MPN-186

# Community Versus Academic Practice in Essential Thrombocythemia and Myelofibrosis – Differences in Clinical Characteristics, Diagnosis, Treatment Patterns, and Symptom Burden: Analysis of Data From the MOST Study


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## Background

- Essential thrombocythemia (ET) is a Philadelphia chromosome–negative myeloproliferative neoplasm (MPN) characterized by clonal blood cell proliferation, excessive platelet production, and an increased risk of thrombotic and hemorrhagic events[1]
  - ET is associated with increased risk of vascular complications as well as constitutional symptoms[2]
  - Treatment of ET is risk-adapted, focused on preventing thrombosis and bleeding, and minimizing symptoms[3,4]
- Myelofibrosis (MF) is a chronic Philadelphia chromosome–negative MPN characterized by extramedullary hematopoiesis, bone marrow fibrosis, splenomegaly, constitutional symptoms, cytopenias, and diminished quality of life[5]
  - Median survival varies markedly depending on disease risk status[6-12]
  - MF treatment decisions are thus risk-adapted and may involve a variety of factors including prognosis and symptomology[13]
- In the United States, 2 primary oncology practice types exist: academic centers (AC) and community centers (CC)
  - Differences between practice types include payer sources, exposure to medical trainees, and federal and state funding
  - How these environments influence MPN patient care remains to be explored
- The **M**yelofibrosis and Essential Thrombocythemia **O**bservational **ST**udy (MOST; NCT02953704) is an ongoing study designed to collect data on demographics, disease burden, patient-reported outcomes, and management of patients with ET or MF in clinical practices throughout the United States

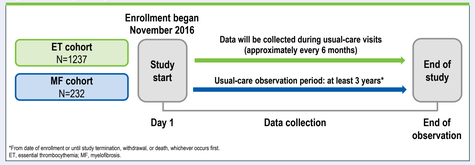
## Objective

- To examine baseline characteristics, diagnosis, treatment patterns, and symptom burden in patients with ET and MF being cared for in AC and CC

## Study Design and Results

- Ongoing longitudinal, noninterventional, prospective, observational study in patients with clinical diagnoses of specific risk categories of ET or MF enrolled at clinical practices throughout the United States (**Figure 1, Table 1, Table 2**)
- Patient disposition, baseline demographics, and disease characteristics are shown in **Figure 2** and **Tables 3** and **4**

**Figure 1. Study Design**




*From date of enrollment or until study termination, withdrawal, or death, whichever occurs first.
ET, essential thrombocythemia; MF, myelofibrosis.

**Table 1. Key Patient Eligibility Criteria**

| | ET cohort | MF cohort |
|---|---|---|
| Inclusion Criteria | • ≥18 years of age<br>• Clinical diagnosis of ET<br>• Classified by ELN criteria[3] as having:<br>– High-risk ET (≥60 years of age and/or thromboembolic event history)<br>*OR*<br>– Low-risk ET if receiving ET-directed therapy (not including aspirin only) | • ≥18 years of age<br>• Clinical diagnosis of primary or secondary MF (including primary MF, post–polycythemia vera MF, or post-ET MF)<br>• Classified by DIPSS[7] as having:<br>– Low-risk MF<br>*OR*<br>– INT-1–risk MF (by >65 years of age alone) |
| Exclusion Criteria | • Participation in an active, blinded study or Incyte-funded investigational/interventional drug trial<br>• Life expectancy ≤6 months | • Diagnosis of secondary acute myeloid leukemia, myelodysplastic syndrome, chronic myelogenous leukemia, or secondary thrombocytosis |

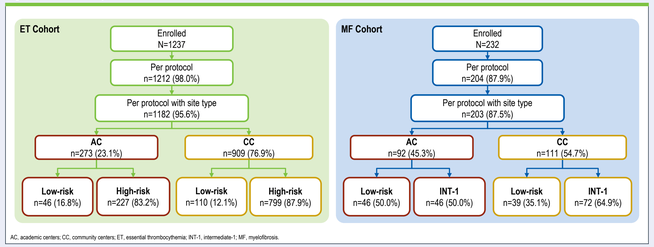
DIPSS, Dynamic International Prognostic Scoring System; ELN, European LeukemiaNet; ET, essential thrombocythemia; INT-1, intermediate-1; MF, myelofibrosis.

