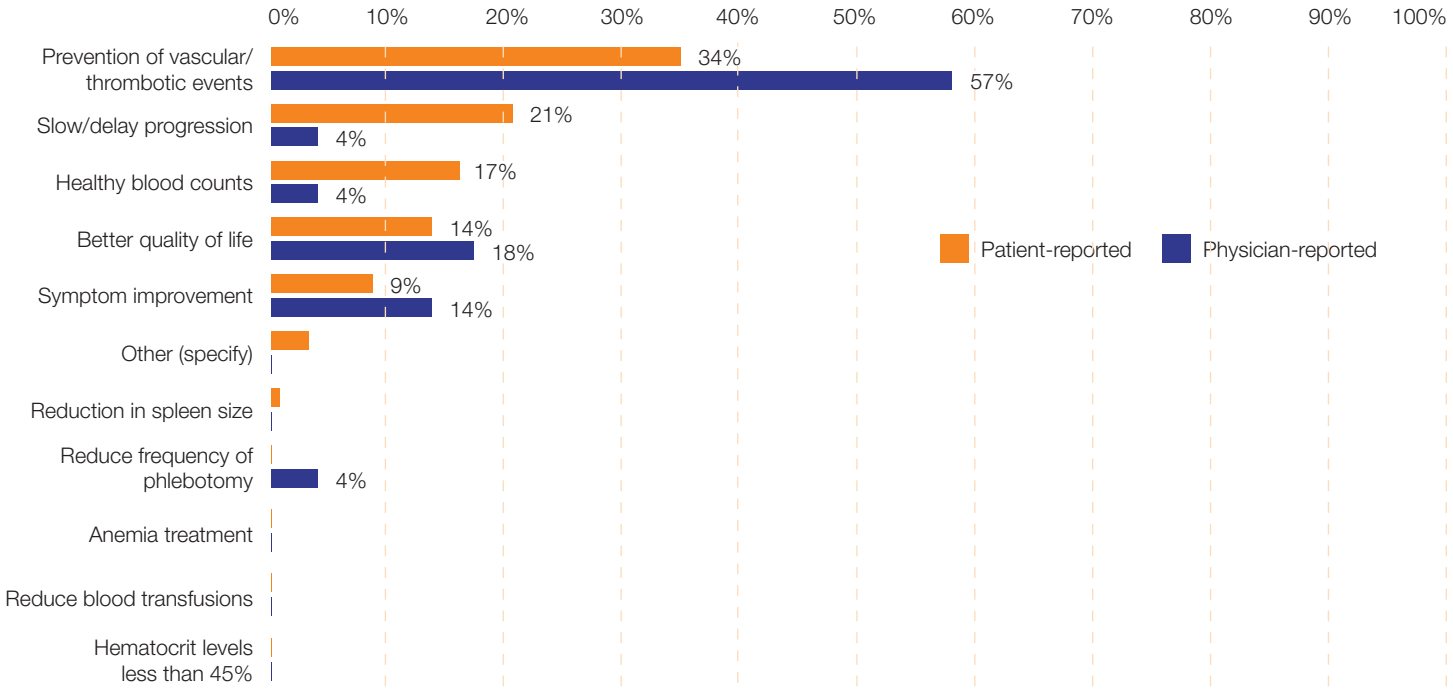


Figure 47. Question 36: Other than a cure for this diagnosis, what is your most important treatment goal for therapy? (n = 51)
Note: Labels for data under 4% are not displayed

Patient-Reported vs Physician-Reported Patient Preference for Involvement in Treatment Decisions

There was alignment between patients and physicians when it came to the extent of patient involvement in treatment decisions. Patients were asked about their desired involvement in treatment decisions, and conversely physicians were asked to rank the extent to which patients should be involved in treatment (1 = not at all to 5 = a great deal). Most patients surveyed reported a preference to have at least 50% involvement in treatment decisions (Figure 48). Most physicians reported that their patients want to be involved, reporting a ranking of either a "4" (55%) or a "5" (18%) (Figure 49).

Physicians were subsequently asked about patient compliance with their primary treatment recommendations. Interestingly, 79% of physicians reported that their patients sometimes (69%) or often (10%) did not wish to comply with their recommendation.

Preference for Decision Maker Regarding ET Treatments: Patient-Reported vs Physician-Reported

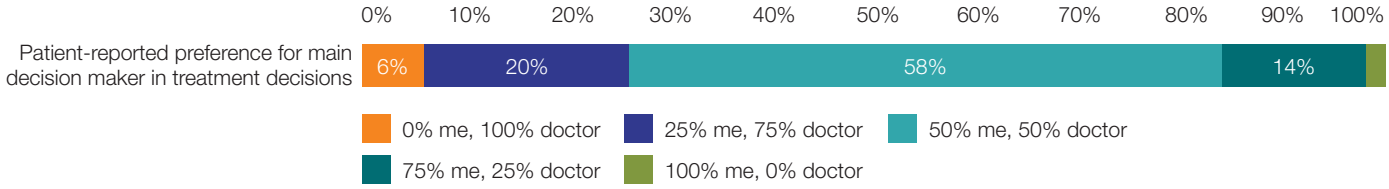


Figure 48. Question 45A: Now select the percentage that best represents your preference for who the main decision maker should be when it comes to decisions regarding your ET treatment. (n = 153)
Note: Labels for data under 5% are not displayed

Figure 49. Question 40: To what extent do your patients want to be involved in decisions regarding their treatment? (n = 51)
Note: Labels for data under 5% are not displayed

Patient-Reported Emotional Impact of ET vs Physician Perception

Most physicians ranked all of the items with the exception of "Avoided social interactions" between a "3" to "5," suggesting moderate to a great deal of emotional impact to patients (Figure 50). Physicians seemed to overestimate the emotional impact of ET on their patients. Most patients and physicians agreed that patients reported feeling anxious or worried and discouraged or depressed about their condition.

Patient-Reported Emotional Impact of ET

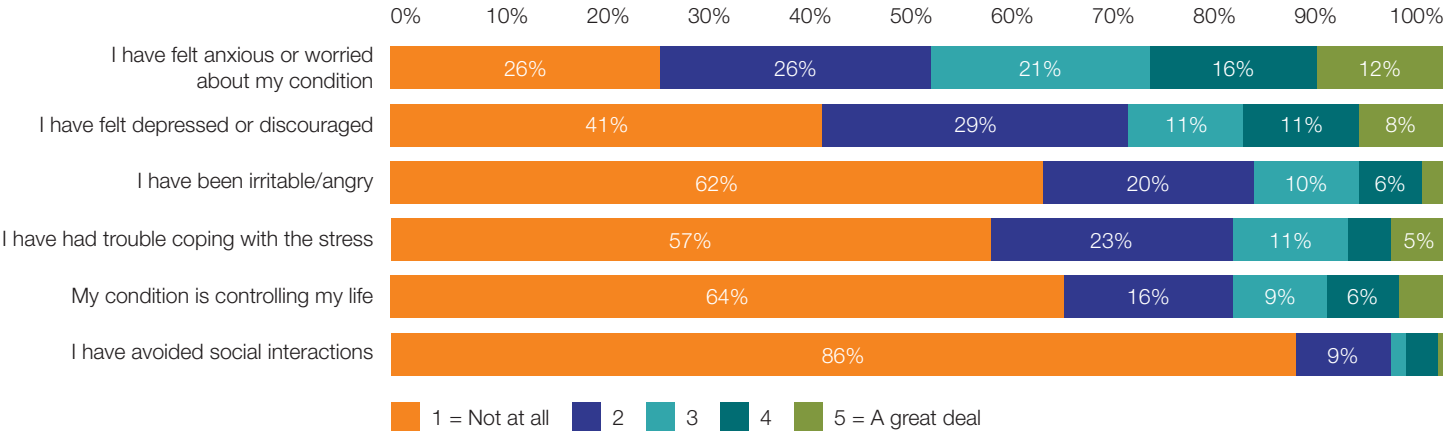


Figure 50. Question 24a-k: Rank the following statements as they have occurred during the past month, as a result of your ET (n = 226)
Note: Labels for data under 5% are not displayed

Physician-Reported Perceptions of Emotional Impact of ET

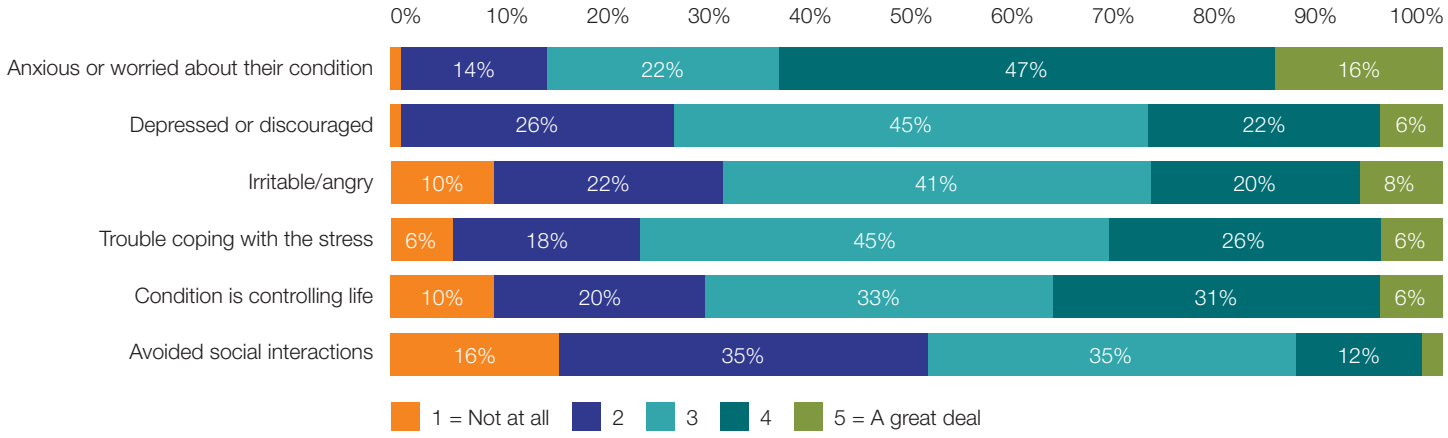


Figure 51. Question 20a-k: As a result of their condition, do you think patients with ET may feel/experience...? (n = 51)
Note: Labels for data under 5% are not displayed

Patient-Reported Physical Impact of ET vs Physician Perception

Most physicians ranked all, with the exception of "Condition has caused changes in looks," items between a "3" to "5" on a scale from 1 (not at all) to 5 (a great deal), reporting moderate to a great deal of physical impact (Figure 53). However, most patients ranked all the items between a "1" to "2," suggesting no impact or very little physical impact (Figure 52).

Patient-Reported Perceptions of Physical Impact of ET

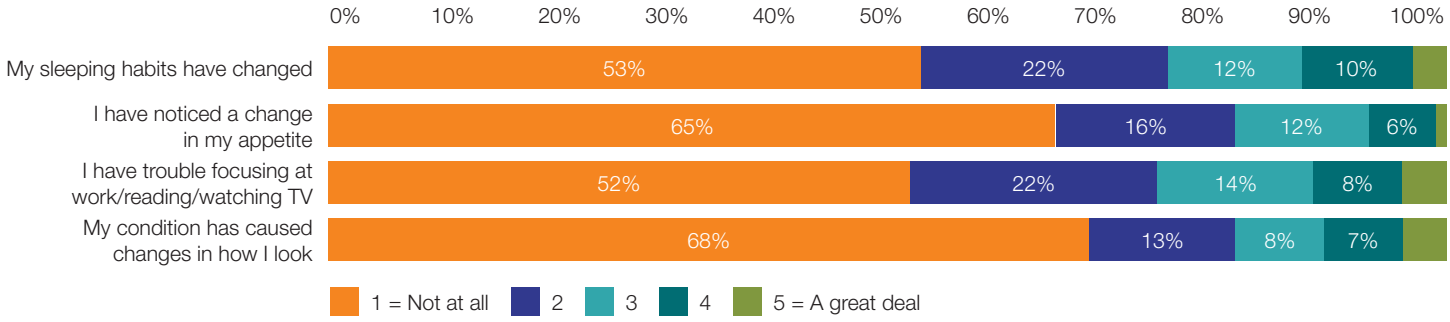


Figure 52. Question 24a-k: Rank the following statements as they have occurred during the past month, as a result of your ET. (n = 226)
Note: Labels for data under 5% are not displayed

Physician-Reported Perceptions of Physical Impact of ET

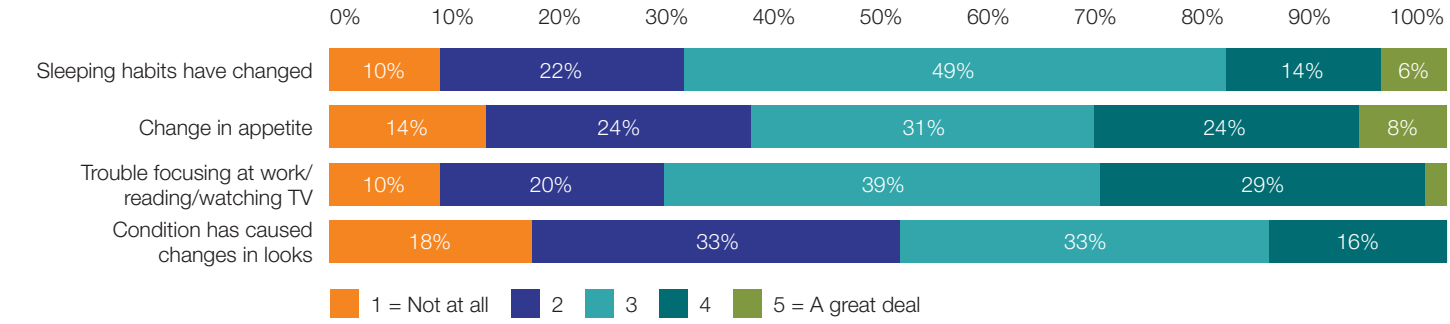


Figure 53. Question 20a-k: As a result of their condition, do you think patients with ET may feel/experience...? (n = 51)
Note: Labels for data under 5% are not displayed

Patient-Reported vs Physician-Reported Attitudes Towards ET

Patients and physicians were asked a series of questions regarding their attitudes towards ET. There was alignment on all of the questions. More than half of ET patients (57%) and most physicians (84%) agreed or strongly agreed that symptoms reduced a patient's QOL (Figures 54 and 55). Additionally, 93% of patients agreed or strongly agreed that ET is a serious condition. Most physicians agreed or strongly agreed (78%) that ET is a blood cancer. Lastly, 95% of patients and 92% of physicians agreed or strongly agreed that ET may progress to more serious conditions (Figures 54 and 55).

