Disease Progression in Patients with Systemic Mastocytosis: A US Population-Level Analysis Using Claims-Based Dataset

Sudipto Mukherjee, 1 Douglas J. Cattie, 1 Naven Penmargu, 1 Daniel Shalaby, 2 Teresa Green 3

1Department of Hematology and Medical Oncology, Cleveland Clinic Main Campus, Cleveland, OH, USA; 2Blueprint Medicines Corporation, Cambridge, MA, USA; 3Department of Advanced Analytics, The University of Texas MD Anderson Cancer Center, Houston, TX, USA

Results

- 8,713 patients with SM qualified for analysis (Figure 1), including 1,587 advanced SM, 2,705 ISM (all stages combined), and 6,643 SM-lower (Table 1).
- Mean age at diagnosis was 49 years.
- Racial breakdown strongly biased towards whites (85.7% of all SM) but may reflect inequities regarding access to healthcare rather than true underlying epidemiological differences.
- Female predominance (68.8% of all SM) aligns with findings from other claims-based studies.
- A majority of patients were enrolled in commercial insurance plans (67.2% of all SM).

Analysis Design

- This analysis utilized a large nationally representative United States (US) claims database including patients with commercial, Managed Medical, and Medicare Advantage insurance coverage, 2015-2021. Patients were included if they had claims in each year 2010-2021, pragmatically approximating continuous enrollment.
- A small claims-based algorithm based on WHO diagnostic criteria (2018) was developed.
- Patients were selected if they fulfilled ≥1 of the following criteria (Table 1):
  - ≥2 diagnoses with an SM disorder code (D47.0, C96.21, C94.3X) ≥30 days apart in any setting of care.
  - Bone marrow biopsy followed by an SM diagnosis code in any setting of care.
  - 1 SM diagnosis code in any setting of care and ≥1 prescription claim for an SM-specific treatment (cromolyn, ephedrine, histamines, interferons, hydroxyurea, imatinib, interferon, letrozole, midostaurin, or corticosteroids).
- Qualifying patients stratified by SM subtype: advanced SM, higher symptom burden SM, or lower symptom burden SM (Figure 2).

Conclusions

- This analysis utilized a large US claims dataset and a novel algorithm to identify patients with SM and describe patterns of disease progression and worsening over a 24-month period and 12-month resource utilization.
- The analysis reflects the accumulation of symptoms over time, by a meaningful subset of patients.
- There is wide heterogeneity in clinical phenotypes among patients with SM, leading to treatment differences.
- 30% of patients with ISM were categorized as higher symptom burden ISM, requiring greater use of symptom-directed and disease-specific therapies.
- Finally, this proportion of advanced SM patients on FDA-approved SM-directed TKI therapies remains low, highlighting large unmet clinical treatment needs in this rare disease field.

Limitations

- Approximately 25% of qualifying patients did not have a SM diagnosis 2 years prior, possibly contributing to the time from enrollment to the SM diagnosis.
- There is wide heterogeneity in clinical phenotypes among patients with SM, leading to treatment differences.
- 30% of patients with ISM were categorized as higher symptom burden ISM, requiring greater use of symptom-directed and disease-specific therapies.
- Finally, this proportion of advanced SM patients on FDA-approved SM-directed TKI therapies remains low, highlighting large unmet clinical treatment needs in this rare disease field.

References