**Table 2. Key Study Assessments and Statistical Analyses**

| | |
|---|---|
| Assessments | • Data from patient records and physician-reported baseline data (demographics, diagnosis, treatment, and symptom burden) were collected as previously characterized[14] during usual-care visits over a planned observation period of 36 months from 24 AC and 82 CC<br>• Patient-reported symptom burden was assessed with the Myeloproliferative Neoplasm Symptom Assessment Form[15]<br>– TSS was calculated (0 [absent] to 100 [highest level of severity]) by summing scores for 10 items (fatigue, early satiety, abdominal discomfort, inactivity, concentration problems, night sweats, itching, bone pain, fever [>100°F], and weight loss)<br>– Numbness/tingling was also included on the questionnaire but was not included in the TSS calculation |
| Statistical Analyses | • Descriptive statistics were used to summarize data<br>• *P* values were calculated using:<br>– Fisher exact test or Chi-square test for categorical data<br>– *t* test for continuous data<br>• Missing data were not imputed |

AC, academic centers; CC, community centers; TSS, total symptom score.

**Figure 2. Patient Disposition**




AC, academic centers; CC, community centers; ET, essential thrombocythemia; INT-1, intermediate-1; MF, myelofibrosis.

**Table 3. Baseline Demographics**

| | ET Cohort (N=1182) | | MF Cohort (N=203) | |
|---|---|---|---|---|
| Characteristic | AC (n=273) | CC (n=909) | AC (n=92) | CC (n=111) |
| Age, mean (SD), years*,† | **63.7 (14.4)** | 69.2 (12.0) | **65.4 (9.7)** | 68.5 (10.7) |
| Women, n (%) | 173 (63.4) | 601 (66.1) | 46 (50.0) | 55 (49.5) |
| Race, n (%)* | | | | |
| White* | **228 (84.4)** | 813 (90.3) | 79 (85.9) | 99 (89.2) |
| Black | 23 (8.5) | 65 (7.2) | 7 (7.6) | 7 (6.3) |
| Other‡ | 19 (7.0) | 22 (2.4) | 6 (6.5) | 5 (4.5) |
| Ethnicity, n (%)*,† | | | | |
| Hispanic or Latino*,† | **11 (4.0)** | 78 (8.6) | **0** | 7 (6.3) |
| Not Hispanic or Latino | 244 (89.4) | 773 (85.0) | 85 (94.4) | 96 (86.5) |
| Other§ | 18 (6.6) | 58 (6.4) | 5 (5.6) | 8 (7.2) |
| Education level, n (%)*,† | | | | |
| ≤High school | 60 (28.0) | 318 (39.5) | 11 (15.9) | 37 (38.5) |
| >High school | 154 (72.0) | 488 (60.5) | 58 (84.1) | 59 (61.5) |
| Employment status, n (%)* | | | | |
| Employed (full- or part-time) | 117 (43.2) | 256 (28.2) | 41 (45.1) | 33 (29.7) |
| Retired | 116 (42.8) | 517 (56.9) | 40 (44.0) | 61 (55.0) |

Significant differences are shown in bold: †ET cohort, AC versus CC: age *P*<0.0001; race *P*=0.0039; White *P*<0.0104; ethnicity *P*=0.0085; Hispanic or Latino *P*=0.0124; education level *P*<0.0001; employment status *P*=0.0001. ‡MF cohort, AC versus CC: age *P*<0.0297; ethnicity *P*=0.0206; Hispanic or Latino *P*=0.0175; education level *P*=0.0002. §Other includes Asian, American Indian/Alaskan Native, and Other. ¶Other includes not reported, unknown, and other.
AC, academic centers; CC, community centers; ET, essential thrombocythemia; MF, myelofibrosis; SD, standard deviation.