Patient-Reported Attitudes Towards ET

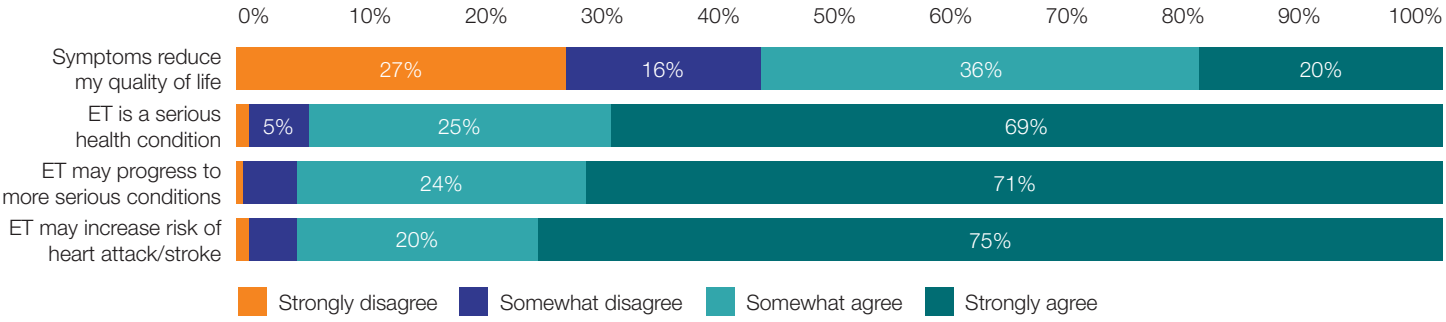


Figure 54. Question 35a-c: Please indicate whether you agree/disagree with the following statements... (n = 226)
Note: Labels for data under 5% are not displayed

Physician-Reported Attitudes Towards ET

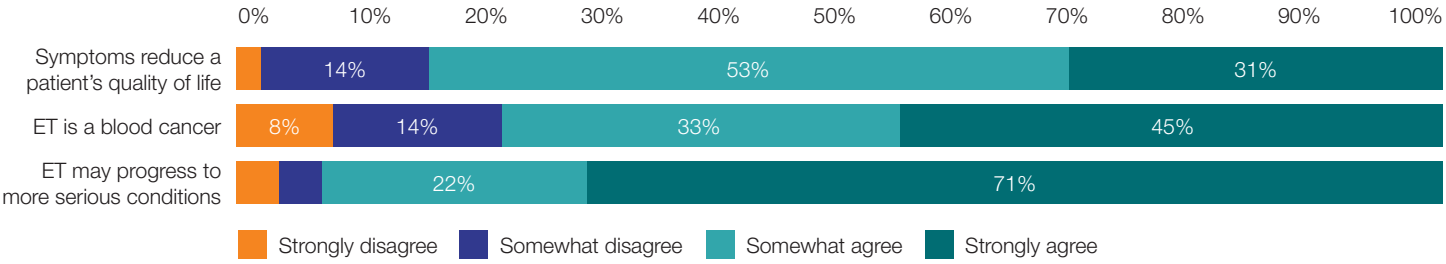


Figure 55. Question 45a-c: Please indicate whether you agree/disagree with the following statements... (n = 51)
Note: Labels for data under 5% are not displayed

Summary of Findings

- Most ET patients (44%) stated that physicians just proactively asked how they were feeling overall, whereas most physicians (55%) reported that they specifically asked about most important symptoms (Figures 39 and 40).
- Findings showed that there was similar agreement between what patients reported as top symptoms experienced versus top symptoms physician's heard (fatigue, bruising easily). However, gaps existed. For example, half of ET patients surveyed (50%) experienced numbness or tingling in their hands or feet, whereas only 14% of ET-treating physicians reported it in the top 5 symptoms heard from patients (Figures 41 and 42).
- Overall, 84% of patients reported symptoms at time of diagnosis. In comparison, physicians were asked to estimate the proportion of patients who presented with symptoms at time of diagnosis. Physicians estimated a mean of 51% of patients who presented with symptoms at time of diagnosis (Figure 43).
- Patients (33%) and physicians (22%) selected fatigue as one of the most important symptoms patients wanted to resolve (Figure 44).
- Most physicians (57%) reported that some of their patients were able to recognize the symptoms associated with ET (Figure 45). Twenty-six percent reported that almost all of their patients were able to recognize the symptoms associated with ET (Figure 45).
- Patients reported fatigue or tiredness, problems with sexual desire, weakness, and inactivity as the most severe symptoms, and physicians reported blood clot(s), stroke, fatigue or tiredness, bone pain, and abdominal pain as the top symptoms with the most negative impact on a patient's QOL. Figure 46 shows the discordance between patients and physicians with alignment on only 1 symptom, fatigue or tiredness, within each group's top 5 rankings.
- Among the ET patients in the survey sample, prevention of vascular/thrombotic events (34%) was the single most important goal for therapy; similarly, most physicians (57%) selected this response (Figure 47).
- Most patients surveyed reported a preference to have at least 50% involvement in treatment decisions (Figure 48). When looking at physician's preference, most physicians reported that their patients want to be involved, recording a ranking of either a "4" (55%) or a "5" (18%) (Figure 49).
- Most patients and physicians agreed that patients reported feeling anxious or worried and discouraged or depressed about their condition (Figures 50 and 51). Physicians tended to overestimate the emotional impact, as they ranked most items between a 3 to 5 suggesting moderate to a great deal of emotional impact to patients (Figure 51).
- Most physicians ranked all items with the exception of "Condition has caused changes in looks," between a 3 to 5 on a scale from 1 (not at all) to 5 (a great deal), reporting moderate to a great deal of physical impact (Figure 53). However, most patients ranked the items between a 1 to 2 suggesting no impact or very little physical impact (Figure 52).
- More than half of ET patients (57%) and most physicians (84%) agreed or strongly agreed that symptoms reduced a patient's QOL (Figures 54 and 55).

Satisfaction With Communication About Condition

The survey findings showed that nearly all ET patients (84%) were satisfied with their current physician's communication about their condition (Figure 56). Similarly, nearly all physicians (82%) were satisfied with their communication to ET patients about their condition (Figure 57).

Patient-Reported Satisfaction With Physician's Communication Regarding ET

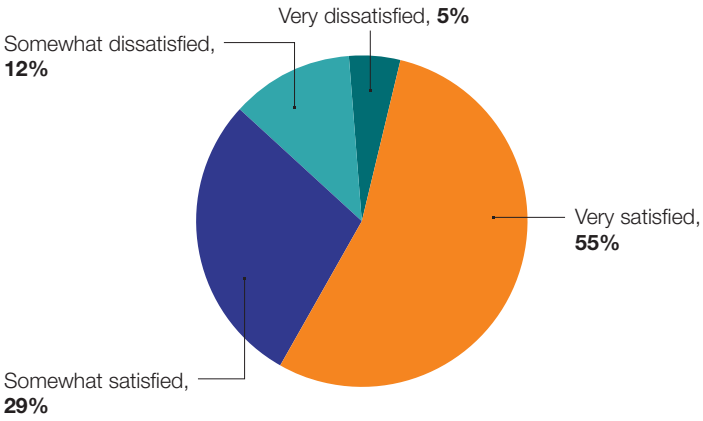


Figure 56. Question 42: How satisfied are you with your doctor's communication about your condition and its treatment? (n = 226)
Note: Individual values are rounded and may not total 100%

Physician-Reported Satisfaction With Communication to Patient Regarding ET

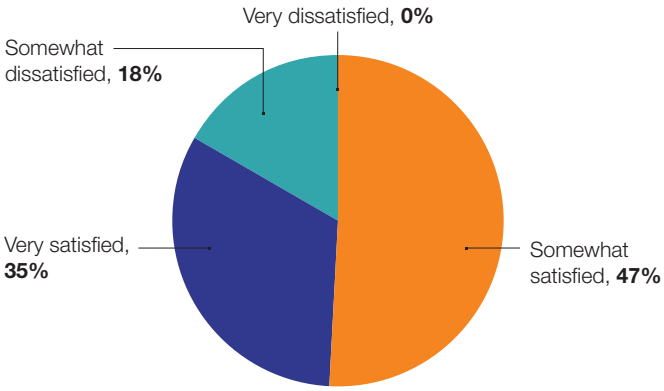


Figure 57. Question 48: How satisfied are you with your communications with ET patients about their condition and its treatment? (n = 51)
Note: Individual values are rounded and may not total 100%

Satisfaction With Management of Condition

The vast majority of ET patients (88%) were satisfied with their current physician's treatment and management of their condition (Figure 58). Physicians, however, were more likely to underestimate their patients' satisfaction with how they managed their condition. Most physicians (90%) thought their patients were satisfied with the treatment and management of their ET, but only a third of physicians (33%) thought their patients were very satisfied (Figure 59). By contrast, 58% of patients reported being very satisfied with their physician's treatment and management of their condition.

Patient-Reported Satisfaction With Overall Treatment & Management of ET

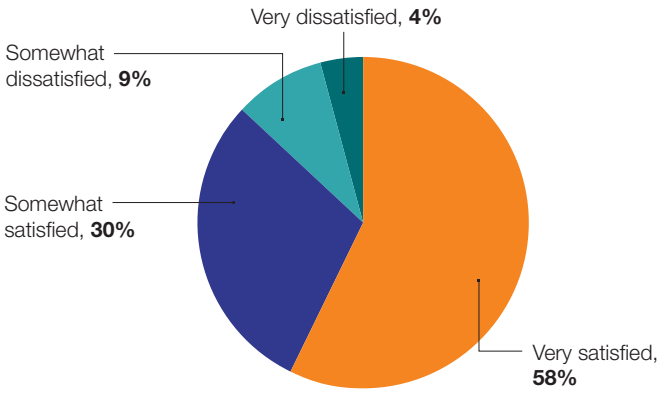


Figure 58. Question 43: How satisfied are you with your doctor's management and treatment of your diagnosis? (n = 226)
Note: Individual values are rounded and may not total 100%

Physician Perception of Patient Satisfaction With Overall Treatment & Management of ET

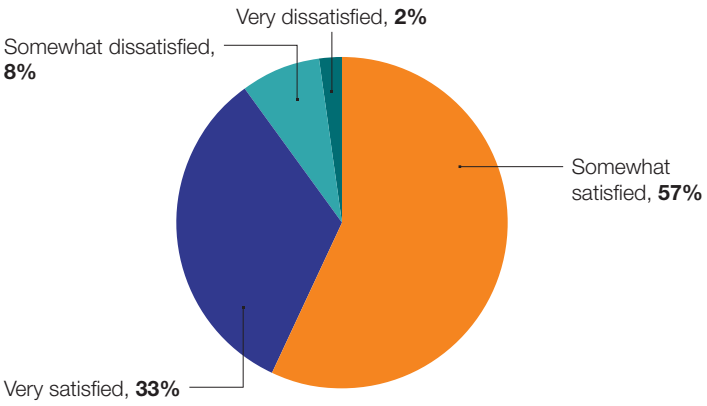


Figure 59. Question 43: In general, how satisfied are your patients with the overall treatment and management of their condition? (n = 51)
Note: Individual values are rounded and may not total 100%

Attitudes Towards Communication Regarding Symptoms

Both patients and physicians were asked a series of questions to describe their attitude towards communication with regard to symptoms. Most patients disagreed with all statements that implied any difficulty or issues in their communication with their physician about their symptoms. In comparison, although physicians disagreed with most statements implying any difficulty or issues in their communication with patients, they were not as likely as patients to strongly disagree.

