

Patient-Reported Outcomes Data From Patients With Essential Thrombocythemia Enrolled in the MOST Study

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Introduction

- Essential thrombocythemia (ET) is a Philadelphia chromosome–negative myeloproliferative neoplasm (MPN) characterized by clonal platelet production and an increased risk of thrombotic and hemorrhagic events¹
- Approximately 50% of patients with ET present with systemic manifestations of the disease²
 - ET is commonly associated with microvascular (eg, headache, dizziness) and macrovascular (eg, stroke, myocardial infarction) complications, as well as constitutional symptoms (eg, fatigue, night sweats, weight loss)²
 - Patients with ET experience substantial symptom burden and reduced self-reported functional status, quality of life (QoL), activities of daily living, and work-related productivity³
- There are limited patient-reported outcome (PRO) data pertaining to symptom burden in patients with ET and its impact on QoL
- The Myelofibrosis and Essential Thrombocythemia Observational Study (MOST) is an ongoing study designed to collect data on demographics, disease burden, PROs, and management of patients with ET or myelofibrosis (MF) in clinical practices throughout the United States

Objective

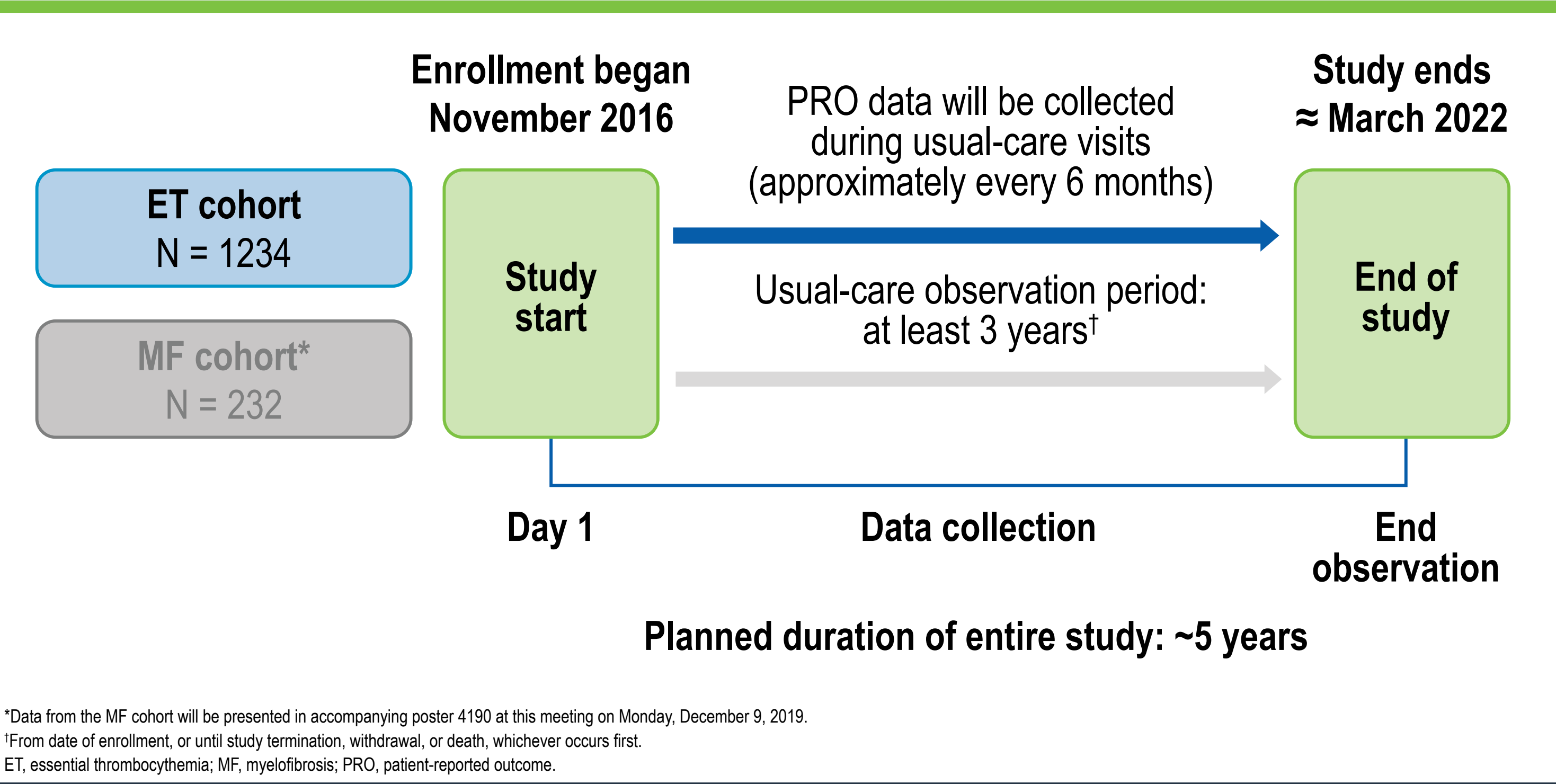
- To describe PROs at enrollment from patients with ET in the MOST study

