Poster #157

Symptomatology and Diagnostic Journey of Patients Diagnosed with Systemic Mastocytosis in The US Oncology Network

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Background/Objectives

- Systemic mastocytosis (SM) is a rare disease characterized by severe and unpredictable symptoms across multiple systems.[1,2]
- Given the heterogeneity and fluctuations in severity of symptoms, patients with SM may interact with many different clinical specialists over several years before being diagnosed with the disease. [3]
- SM is broadly categorized into non-advanced SM and advanced SM. Most patients (90% to 95%) are considered to have non-advanced SM, which primarily includes indolent systemic mastocytosis (ISM) (90% of patients with SM) and a small subset of patients who have smoldering SM (SSM).[4,5,6]
- **The objective of this study was to describe the presenting signs, symptoms, and clinical characteristics of patients receiving a diagnosis from a community oncologist within the US Oncology network.**

Methods

Study Design

- This was a retrospective observational cohort study of patients with SM treated in The US Oncology Network.
- Patients with a diagnosis of SM between January, 2010 – May, 2021 were eligible for inclusion in the study.
  - Patients with SSM or ISM were categorized as non-advanced SM. Remaining patients were included in the advanced SM cohort.
- Eligible patients were followed longitudinally after their SM diagnosis until August 31, 2021, last patient record, or date of death, whichever occurs first.

Data Source

- Data were abstracted through chart review from iKnowMed, the electronic health records (EHR) database of The US Oncology Network.
- The Limited Access Death Master File of the Social Security Administration was an additional source of vital status (death).

Inclusion/Exclusion Criteria

- Diagnosis of SM
  - Qualifying patients categorized by SM subtype (i.e., advanced, non-advanced)
- Age ≥ 18 years at SM diagnosis
- Had ≥ 2 visits within The US Oncology Network
- Not enrolled in clinical trials during the study observation period
- Not receiving treatment for other documented, SM-unrelated primary cancer diagnoses during the study observation period

Statistical Analysis

- Descriptive statistics were utilized to summarize patient characteristics.

Results

- 33 eligible SM patients, 17 with non-advanced SM and 16 with advanced SM, were identified and included in the analysis (Figure 1).
- Demographics and clinical characteristics for advanced and non-advanced SM patient cohorts are provided in Table 1.
- The median observation period following SM diagnosis was 12.9 months for the advanced SM cohort and 20.4 months for non-advanced SM cohort.
- At the end of the study period, 1 non-advanced patient (5.9%) and 3 advanced patients (18.8%) were deceased.

Results

Figure 1. Patient Identification

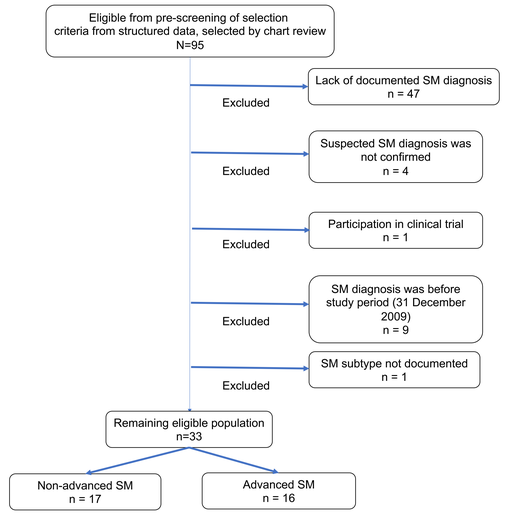

Table 1. Patient Demographics and Clinical Characteristics

Characteristic	Non-advanced SM (N=17)	Advanced SM (N=16)
Age at SM diagnosis (years) Median (IQR)	61 (55,70)	70 (66,77)
Age group at SM diagnosis, n (%) <65 years 65+ years	11 (64.7) 6 (35.3)	3 (18.8) 13 (81.3)
Male sex, n (%)	3 (17.6)	9 (56.3)
Practice region, n (%) West South Midwest Northeast	6 (35.3) 4 (23.5) 4 (23.5) 3 (17.6)	8 (50.0) 4 (25.0) 3 (18.8) 1 (6.3)
BMI (kg/m²) Median (IQR)	28.8 (27.1,32.2)	24.8 (23.0,28.9)
Follow-up time (months) Median (IQR)	20.4 (7.8,24.8)	12.9 (8.2,19.8)

- Advanced SM patients were referred to the US Oncology network by primary care physicians (50%) and the majority were diagnosed by a hematologist (81.3%). (Figures 2 and 3).
- Non-advanced SM patients were referred and diagnosed by multiple specialist types.

- For advanced patients, symptoms and low blood count were listed as a reason for referral in 43.8% of patients. 32% of patients listed elevated blood counts as a reason for referral (Figure 4).
- Among non-advanced patients, 47% of patients were referred due to symptoms and 35.3% of patients were referred due to mastocytosis.
- The symptomatology profile differed between the SM cohorts but across both cohorts, reported symptoms were widespread and diverse.
  - Among patients with advanced SM, 68.8% reported at least one systemic symptom, 43.8% reported at least one skin symptom, and 31.3% of patients reported at least one neurological or GI symptom at initial presentation.
  - Among the non-advanced patients included in this study, 70.6% of patients report at least one neurological symptom, 64.7% at least one skin symptom, and 47.1% reported at least one GI symptom.
- Patients in the non-advanced cohort reported, an average of 3.9 symptoms as presentation, while advanced patients presented with 2.6 symptoms on average.

Figure 2. Specialty of Referring Physician to US Oncology Network for SM Patients

Figure 3. Specialty of Diagnosing Physician for SM Patients

Figure 4. Reasons for Referral to Specialist in Patients with Non-advanced and Advanced SM, % of Patients

Non-Advanced (n=17)		Advanced (n=16)	
Symptoms (47%)	Mastocytosis (35%)	Symptoms (44%)	Low Blood Counts (44%)
Other (24%)	Elevated Blood Counts (6%)	Elevated Blood Counts (31%)	Organ/System Specific Symptoms (13%)
Organ/System Specific Symptoms (6%)	Anaphylactic Shock/Anaphylactoid Event (6%)	Mastocytosis (6%)	Anaphylactic Shock/Anaphylactoid Event (6%)
Splenomegaly (6%)	Lymphadenopathy (6%)	Splenomegaly (6%)	Other (6%)

Figure 5. Prevalence of Symptoms at SM Diagnosis

Conclusions

- This analysis provides insight into the complex diagnostic journey for SM patients that involves multiple physician specialties.
- Advanced and non-advanced SM patients presented with multiple symptoms across a number of domains. Increased awareness of the multiple specialties involved and the heterogeneous nature of ISM symptoms may have improve the diagnosis journey for patients.

Acknowledgements

Poster developed by Ontada

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Disclosures

Study sponsored by Blueprint Medicines. Green, Sullivan, Daddona, and Miller are employees of Blueprint Medicines and own stock in the company.

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