Patients reported they felt comfortable discussing their symptoms with their physician (82%), and most patients (79%) were certain on how to describe their symptoms to their physician (Figure 60).

Patient-Reported Attitudes Towards Communication Regarding Symptoms

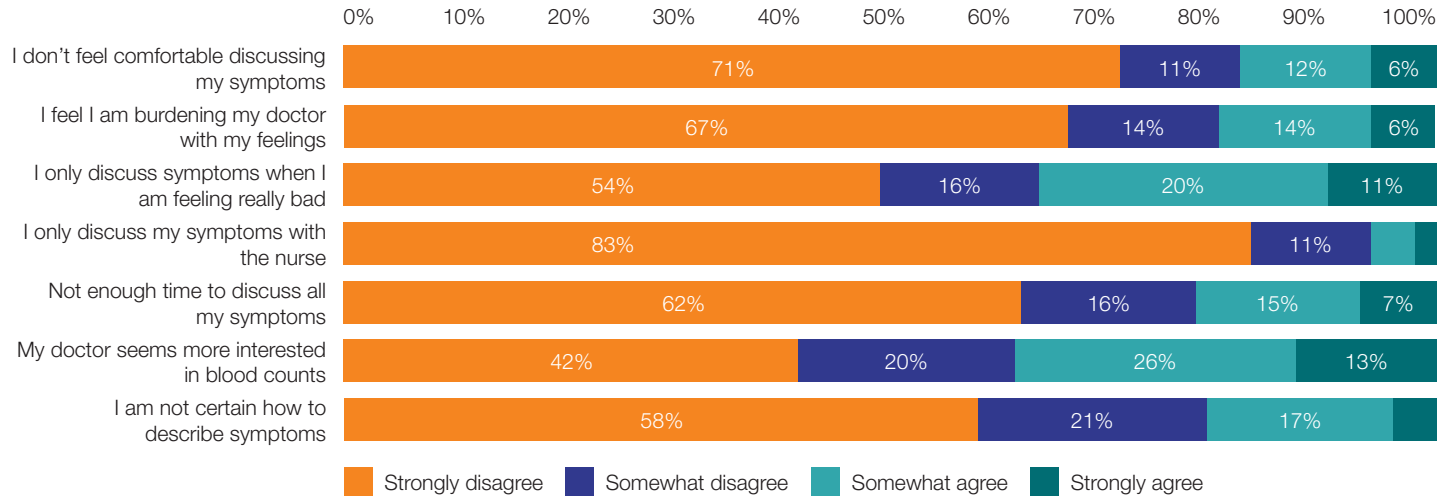


Figure 60. Question 47h-n: How much do you agree with the following statements? (n = 226) Note: Labels for data under 5% are not displayed

Physician-Reported Attitudes Towards Communication Regarding Symptoms

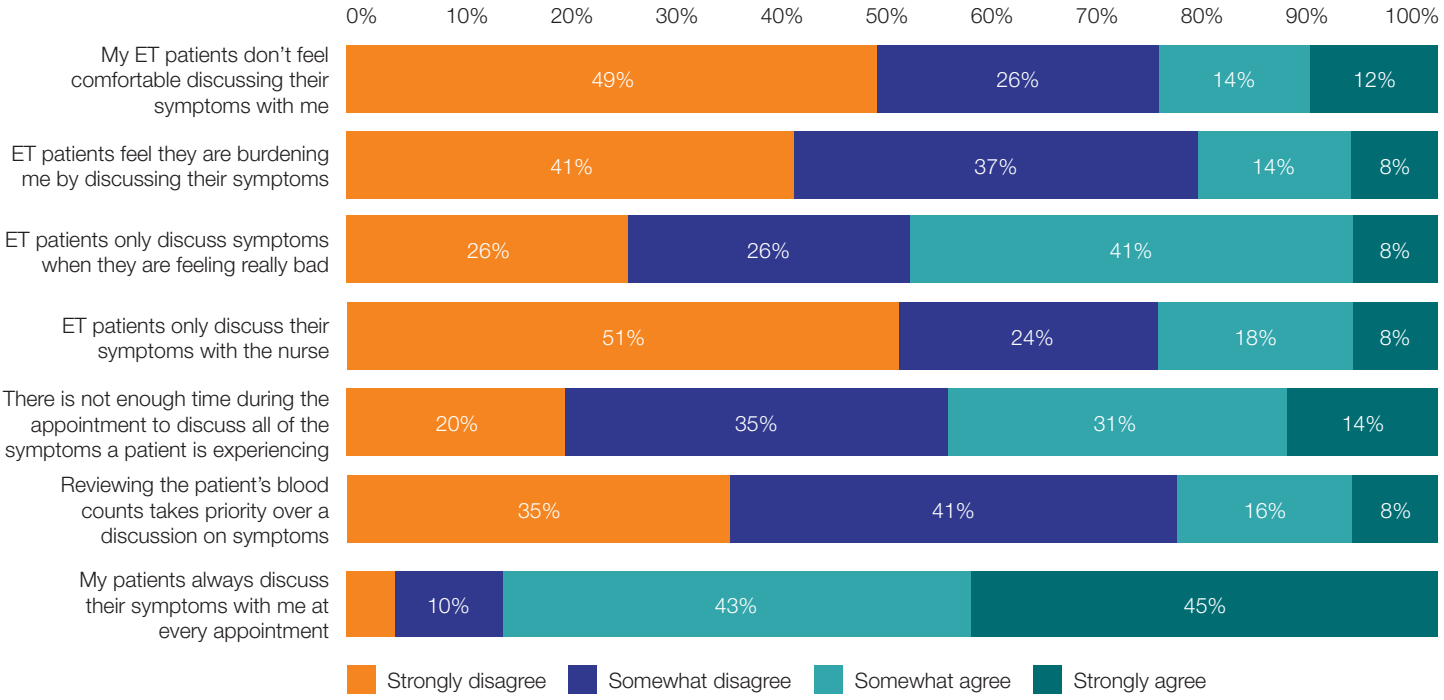


Figure 61. Question 51h-n: How much do you agree with the following statements? (n = 51) Note: Labels for data under 5% are not displayed

Attitudes Towards Communication Regarding Treatment

Although most patients reported a very positive relationship with their physician, there was some discordance worth mentioning. Among ET patients, 41% disagreed that their physician kept them informed about new treatment options; however, 92% of physicians reported that they kept their patients informed (Figures 62 and 63). Another 35% of ET patients disagreed that their physician had created a treatment plan for them, despite 94% of physicians reporting they had created a plan or established goals for their patients.

Patient-Reported Attitudes Towards Communication Regarding Treatment

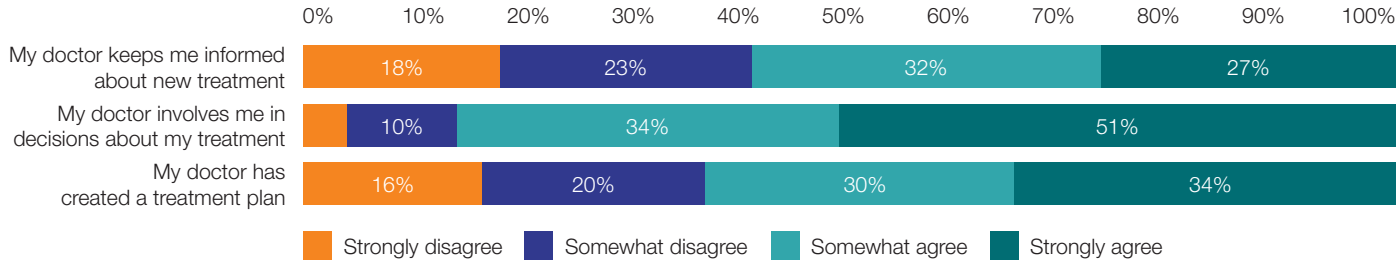


Figure 62. Question 47a-g: How much do you agree with the following statements? (n = 226)
Note: Labels for data under 5% are not displayed

Physician-Reported Attitudes Towards Communication Regarding Treatment

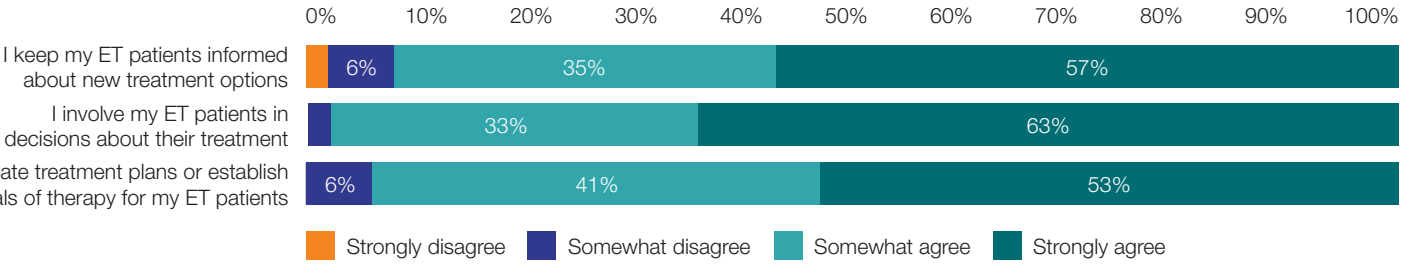


Figure 63. Question 51a-g: How much do you agree with the following statements? (n = 51)
Note: Labels for data under 5% are not displayed

Attitudes Towards Communication Regarding Treatment Goals

There seemed to be some discordance between patients and physicians regarding the understanding of treatment goals. Most patients felt their physicians completely understood and supported their treatment goals; however, physicians didn't feel as certain that patients understood their goals.

Most patients (53%) reported they felt their physician completely understood and supported their ET treatment goals (Figure 64). On the other hand, only 18% of physicians felt their patients completely understood their treatment goals. (Figure 65).

Patient Satisfaction With Physician's Understanding and Support of ET Treatment Goals

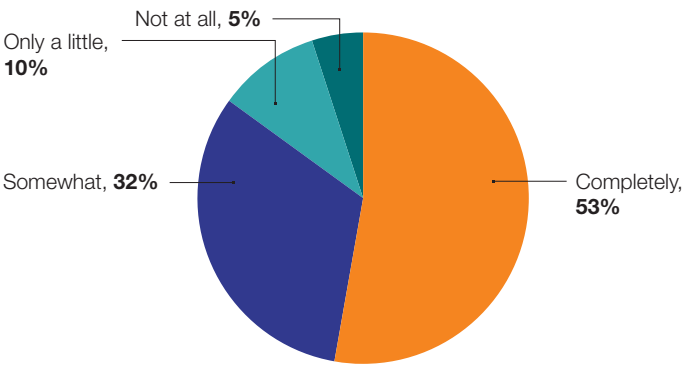


Figure 64. Question 44: How satisfied are you with your ET doctor's understanding and support of your goals for treatment? (n = 226)
Note: Individual values are rounded and may not total 100%

Patient's Understanding of ET Treatment Goals as Reported by Physician

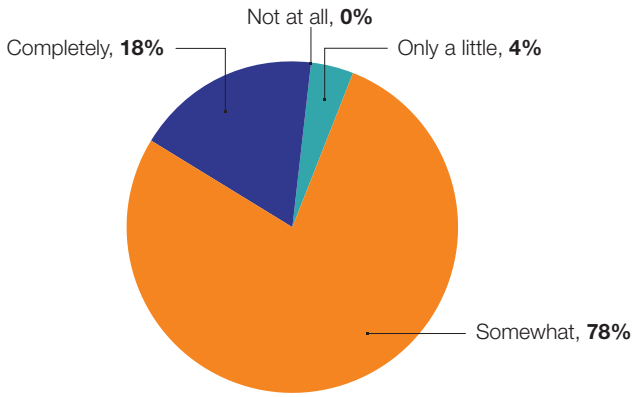


Figure 65. Question 46: How much do you feel that your ET patients understand your treatment goals? (n = 51)
Note: Individual values are rounded and may not total 100%

Attitudes Towards Patient–Physician Relationship

Patients and physicians were asked to describe various aspects of their patient–physician relationship in a series of statements related to their perceptions and experiences. Almost all patients surveyed (95%) agreed their physician was genuinely concerned about helping them and 94% of physicians agreed their ET patients felt they were genuinely concerned about them. They were also asked about various potential barriers to effective patient–physician communication that may exist. The vast majority of ET patients and physicians, did not feel any of the barriers applied to them.