**Table 4. Baseline Disease Characteristics**

| | ET Cohort (N=1182) | | MF Cohort (N=203) | |
|---|---|---|---|---|
| Characteristic | AC (n=273) | CC (n=909) | AC (n=92) | CC (n=111) |
| BM biopsy/aspiration at diagnosis, n/N (%) | 149/270 (55.2) | 466/891 (52.3) | 84/92 (91.3) | 103/111 (92.8) |
| Mutation testing at diagnosis, n/N (%) | 216/268 (80.6) | 690/889 (77.6) | 73/92 (79.3) | 91/109 (83.5) |
| Mutation testing prior to enrollment, n/N (%) | 216/273 (79.1) | 690/909 (75.9) | 73/92 (79.3) | 91/111 (82.0) |
| *JAK2* V617–positive | 115/166 (69.3) | 402/553 (72.7) | 43/57 (75.4) | 49/70 (70.0) |
| *CALR*-positive | 36/56 (64.3) | 58/101 (57.4) | 19/23 (82.6) | 14/22 (63.6) |
| *MPL*-positive | 5/37 (13.5) | 19/88 (21.6) | 2/15 (13.3) | 2/15 (13.3) |
| Triple-negative (*JAK2* V617, *CALR*, *MPL*) | 6/12 (50.0) | 8/32 (25.0) | 0 | 3/8 (37.5) |
| Palpable spleen, n/N (%) | 20/205 (9.8) | 46/707 (6.5) | 29/73 (39.7) | 27/88 (30.7) |
| Laboratory tests, n/N (%) | | | | |
| Leukocytes, G/L >11 | 24/248 (9.7) | 104/860 (12.1) | 25/88 (28.4) | 28/100 (28.0) |
| Leukocytes, G/L <4 | **33/248 (13.3)** | **60/860 (7.0)*** | 11/88 (12.5) | 5/100 (5.0) |
| Platelets, G/L >600 | 59/250 (23.6) | 162/888 (18.2) | 8/87 (9.2) | 16/102 (15.7) |
| Platelets, G/L <150 | 5/250 (2.0) | 38/888 (4.3) | 17/87 (19.5) | 19/102 (18.6) |

Significant differences are shown in bold: *P<0.0026, ET cohort AC versus CC.
AC, academic centers; BM, bone marrow; CALR, calreticulin; CC, community centers; ET, essential thrombocythemia; JAK2, Janus kinase 2; MF, myelofibrosis; MPL, myeloproliferative leukemia.

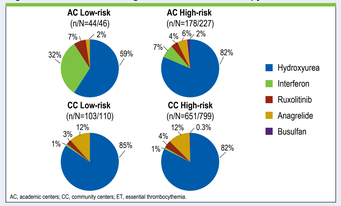
### *Current ET-Directed Therapy at Enrollment*

- For AC versus CC:
  - Percentage of high-risk patients receiving monotherapy (**Figure 3**) was similar
  - Percentage of high-risk patients receiving combination therapy was similar (AC, 3%; CC, 4%)
  - Percentages of low-risk and high-risk patients receiving anagrelide were lower
  - Percentages of low-risk patients receiving hydroxyurea was lower
  - Percentages of low-risk and high-risk patients receiving interferon were higher

### *Physician-Reported ET Signs and Symptoms at Enrollment*

- Percentage of patients with ≥1 physician-reported ET symptom was significantly higher for AC versus CC (**Table 5, Figure 4**)

**Figure 3. Patients Receiving ET-Directed Monotherapy**



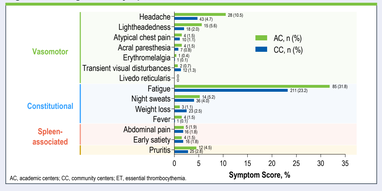

AC, academic centers; CC, community centers; ET, essential thrombocythemia.

**Table 5. Physician-Reported ET Signs and Symptoms**

| | AC (N=273) | CC (N=909) |
|---|---|---|
| Patients with available symptom data, n (%) | 267 (97.8) | 908 (99.9) |
| Patients with ≥1 symptom, n (%) | **112 (41.9)*** | **264 (29.1)** |
| ≥1 vasomotor symptom, n (%) | 38 (14.2) | 71 (7.8) |
| ≥1 constitutional symptom, n (%) | 91 (34.1) | 229 (25.2) |
| ≥1 spleen-associated symptom, n (%) | 8 (3.0) | 27 (3.0) |

Significant differences between groups are shown in bold: *P<0.0001. *P* values calculated using Fisher exact test.
AC, academic centers; CC, community centers; ET, essential thrombocythemia.