Methods

Patients and Study Design

- MOST is an ongoing multicenter, prospective, observational, noninterventional, nonrandomized, open-label study in patients with clinical diagnoses of specific risk categories of MF or ET in community and academic study sites in the United States (Figure 1)
- Eligible patients had a clinical diagnosis of ET and were either classified as high risk (based on age ≥60 years or history of thrombotic events) or were currently receiving ET-directed therapy (excluding aspirin only)
- Key exclusion criteria were participation in blinded investigational drug trials, life expectancy ≤6 months, or diagnosis of other myeloid malignancies

Figure 1. MOST Study Design



*Data from the MF cohort will be presented in accompanying poster 4190 at this meeting on Monday, December 9, 2019.
†From date of enrollment, or until study termination, withdrawal, or death, whichever occurs first.
ET, essential thrombocythemia; MF, myelofibrosis; PV, polycythemia vera; TE, thrombotic event.

Assessments

- Patient-reported symptom burden was assessed with the MPN Symptom Assessment Form Total Symptom Score (MPN-SAF TSS), composed of 10 items (fatigue, early satiety, abdominal discomfort, inactivity, concentration problems, night sweats, itching, bone pain, fever [≥100°F], and weight loss)
 - Numbness/tingling was included on the questionnaire, but was not included in the TSS calculation
 - Symptom severity was graded from 0 (absent) to 10 (worst imaginable), with a possible TSS ranging from 0 to 100
- Health-related QoL was evaluated with the European Organization for Research and Treatment of Cancer Core Quality of Life Questionnaire (EORTC QLQ-C30 v3.0), consisting of 5 multi-item functional scales, 3 multi-item symptom scales, 6 additional single-symptom items, and a multi-item global health/QoL scale
 - For functional and global health/QoL scales, higher scores indicate higher functioning and better global health/QoL, respectively
 - Higher scores for symptom scales/items represent higher symptom burden

Statistical Analyses

- Data were summarized with descriptive statistics

Results

Patients

- The MOST study was initiated on November 29, 2016; as of March 29, 2019, 1234 patients with ET were enrolled from 124 sites
 - 794 patients with PRO data at the time of enrollment qualified for this analysis (data cutoff date: June 17, 2019)
- Baseline demographics and clinical characteristics at enrollment are summarized in Table 1
 - Median age was 70 years (range, 19–93), and 79% of patients were ≥60 years of age
 - The median time from ET diagnosis to enrollment was 4.1 years (range, 0–37)
 - 689 patients (87%) were classified as high risk at the time of enrollment

Table 1. Patient Demographics and Clinical Characteristics at Enrollment

Variable	N = 794
Age, median (range), years	70 (19–93)
Age, n (%)	
≥60 years	631 (79)
Sex, n (%)	
Female	539 (68)
Race, n (%)	
White	703 (90)
Black	56 (7)
Other*	26 (3)
Missing	9
Employment status at diagnosis, n (%)	
Employed full time	286 (36)
Employed part time	43 (5)
Retired	324 (41)
Homemaker	35 (4)
Student	2 (0.3)
Unable to work/disabled due to ET	5 (1)
Unable to work/disabled due to other	21 (3)
Other	19 (2)
Unknown	56 (7)
Missing	3
Family history of MF/ET/PV, n (%)	31 (4)
Median time from ET diagnosis to enrollment, years	4.1 (0–37)
Time from ET diagnosis to enrollment (range), n (%)	
<1 year	172 (22)
1–<5 years	268 (34)
5–<10 years	172 (22)
≥10 years	181 (23)
ET risk status category, n (%)	
Low risk	105 (13)
High risk	689 (87)
Age ≥60 years and no history of TE	478 (69)
Age <60 years and history of TE	58 (8)
Age ≥60 years and history of TE	153 (22)