Although most patients reported a very positive relationship with their physician, there was some discordance worth mentioning on a few aspects. Among ET patients, 41% disagreed that their physician kept them informed about new treatments; however, 92% of physicians reported keeping their patients informed about new treatment options (Figures 66 and 67). Another 35% of ET patients disagreed their physician created a treatment plan, despite 94% of physicians reporting that they had created a plan or established goals for their patients. Finally, 26% of patients disagreed their physician understood how much ET impacts their life, whereas 90% of physicians felt they did understand.

Patient-Reported Attitudes Towards Relationship With Physician

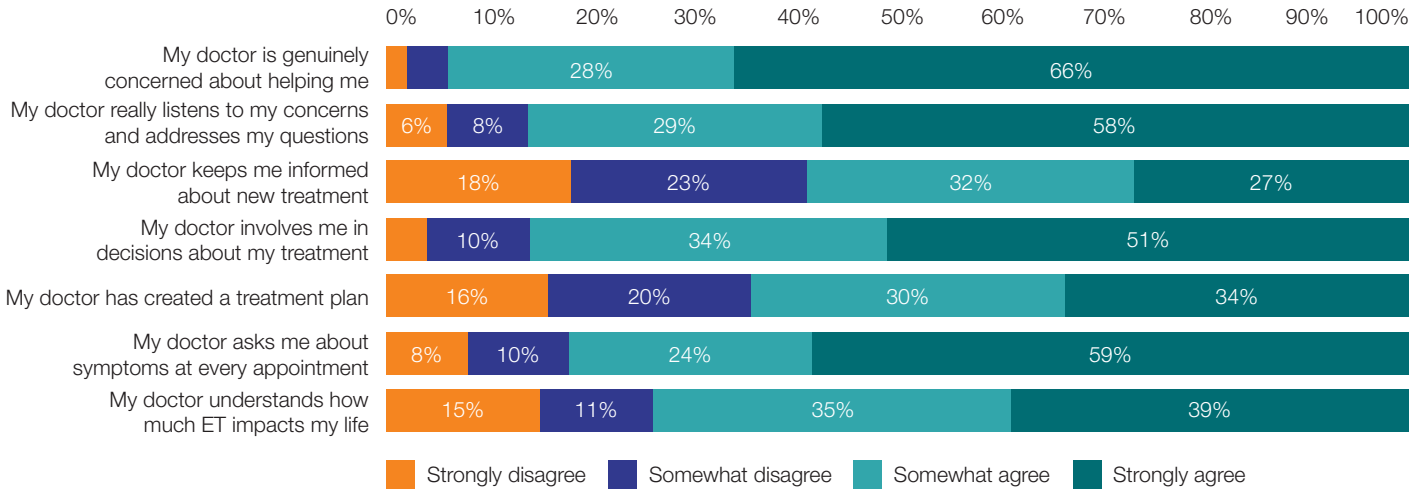


Figure 66. Question 47: How much do you agree with the following statements (n = 226) Note: Labels for data under 5% are not displayed

Physician-Reported Attitudes Towards Relationship With Patient

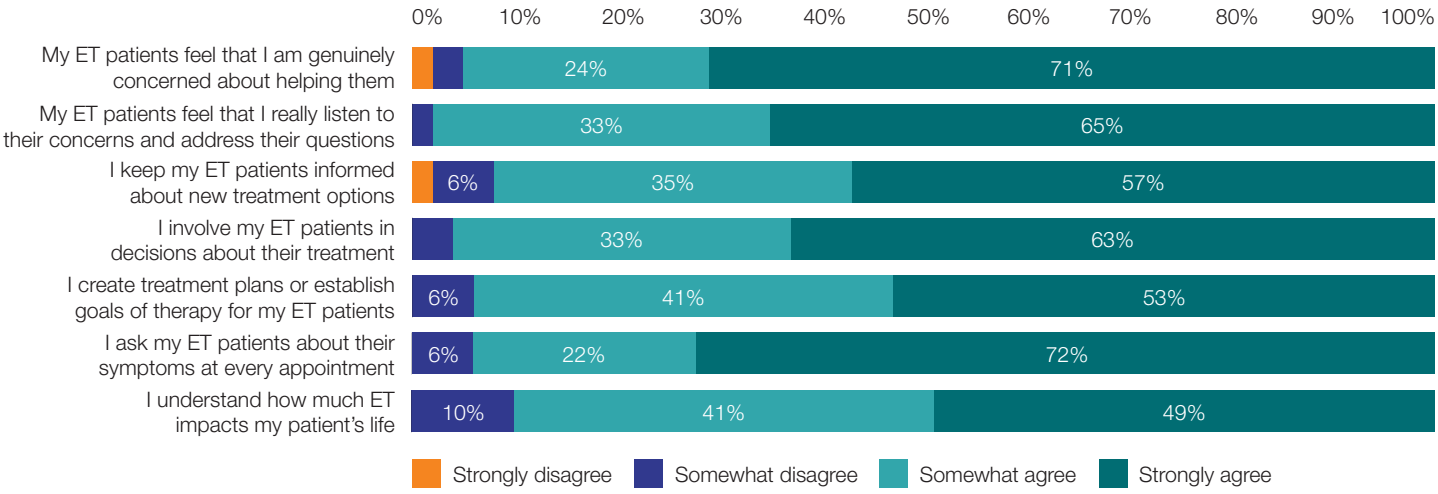


Figure 67. Question 51: How much do you agree with the following statements: (n = 51) Note: Labels for data under 5% are not displayed

Summary of Findings

- Nearly all patients (84%) were satisfied with their current physician's communication about their condition. Similarly, nearly all physicians (82%) were satisfied with their communication to patients about their condition (Figures 56 and 57).
- Patients reported they felt comfortable discussing their symptoms with their physician (82%), and most patients (79%) were certain on how to describe their symptoms to their physician (Figure 60).
- Among ET patients, 41% disagreed that their physician kept them informed about new treatment options; however, 92% of physicians reported that they kept their patients informed (Figures 62 and 63).
- There was some minor but meaningful discordance between patients and physicians when it came to treatment plans and establishing goals for therapy in that 94% of physicians reported they created a plan, whereas 35% of patients disagreed their physician had created a treatment plan for them (Figures 62 and 63).
- Most patients (53%) reported they felt their physician completely understood and supported their ET treatment goals (Figure 64). On the other hand, only 18% of physicians felt their patients completely understood their treatment goals. (Figure 65).
- Twenty-six percent of patients disagreed their physician understood how much ET impacts their life, whereas 90% of physicians felt they did understand (Figures 66 and 67).
- The vast majority of ET patients (95%) and physicians (94%) reported a very positive patient–physician relationship, and patients overwhelmingly felt their physician was genuinely concerned about helping them (Figures 66 and 67).

Types of Allied Health Involved in Patient Care

Patients were asked whether they had seen any other health care professionals (HCPs) within their current ET physician's office in the past 12 months. A Nurse Practitioner (NP) or Physician Assistant (PA) (30%) were the most common reported HCPs seen aside from their current physician. Another 40% of patients reported they had not seen any other HCP in the past 12 months (Figure 68).

As a follow-up question, patients were asked to report the extent to which the HCPs were involved in the counseling regarding ET treatment and management. For those patients who reported interacting with an NP or PA, 10% said they were involved a great deal (5 rating on a scale of 1–5) in their counseling.

Other HCPs Seen in the Past 12 Months for ET

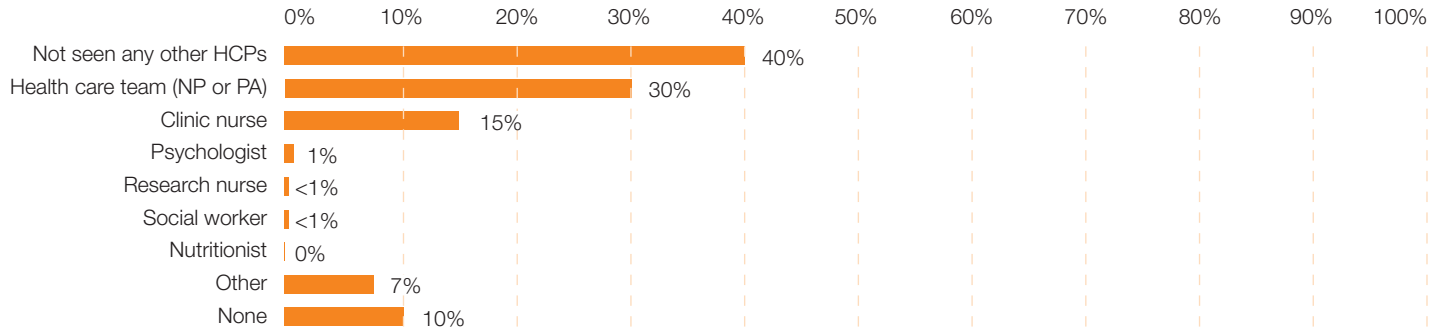


Figure 68. Question 48: Within your current ET doctor's office, have you seen any other health care professionals aside from your ET doctor about your condition in the last 12 months? (n = 226)
Note: "Other" category examples include primary doctor/general doctor, phlebotomist, internist, and cardiologist

Reliance on Caregivers

ET patients were asked whether they relied on a caregiver to help them with their condition. Most ET patients (85%) reported that they never relied on a caregiver, and another 7% said that they rarely relied on a caregiver (Figure 69).

Among those patients who relied on a caregiver to help with their condition, the overwhelming majority (71%) said their main caregiver was a spouse or partner (Figure 70).

Patient-Reported Reliance on Caregiver

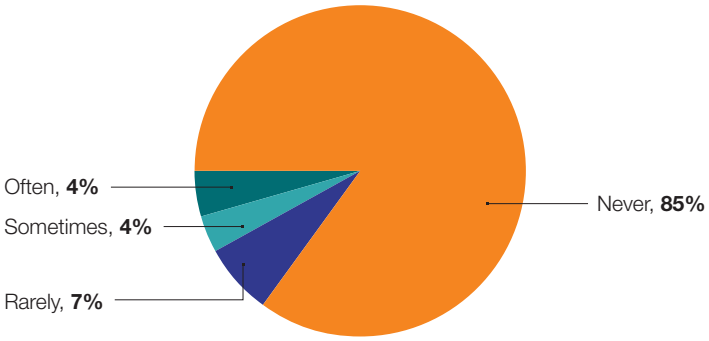


Figure 69. Question 20: Do you rely on a caregiver to help you with your diagnosis condition? (n = 226)
Note: Labels for data under 5% are not displayed

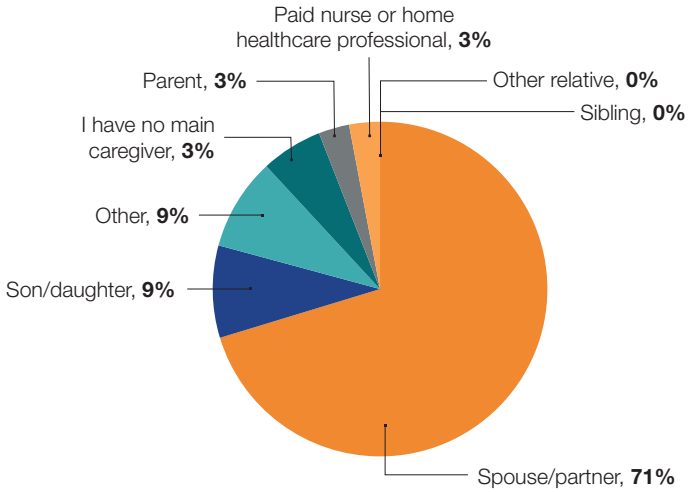


Figure 70. Question 20b: Who is your main caregiver who helps with your diagnosis? (n = 34)

Most Helpful Sources of ET Information

ET patients who did research to learn more about ET were asked where they found the most helpful information about their diagnosis. The Internet was identified by almost all ET patients (88%) as one of the most helpful sources (Figure 71). More than half of all patients (54%) also identified online discussion groups as a source for the most helpful information. Only 20% of information-seeking patients reported physicians' offices as one of the most helpful sources of information.

Patients who indicated use of the Internet as a source of information were also asked about which sites they visited most often for information on ET. They reported they most frequently visited the MPN Advocacy Group web sites (74%), hospital web sites (65%), and health websites (55%) (Figure 72). It should be noted that, because these types of web sites were used in recruiting the sample, the use of these web sites in the general population of ET patients may be overstated in this survey.