**Figure 4. ET Signs and Symptoms at Enrollment**




AC, academic centers; CC, community centers; ET, essential thrombocythemia.

### *ET MPN-SAF at Enrollment*

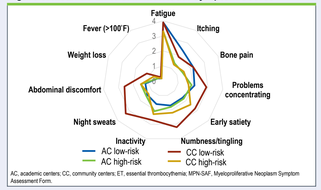
- For AC versus CC:
  - Differences in mean total symptom score were not significantly different in low-risk or high-risk patients (**Table 6**)
  - Mean individual symptom scores were generally lower in patients with low-risk ET (**Figure 5**)
  - Abdominal discomfort (*P*=0.0319), numbness and tingling (*P*=0.0413), and night sweats (*P*=0.0098) scores were significantly lower in patients with low-risk ET
  - Early satiety (*P*=0.0102) score was significantly lower in patients with high-risk ET

**Table 6. ET MPN-SAF TSS**

| | AC (N=273) | | CC (N=909) | |
|---|---|---|---|---|
| MPN-SAF | Low-risk (n=46) | High-risk (n=227) | Low-risk (n=110) | High-risk (n=799) |
| n | 27 | 133 | 78 | 534 |
| TSS, mean (SD) | 17.0 (15.1) | 15.7 (16.3) | 23.9 (19.3) | 16.4 (14.6) |
| TSS ≥20, n (%) | 8 (29.6) | 36 (27.1) | 38 (48.7) | 173 (32.4) |

AC, academic centers; CC, community centers; ET, essential thrombocythemia; MPN-SAF, Myeloproliferative Neoplasm Symptom Assessment Form; SD, standard deviation; TSS, total symptom score.

**Figure 5. Mean ET MPN-SAF Individual Symptom Scores**




AC, academic centers; CC, community centers; ET, essential thrombocythemia; MPN-SAF, Myeloproliferative Neoplasm Symptom Assessment Form.

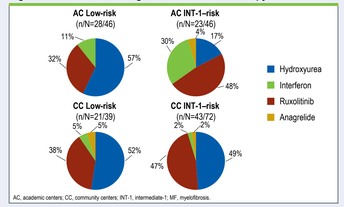
## Conclusions

- **These real-world data from MOST demonstrate that care was generally similar between AC and CC**
  - **The majority of patients were receiving active treatment for their ET or MF**
- **There were key statistically significant differences in demographics (eg, age, ethnicity, and education level), disease features, and treatment history; however, the clinical significance of these differences is unclear**
- **Compared with AC, patients in CC were significantly more likely to be older, of Hispanic/Latino ethnicity (both ET and MF cohorts), and White (ET cohort)**
- **Some of these differences (eg, demographics) could be attributed to the geographic location of the enrollee**
- **The older population and significant minority populations observed in CC versus AC may represent key populations for clinical trial recruitment that are currently under-represented**
- **Further investigation into these differences will help assess and enhance ET and MF disease management across both AC and CC**

### *Current MF-Directed Therapy at Enrollment*

- For AC versus CC:
  - Percentage of low-risk patients receiving monotherapy was higher (**Figure 6**)
  - Percentage of intermediate-1 (INT-1)–risk patients receiving monotherapy was lower
  - Percentage of INT-1–risk patients receiving combination therapy was higher (AC, 4%; CC, 1%)
  - Percentage of low-risk patients receiving anagrelide and ruxolitinib was lower
  - Percentage of INT-1–risk patients receiving hydroxyurea was lower
  - Percentages of low-risk and INT-1–risk patients receiving interferon were higher

**Figure 6. Patients Receiving MF-Directed Monotherapy**




AC, academic centers; CC, community centers; INT-1, intermediate-1; MF, myelofibrosis.