*Other includes Asian (n = 7), American Indian (n = 4), and other (n = 15).
ET, essential thrombocythemia; MF, myelofibrosis; PV, polycythemia vera; TE, thrombotic event.

Patient-Reported Outcomes

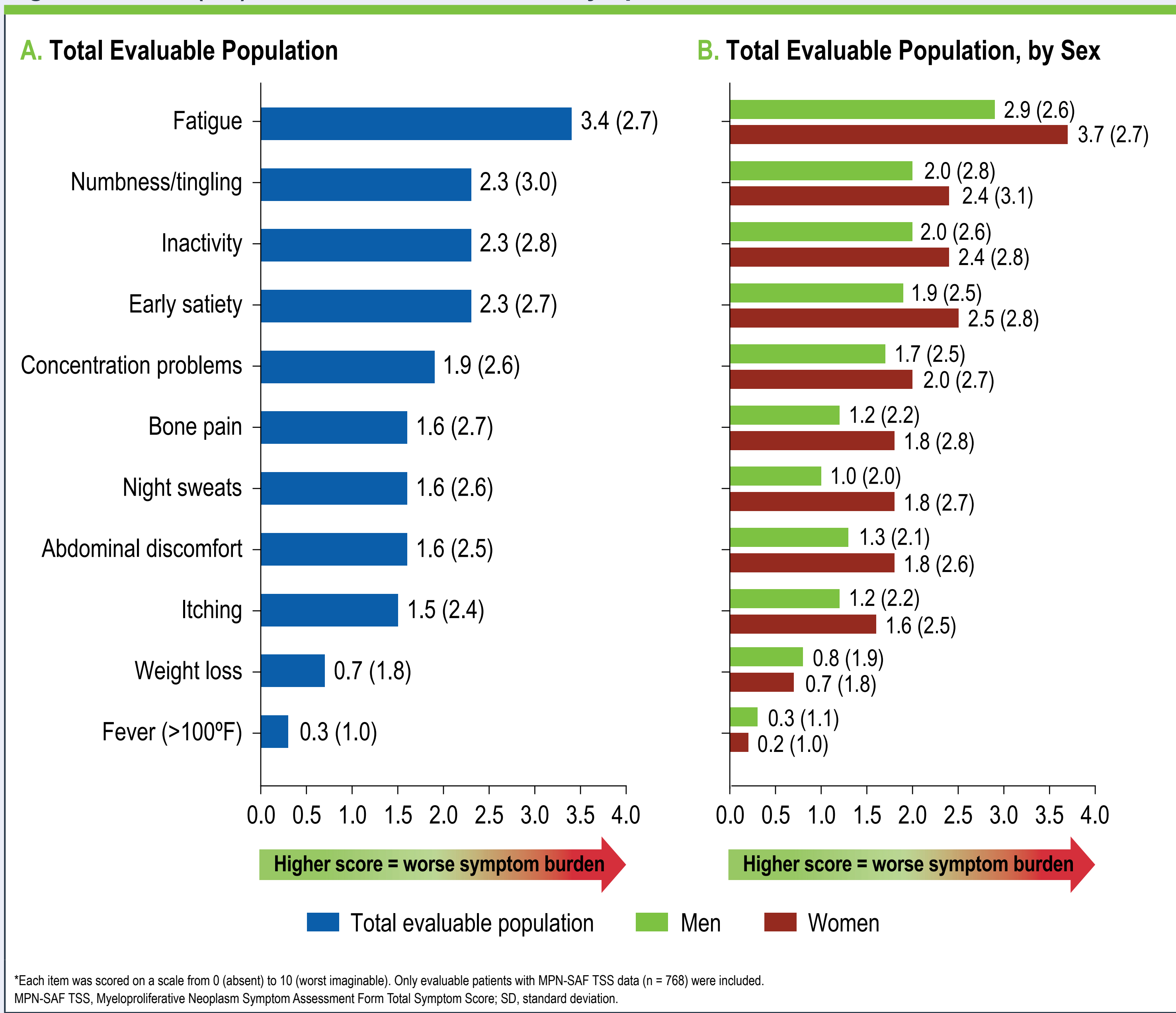
MPN-SAF TSS

- At enrollment, 768 patients completed the MPN-SAF TSS
- Mean TSS was 17.1; 33% of patients had TSS ≥20
 - The highest mean individual symptom scores were fatigue (3.4), numbness/tingling (2.3), inactivity (2.3), and early satiety (2.3) (Figure 2A)