Most Helpful Sources of Information

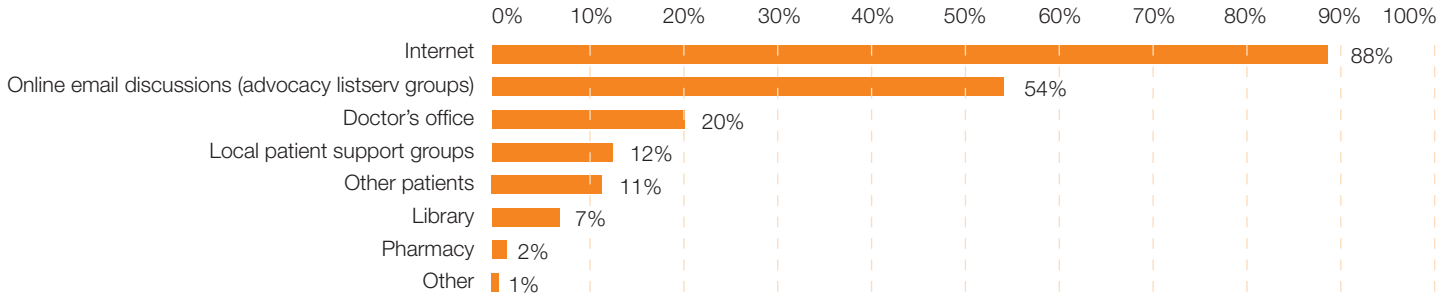


Figure 71. Question 51a: Where did you find the most helpful information about diagnosis? (n = 218)

Most Often Visited Web Sites for ET Information

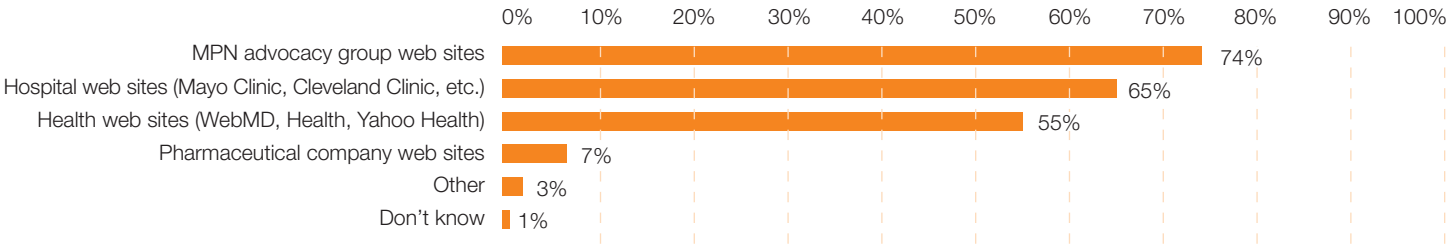


Figure 72. Questions 51b: What sites do you go to most often for information about diagnosis? (n = 193)

Patient Satisfaction With ET Information

An overwhelming majority of patients (90%) said they were satisfied with how informed they were about ET, and more than a third of those patients (36%) said they were very satisfied. A small minority of patients reported they were somewhat (8%) or very dissatisfied (2%) with how informed they were about their condition.

Patient-Reported Attitudes Towards Search for ET Information

ET patients were asked a series of questions regarding their attitudes towards their search for ET information. Overall, results showed that patients felt it did not take a lot of effort to get the information needed (69%) and they were not feeling frustrated during their search (69%) (Figure 73). Additionally, patients were not concerned with the credibility of the information (63%) and did not find the information confusing (73%). Patients also reported they found a lot of good information during their search (78%).

Patients were asked about how or where they preferred to receive their information, and 81% preferred to receive these materials directly from their physician, and 68% of patients preferred to learn about ET on the Internet. Seventy-two percent of patients agreed they were more trusting of information given to them by their physician.

Patient-Reported Attitudes Towards Search

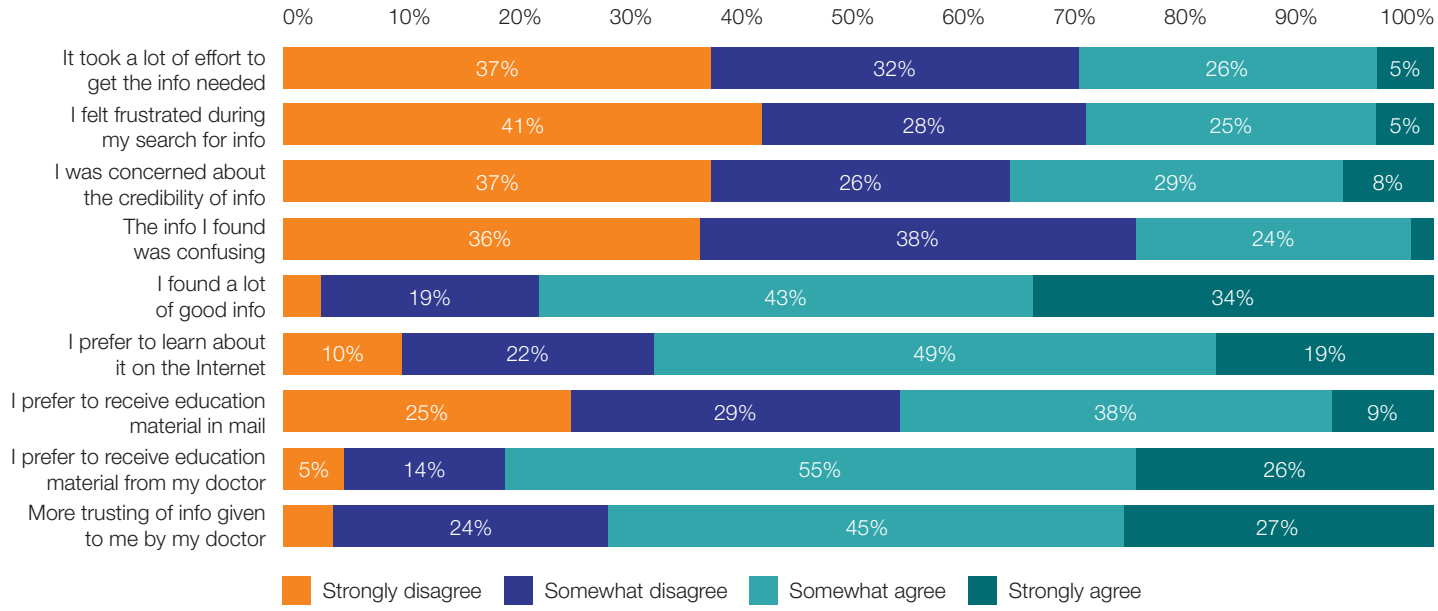


Figure 73. Question 55a–i: How much do you agree or disagree with each of the following? (n = 226)
Note: Labels for data under 5% are not displayed

Type of Health Care Coverage Reported by Patients

Almost all ET patients in the survey reported that they had some sort of health insurance or health plan to cover their health care costs (99%). Nearly half of patients (46%) reported their primary source of health coverage as group commercial insurance through an employer or union (Figure 74). Another 38% of patients reported Medicare as their health care coverage. (Figure 74).

Type of Health Care Coverage Reported by Patients

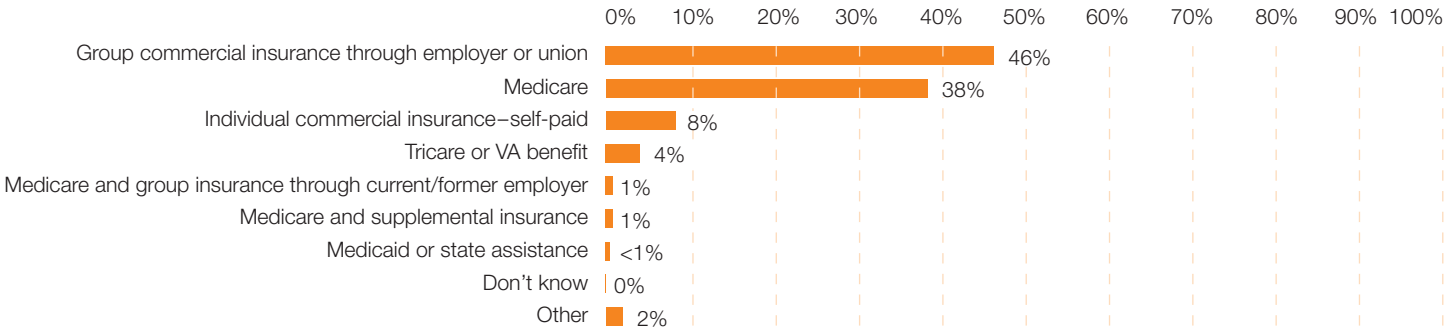


Figure 74. Question 56a: What best describes your type of coverage? (n = 223)
Note: Individual values are rounded and may not total 100%

Monthly Out-of-Pocket Prescription Drug Costs

Close to two thirds of ET patients (62%) reported that they paid out-of-pocket costs of $25 or less per month for prescription drug costs related to the treatment and management of their condition. A minority of patients (7%) paid more than $100 per month in out-of-pocket prescription drug costs related to their condition.

Summary of Findings

- An NP and PA (30%) were the most commonly reported HCPs seen aside from ET patient's current physician (Figure 68).
- Most ET patients (85%) reported that they never relied on a caregiver, and of the 15% who relied on a caregiver, 71% relied on a spouse or partner (Figures 69 and 70).
- The Internet was identified by almost all ET patients (88%) as one of the most helpful sources, and more than half of ET patients (54%) also identified online discussion groups as a source for the most helpful information (Figure 71).
- Overall, results showed that patients felt it did not take a lot of effort to get the information needed (69%) and were not frustrated during their search (69%) (Figure 73).

REFERENCES

1. Leukemia & Lymphoma Society. Essential Thrombocythemia Facts 2012. https://www.lls.org/content/nationalcontent/resourcecenter/freeeducationalmaterials/mpd/pdf/essentialthrombocythemiapdf. Accessed February 18, 2015.
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3. MPN Research Foundation. Essential Thrombocythemia. http://www.mpnresearchfoundation.org/Essential-Thrombocythemia. Accessed February 18, 2015.
4. Scherber, Dueck, Johansson, et al. The Myeloproliferative Neoplasms Symptom Assessment Form (MPN-SAF): international prospective validation and reliability trial in 402 patients. *Blood.* 2011;118(2):402-408.