### *Physician-Reported MF Signs and Symptoms at Enrollment*

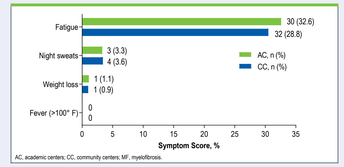
- Differences in the percentage of patients with ≥1 physician-reported MF sign or symptom were not significant for AC versus CC (**Table 7, Figure 7**)

**Table 7. Patients With Available Symptom Data**

| | AC (N=92) | CC (N=111) |
|---|---|---|
| Patients with ≥1 sign or symptom, n (%) | 59 (64.1) | 65 (58.6) |
| Anemia* | 0 | 5 (4.5) |
| Leukocytosis† | 14 (15.2) | 16 (14.4) |
| LDH above ULN | 33 (35.9) | 19 (17.1) |
| Leukoerythroblastosis‡ | 1 (1.1) | 1 (0.9) |
| Palpable spleen | 20 (21.7) | 18 (16.2) |
| Increasing splenomegaly | 3 (3.3) | 3 (2.7) |
| Constitutional symptoms§ | 31 (33.7) | 32 (28.8) |
| Other | 2 (2.2) | 5 (4.5) |

*Hemoglobin <10 g/dL. †White blood cell count ≥11×10⁹/L. ‡Shown on peripheral blood smear. §Including fatigue, night sweats, weight loss, and fever.
AC, academic centers; CC, community centers; LDH, lactate dehydrogenase; ULN, upper limit of normal.

**Figure 7. Constitutional Symptoms Reported in Patients With MF at Enrollment**




AC, academic centers; CC, community centers; MF, myelofibrosis.

### *MF MPN-SAF at Enrollment*

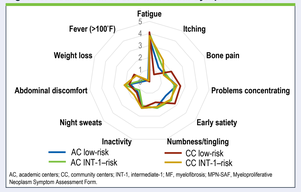
- For AC versus CC:
  - No significant differences were observed in mean total symptom score in low-risk or INT-1–risk patients (**Table 8**)
  - Individual symptom scores were generally similar in both low-risk and INT-1–risk patients (**Figure 8**), except for unintentional weight loss score, which was significantly lower in low-risk patients (*P*=0.0463)

**Table 8. MF MPN-SAF TSS**

| | AC (N=273) | | CC (N=909) | |
|---|---|---|---|---|
| MPN-SAF | Low-risk (n=46) | INT-1–risk (n=46) | Low-risk (n=39) | INT-1–risk (n=72) |
| n | 26 | 24 | 26 | 46 |
| TSS, mean (SD) | 16.3 (13.3) | 17.2 (13.8) | 19.0 (14.1) | 19.2 (17.5) |
| TSS ≥20, n (%) | 7 (26.9) | 10 (41.7) | 9 (34.6) | 18 (39.1) |

AC, academic centers; CC, community centers; INT-1, intermediate-1; MF, myelofibrosis; MPN-SAF, Myeloproliferative Neoplasm Symptom Assessment Form; SD, standard deviation; TSS, total symptom score.

**Figure 8. Mean MF MPN-SAF Individual Symptom Scores**




AC, academic centers; CC, community centers; INT-1, intermediate-1; MF, myelofibrosis; MPN-SAF, Myeloproliferative Neoplasm Symptom Assessment Form; SD, standard deviation.


### Disclosures

**Lyons:** Leadership – *McKesson*; Stock and other ownership interests – *Texas Oncology*; Consulting or advisory role – *Amgen*. **Lessen:** Research funding – *Astellas Pharma, Exact Sciences, Incyte Corporation, Pfizer*; Speakers' bureau – *Amgen, Bayer, Incyte Corporation*; Consulting/honoraria – *Bayer, Incyte Corporation*. **Fazal:** Honoraria, Speakers' bureau, Consulting or advisory role – *Agios, Amgen, Bristol Myers Squibb, Gilead Sciences, GlaxoSmithKline, Incyte Corporation, Janssen Oncology, Jazz Pharmaceuticals, Karyopharm Pharmaceuticals, Novartis, Sanofi Genzyme, Stemline Therapeutics, Taiho Pharmaceuticals, Takeda*. **Scherber, Kalafut, Ren:** Employment and stock ownership – *Incyte Corporation*. **Ritchie:** Consultancy – *AbbVie, Astellas, Bristol Myers Squibb, Jazz Pharmaceuticals, Protagonist, Takeda*; Honoraria – *AbbVie, Novartis, Protagonist, Takeda*; Research funding – *Jazz Pharmaceuticals*; Speakers' bureau – *AbbVie*.

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