 - Women had higher mean TSS than men (18.5 vs 14.2) and had higher mean individual symptom scores, except for weight loss and fever (Figure 2B)
- The most frequently reported severe symptoms (ie, score ≥7) were fatigue (17%), numbness/tingling (14%), and inactivity (11%) (Figure 3)

EORTC QLQ-C30

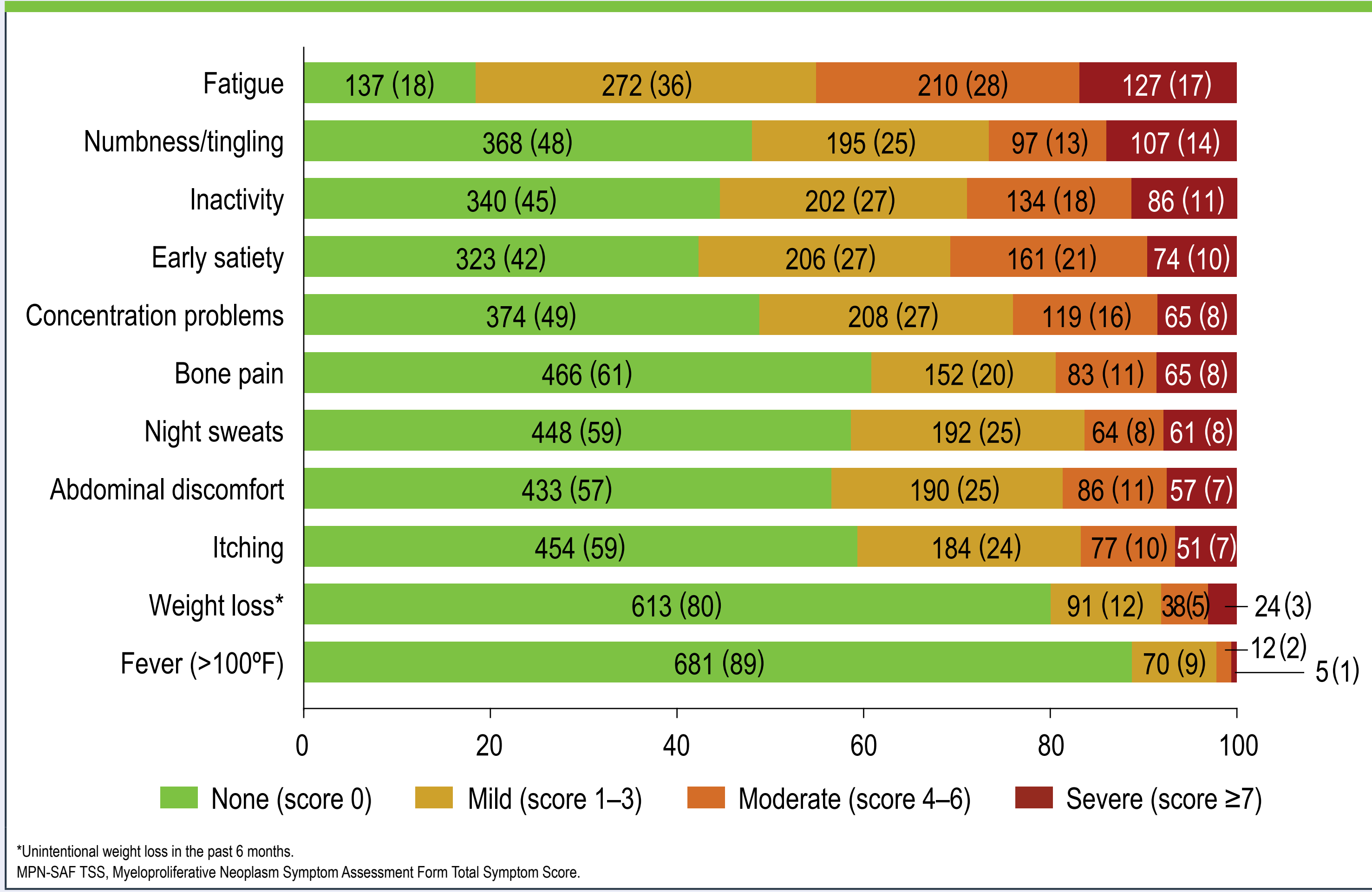
- At enrollment, 794 patients completed the EORTC QLQ-C30
- The highest mean symptom scale/individual scores (score ≥15) were fatigue (29.6), insomnia (28.6), pain (22.1), dyspnea (17.2), and constipation (15.7) (Figure 4A)
- The average symptom scores were higher in women than men (Figure 4B)