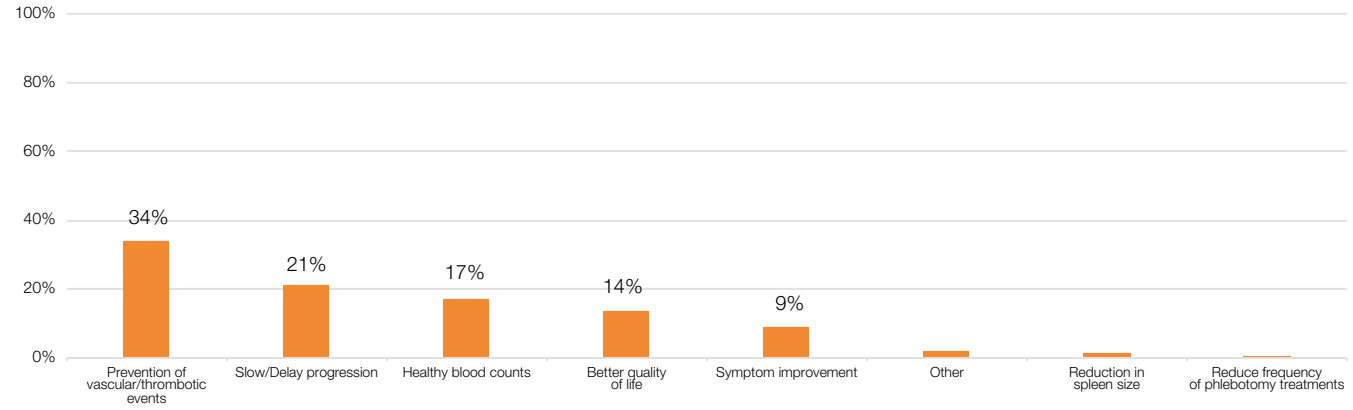
NOTES

NOTES

APPENDIX A

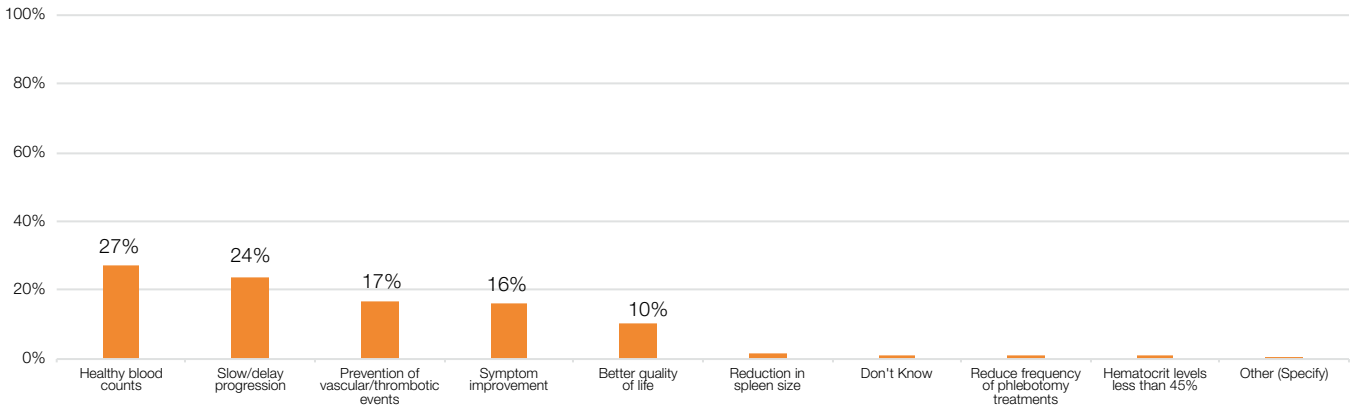
Additional Patient Data

Most Important Treatment Goal for ET Patients



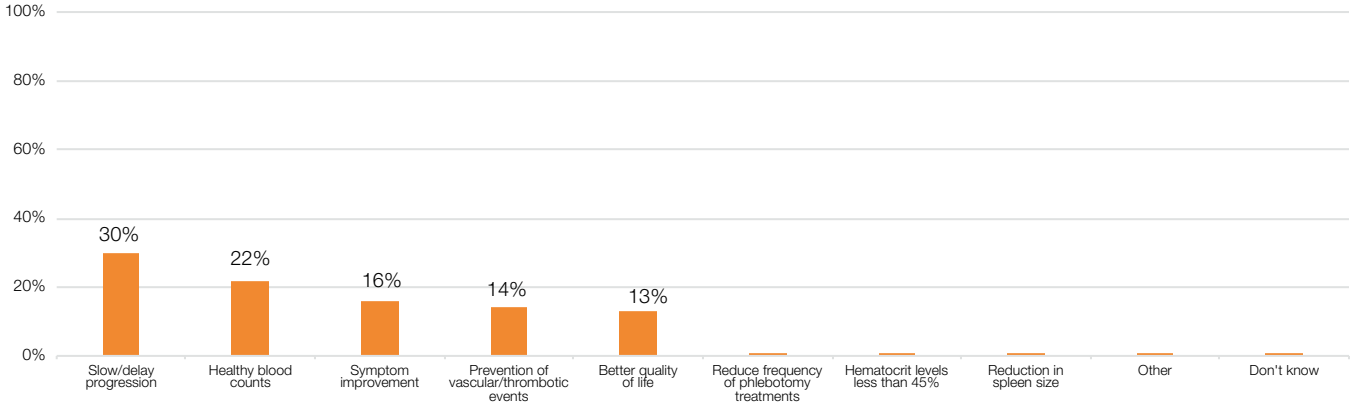
Q32: Other than a cure for diagnosis, what is your most important treatment goal for therapy? (n=226)
Note: Labels for data under 5% are not displayed

2nd Most Important Treatment Goal for ET Patients



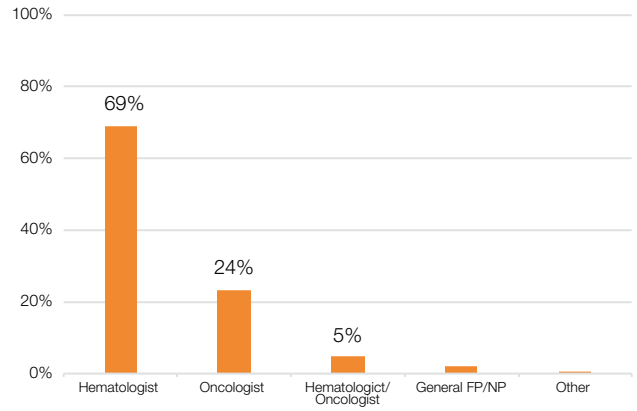
Q33: What is your next (2nd) most important treatment goal for therapy? (n=226)
Note: Labels for data under 5% are not displayed

3rd Most Important Treatment Goal for ET Patients

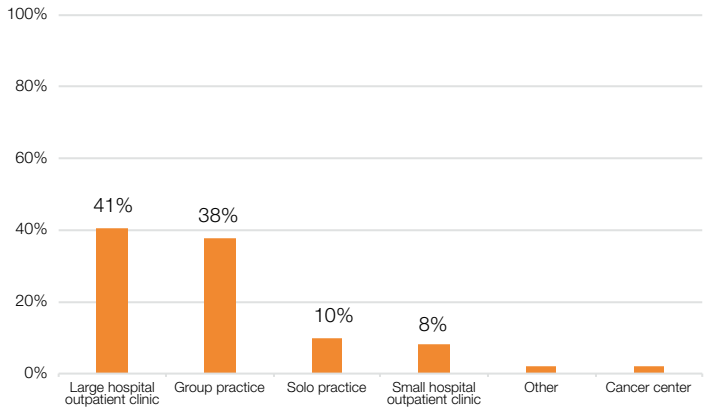


Q34: What is your next (3rd) most important treatment goal for therapy? (n=226)
Note: Labels for data under 5% are not displayed

Physician Specialty and Setting

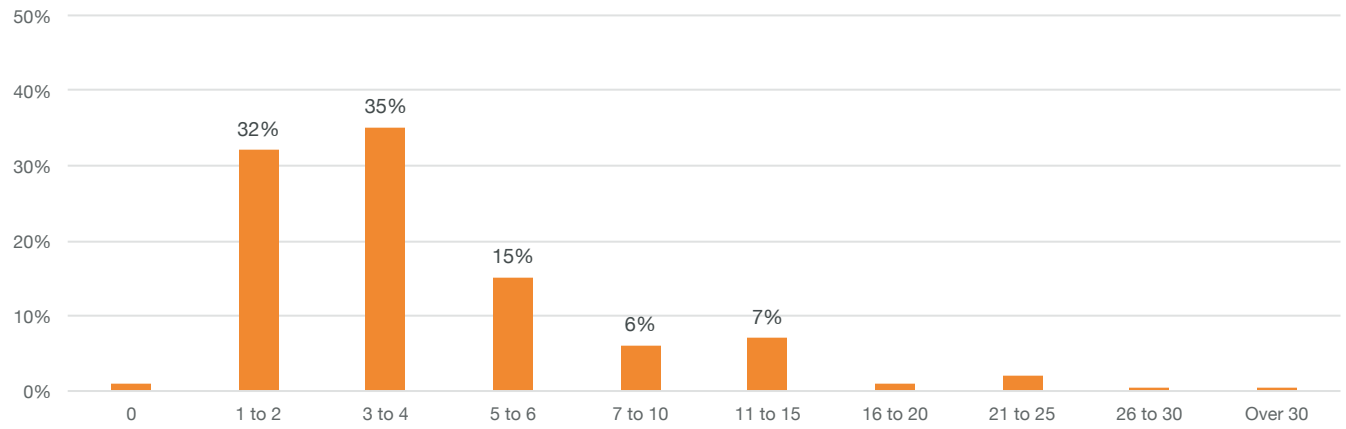


Q36: What is the medical specialty of the doctor that you see most often for your diagnosis? (n=226)
Note: Labels for data under 5% are not displayed



Q37: How would you describe the setting where you see this doctor most often for your diagnosis? (n=226)
Note: Labels for data under 5% are not displayed

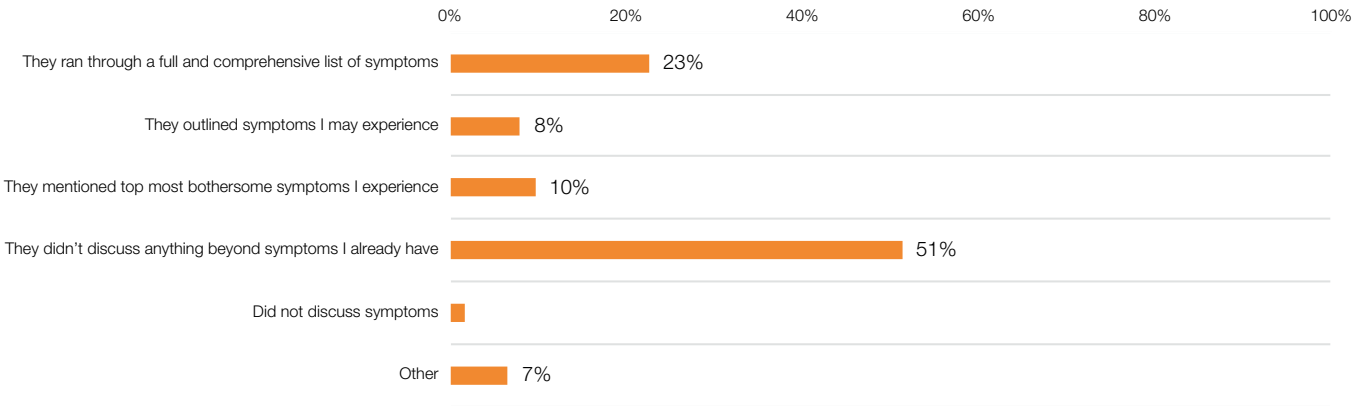
Frequency of Physician Visits in the Last 12 Months



Q38: How many times have you seen your doctor in the last 12 months? (n=226)
Note: Labels for data under 5% are not displayed

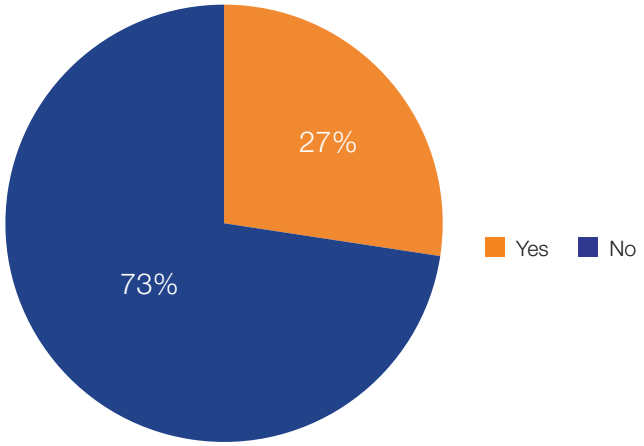
ADDITIONAL PATIENT DATA (CONT.)

Explanation of Symptoms and ET Disease Progression



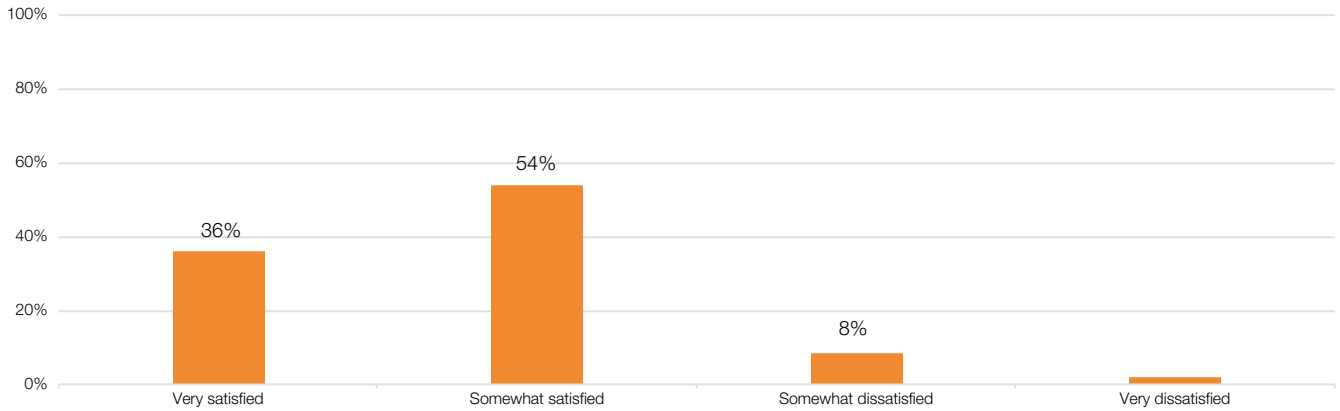
Q46: How did your doctor explain the potential diagnosis symptoms you may experience and overall progression of the disease? (n=226)
Note: Labels for data under 5% are not displayed

Learning More About Diagnosis



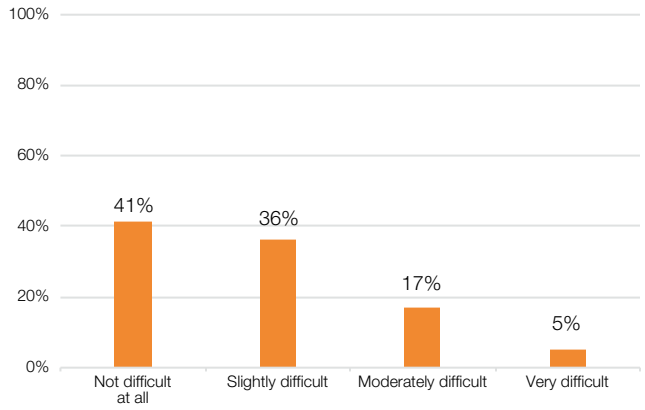
Q50: Did your doctor give you any information to learn more about your diagnosis? (n=226)