Figure 2. Mean (SD) MPN-SAF TSS Individual Symptom Scores*



*Each item was scored on a scale from 0 (absent) to 10 (worst imaginable). Only evaluable patients with MPN-SAF TSS data (n = 768) were included.
MPN-SAF TSS, Myeloproliferative Neoplasm Symptom Assessment Form Total Symptom Score; SD, standard deviation.

Figure 3. MPN-SAF TSS Symptom Severity at Enrollment, n (%)



*Unintentional weight loss in the past 6 months.
MPN-SAF TSS, Myeloproliferative Neoplasm Symptom Assessment Form Total Symptom Score.

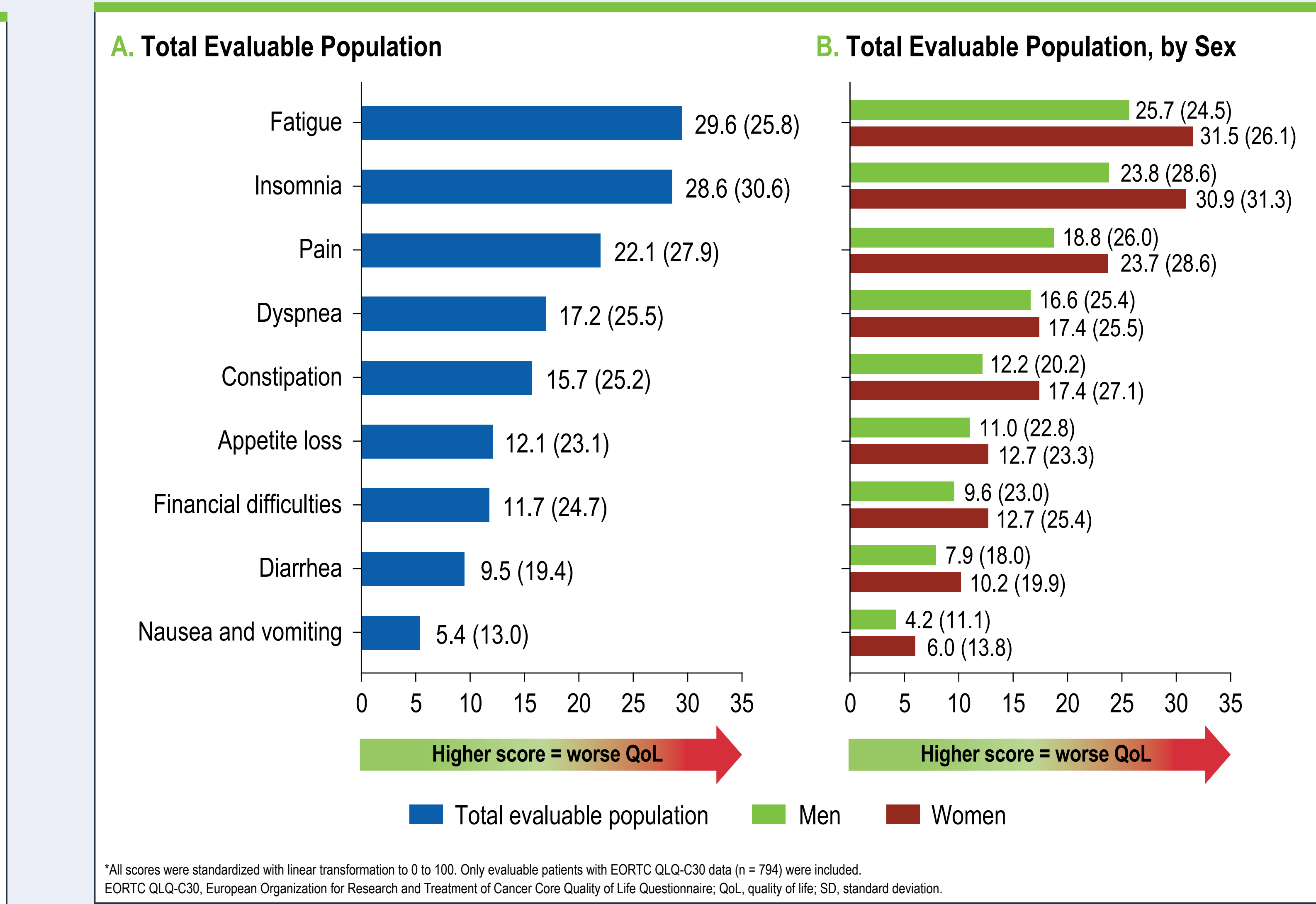
Conclusions

- MOST is the first prospective, longitudinal, observational study assessing the demographics, disease burden, PROs, and management of patients with ET or MF in clinical practice throughout the United States**
- Symptom burden (MPN-SAF TSS) and QoL scores (EORTC QLQ-C30) in MOST were similar to prior published studies^{4,5}**
- Patients with ET reported symptom burden at the time of enrollment, with fatigue being the most common and most severe symptom**
 - Notably, numbness/tingling, which is not included in the MPN-SAF TSS, was one of the most frequently reported severe symptoms for patients with ET
- ET symptoms appeared to have a negative effect on QoL**
 - Women reported higher symptom burden than men on both the MPN-SAF TSS and the EORTC QLQ-C30
 - This same sex difference was also noted in patients with polycythemia vera enrolled in REVEAL, an observational study in polycythemia vera⁶
- Additional prospective analyses from MOST will continue to increase the understanding of symptom burden and its impact on QoL in patients with ET**

Disclosures

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Figure 4. Mean (SD) EORTC QLQ-C30 Symptom Scores*

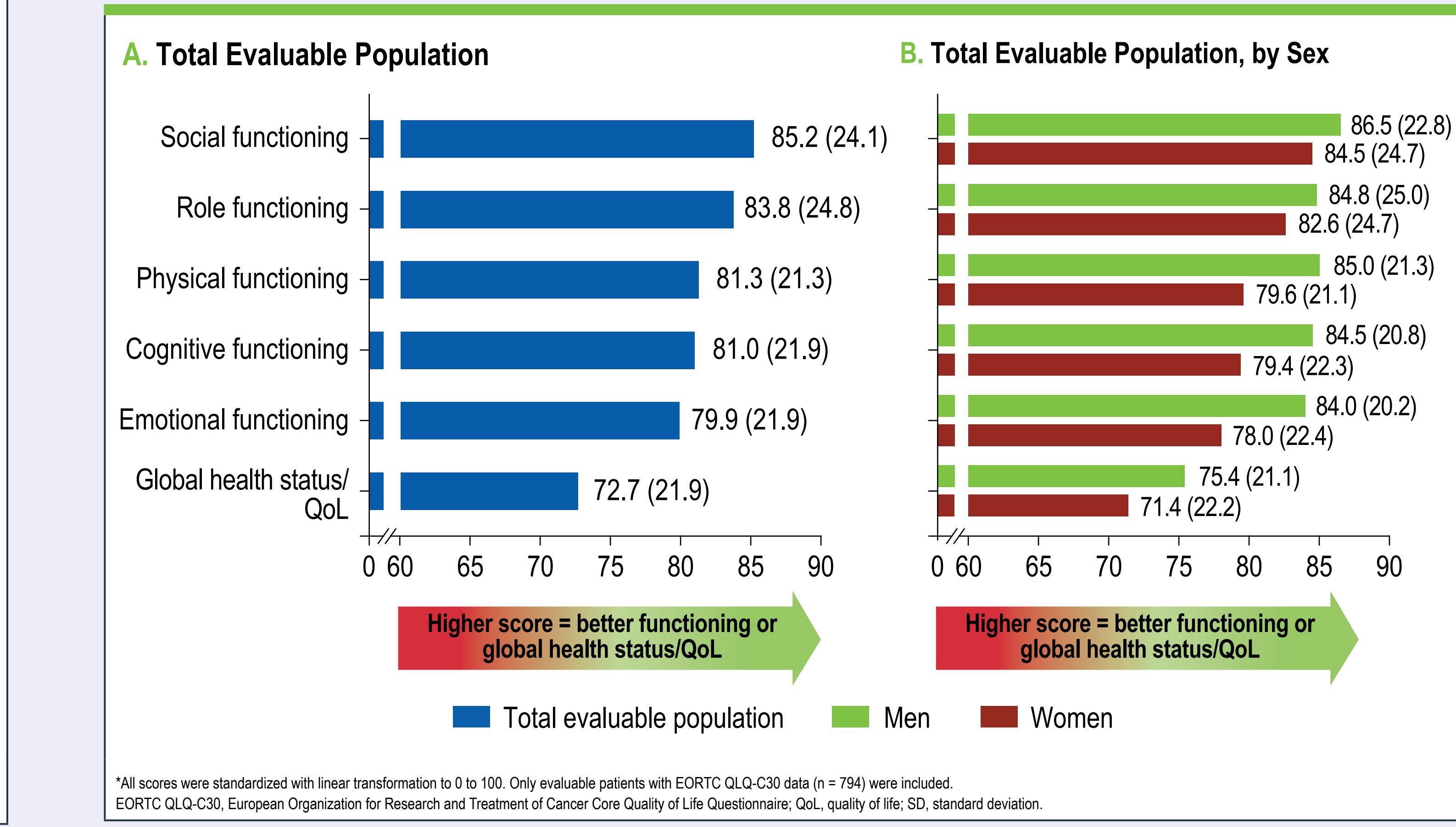


*All scores were standardized with linear transformation to 0 to 100. Only evaluable patients with EORTC QLQ-C30 data (n = 794) were included.
EORTC QLQ-C30, European Organization for Research and Treatment of Cancer Core Quality of Life Questionnaire; QoL, quality of life; SD, standard deviation.

EORTC QLQ-C30 Subscales

- The mean global health status/QoL score was 72.7; functional scores ranged from 79.9 for emotional functioning to 85.2 for social functioning (Figure 5A)
- The average functional scale scores indicate lower functioning in women compared with men (Figure 5B)

Figure 5. Mean (SD) EORTC QLQ-C30 Subscales*



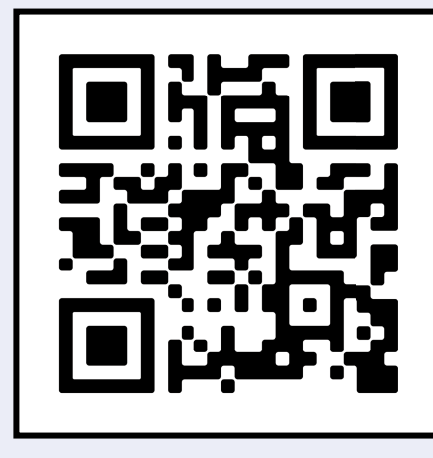
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EORTC QLQ-C30, European Organization for Research and Treatment of Cancer Core Quality of Life Questionnaire; QoL, quality of life; SD, standard deviation.

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