Patient Satisfaction With How Informed They Are About ET

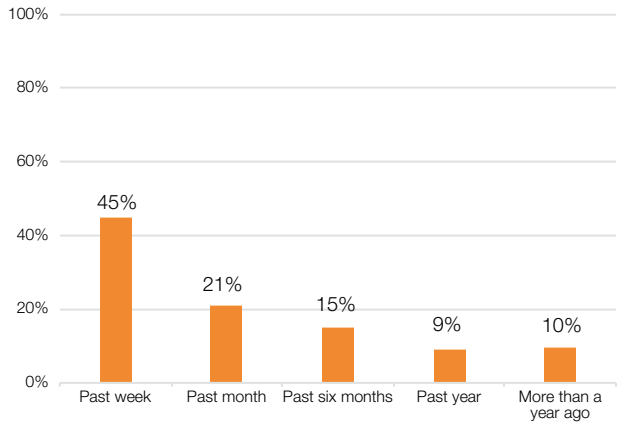


Q52: How satisfied are you currently with how informed you are about ET? (n=226)
Note: Labels for data under 5% are not displayed

Difficulty and Frequency of Research

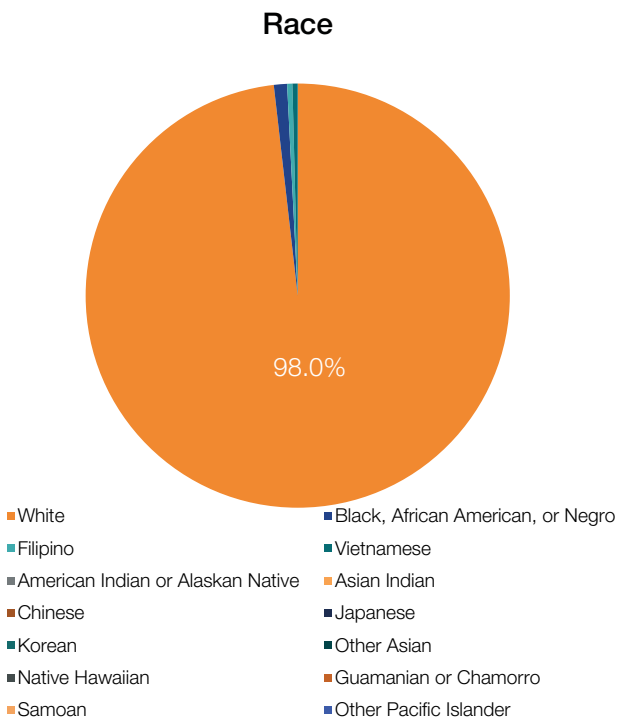


Q53: How difficult was it to find good information about diagnosis? (n=226)
Note: Labels for data under 5% are not displayed

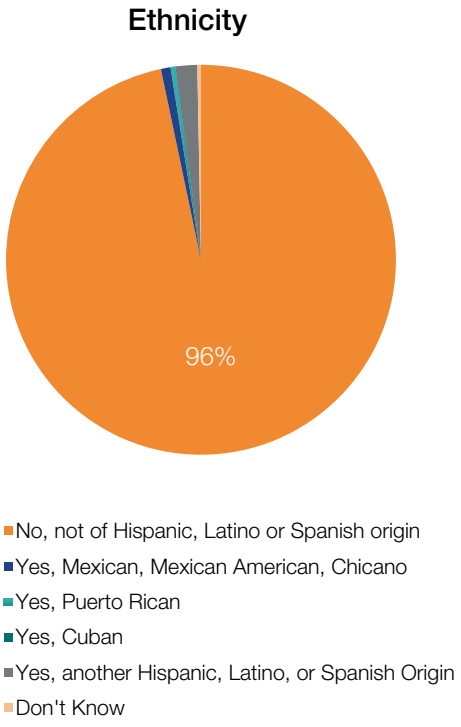


Q54: When was the most recent time you looked for information about diagnosis or its treatment? (n=226)
Note: Labels for data under 5% are not displayed

Race and Ethnicity



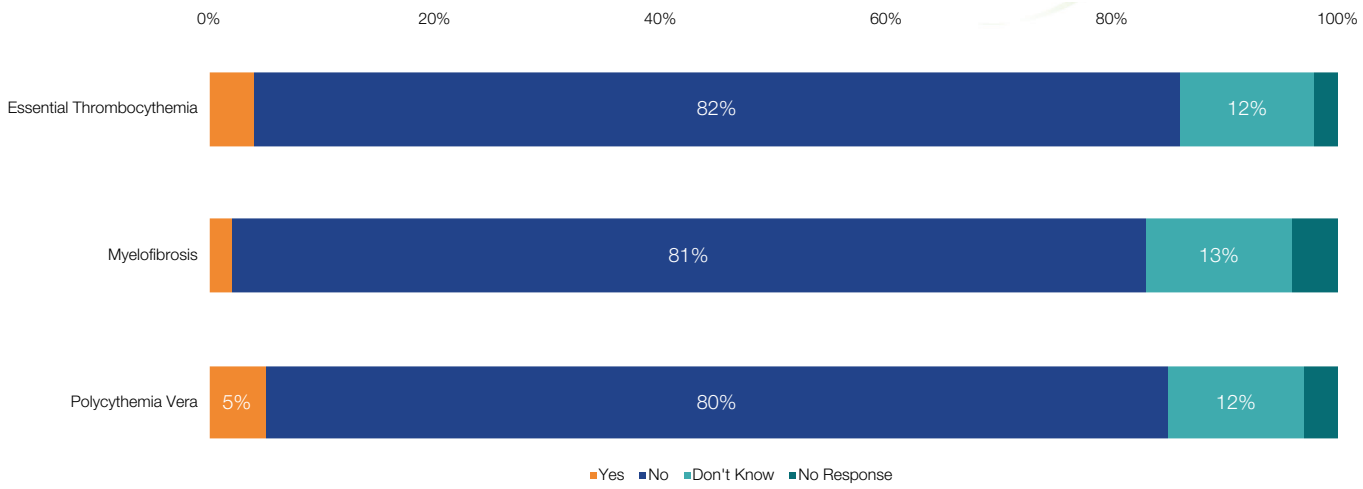
Q60: Select your race from the following (n=226)
Note: Labels for data under 5% are not displayed



Q59: Are you of Hispanic, Latino or Spanish origin? (n=226)
Note: Labels for data under 5% are not displayed

ADDITIONAL PATIENT DATA (CONT.)

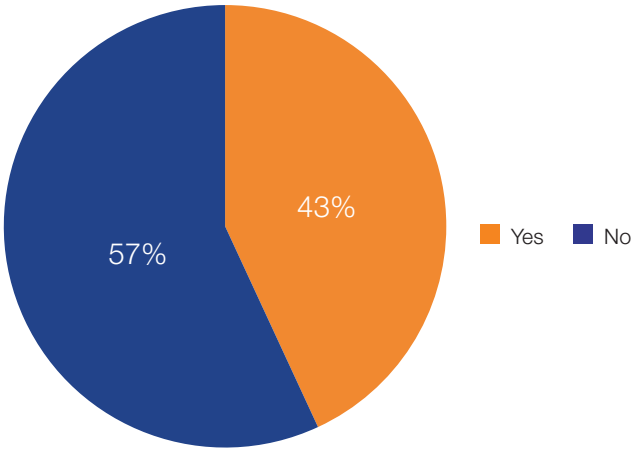
Family History of an MPN Diagnosis



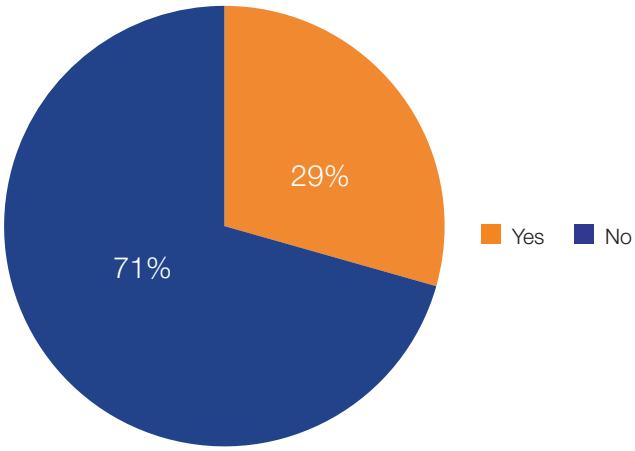
Q64a-c: Has anyone else in your family ever been diagnosed with: essential thrombocythemia, polycythemia vera, myelofibrosis? (n=226)
Note: Labels for data under 5% are not displayed

Additional Physician Data

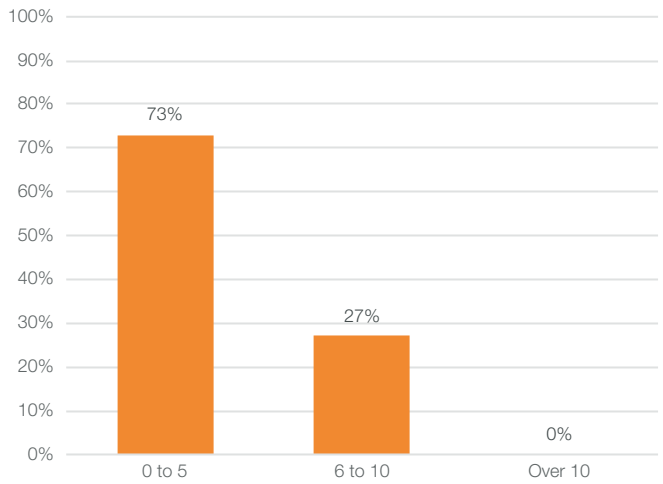
Stem Cell Transplant Program and MPN Clinical Trial Experience



Q6: Does your center have a stem cell transplant program? (n=51)



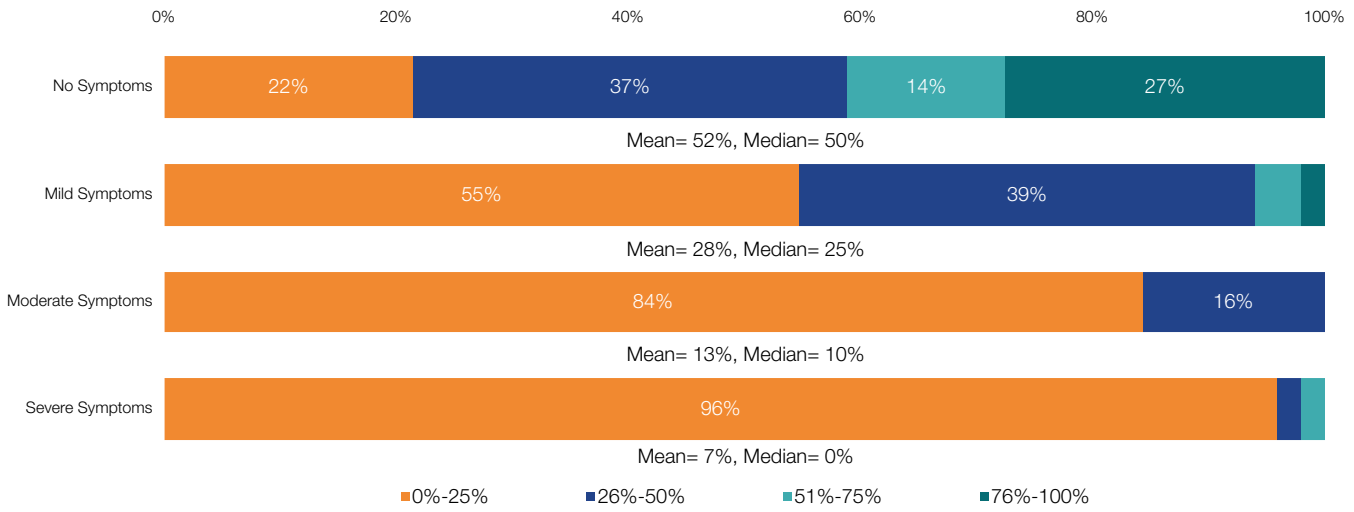
Q7: Has your clinic/practice ever participated as a study site for MPN clinical trials? (n=51)
Note: Labels for data under 5% are not displayed



Q8: How many MPN clinical trials has your clinic/practice been involved in the last 2 years? (n=15)
Note: Labels for data under 5% are not displayed

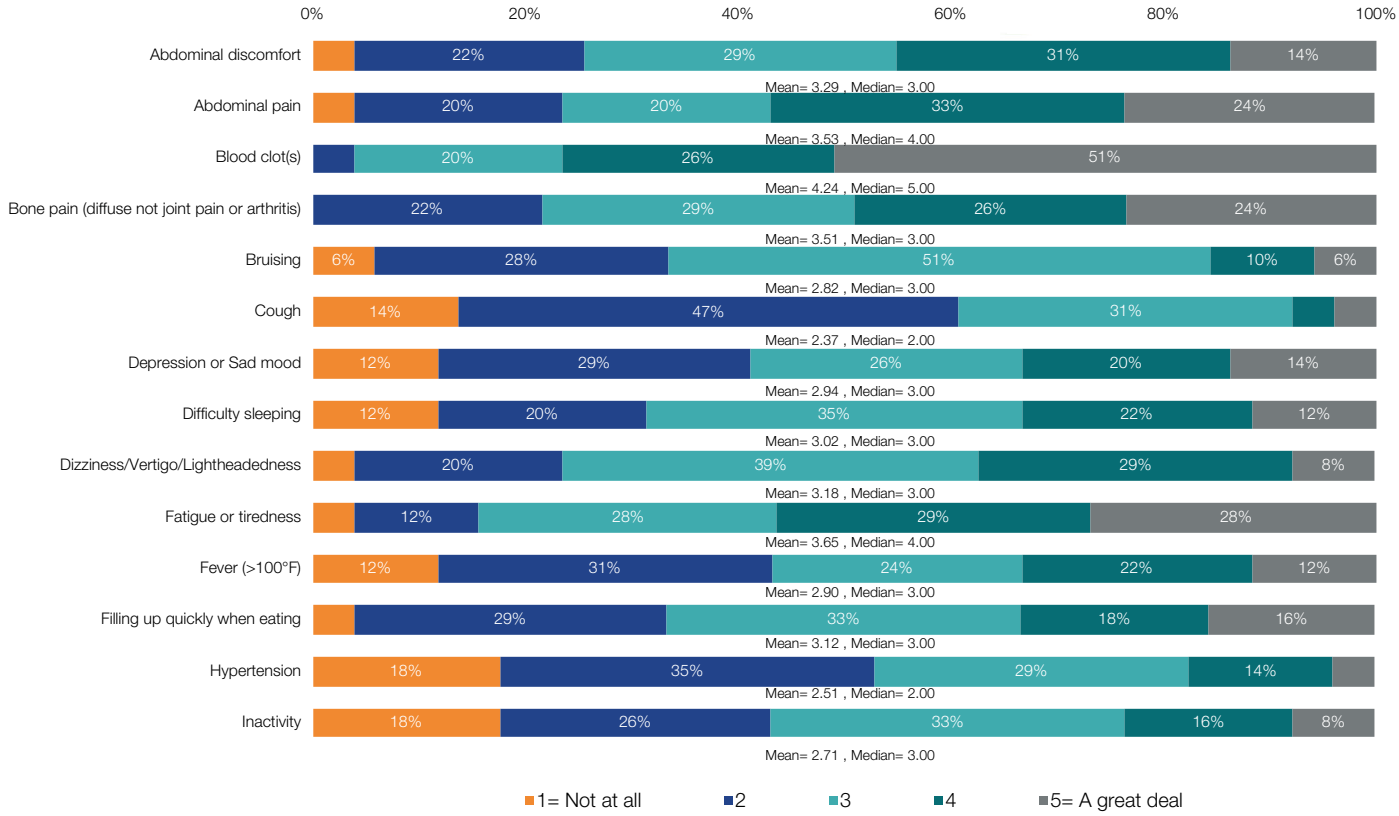
ADDITIONAL PHYSICIAN DATA (CONT.)

Symptom Severity in All ET Patients Currently Under Physician Care



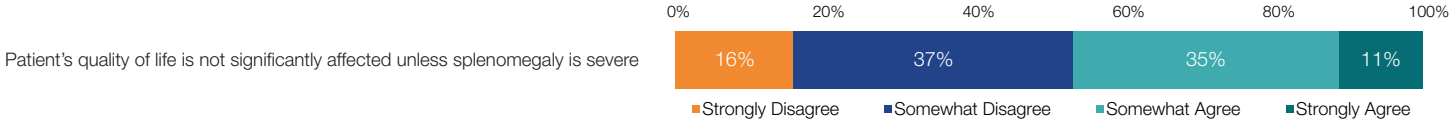
Q14a-d: Out of 100%, what proportion of all ET patients that you currently follow have [no, mild, moderate or severe] symptoms? (n=51)
Note: Labels for data under 5% are not displayed

Physician-Reported Symptoms Impacting Patients QOL



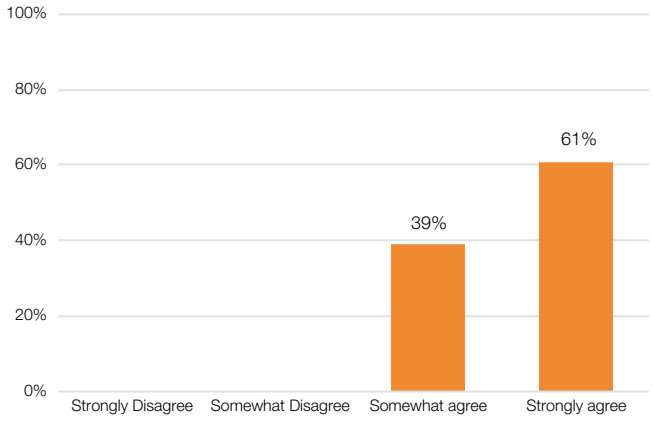
Q16a-r: On a scale from 1(not at all) to 5 (a great deal), which of the following symptoms have a major negative effect on an ET patient's quality of life. (n=51)
Note: Labels for data under 5% are not displayed

Symptom and Splenomegaly Impact on QOL

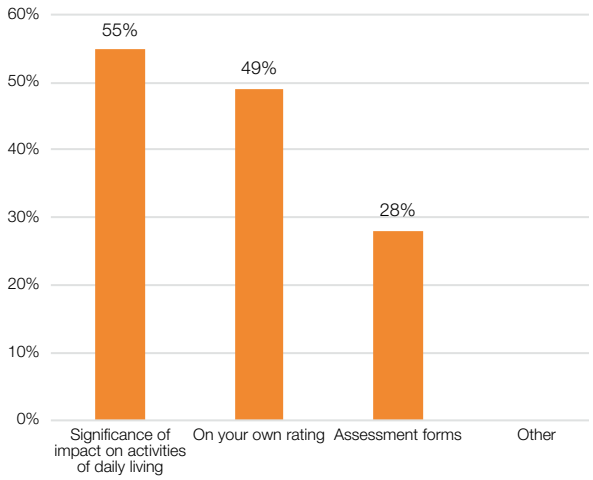


Q23: Agree or disagree with the following statements: Patient's quality of life is not significantly affected unless splenomegaly is severe (n=51)
Note: Labels for data under 5% are not displayed

Comfort Assessing Symptoms and Approaches Most Utilized by Physicians

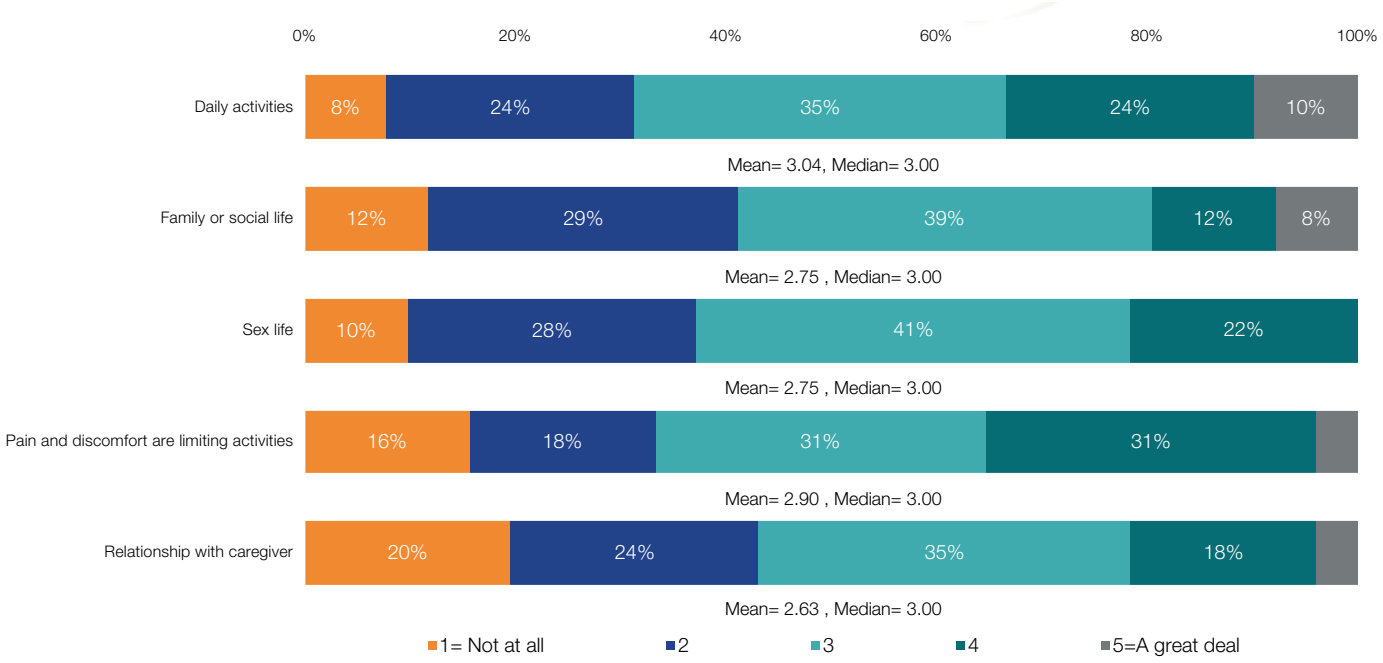


Q24: Agree or disagree with the following statements: I am comfortable assessing my patients' symptoms (n=51)
Note: Labels for data under 5% are not displayed



Q30: What tool(s) or approaches do you use to assess symptom severity in your patients? (n=51)
Note: Labels for data under 5% are not displayed

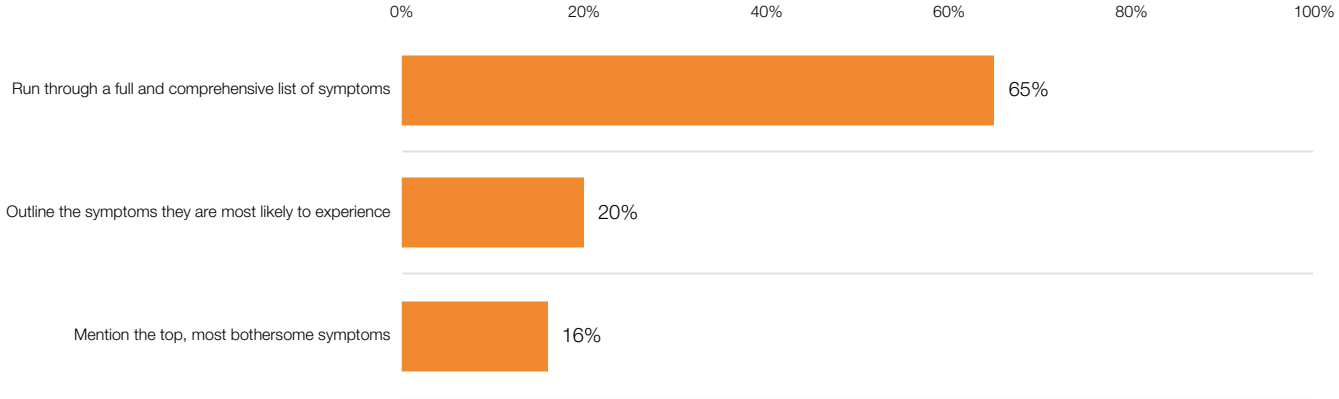
Interference with Activities of Daily Living



Q25a-e: To what extent do you feel that ET interferes with the following activities in a patient's life? (n=51)
Note: Labels for data under 5% are not displayed

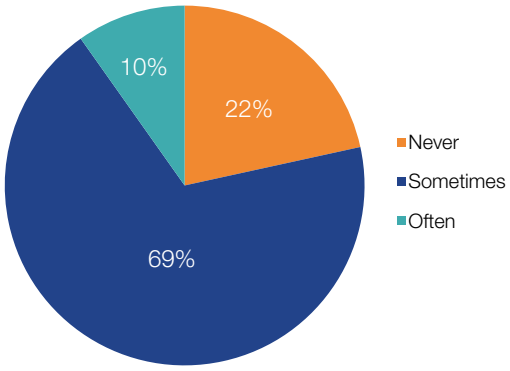
ADDITIONAL PHYSICIAN DATA (CONT.)

Physician-Reported Explanation of Symptoms and ET Disease Progression



Q28: During an average patient visit, how do you discuss the symptoms that an ET patient might experience? Do you..... (n=51)
Note: Labels for data under 5% are not displayed

Noncompliance With Physician Recommendation



Q41: How often does an ET patient not wish to comply with your primary treatment recommendation? (n=51)
Note: Labels for data under 5% are not displayed. Individual values are rounded and may not total 100%