# Healthcare Resource Utilization and Costs of Advanced Systemic Mastocytosis Among Medicare Fee for Service Beneficiaries

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# Disclosures

- ES, JC, CN, UN, and CS are employees of Blueprint Medicines, which sponsored this study

- AP, ARS, AM, and JS are employees of Avalere Health, which received funding from Blueprint Medicines to complete the study

# Background and Objectives

## Background /

- Advanced systemic mastocytosis (AdvSM) is a rare mast cell neoplasm driven by the KIT D816V mutation and includes ASM, SM-AHN, and MCL.[1]
- Diagnosis of AdvSM is challenging and patients experience a range of severe symptoms including organ damage and shortened survival,[2] as well as a negative impact on mental health and ability to perform usual activities.[3]
- Typical age of onset is age 60 and older.[1]
- There is limited research quantifying the incremental economic burden of AdvSM, particularly within the Medicare population.

## Study Objectives /

- Estimate and compare direct HCRU and costs in Medicare FFS beneficiaries with AdvSM to a matched cohort of Medicare beneficiaries without SM.

AdvSM: advanced Systemic Mastocytosis; SM: Systemic Mastocytosis; ASM: aggressive SM; SM-AHN: SM with associated hematological neoplasm; MCL: mast cell leukemia; HCRU: healthcare resource utilization; FFS: Fee for Service


1. Pardanani, A. (2019). Systemic mastocytosis in adults: 2019 update on diagnosis, risk stratification and management. *American journal of hematology*, *94*(3), 363-377.
2. Valent, P., Akin, C., Gleixner, K. V., Sperr, W. R., Reiter, A., Arock, M., & Triggiani, M. (2019). Multidisciplinary challenges in mastocytosis and how to address with personalized medicine approaches. *International journal of molecular sciences*, *20*(12), 2976.
3. Mesa, R. A., Sullivan, E. M., Dubinsky, D., Carroll, B., Slee, V. M., Jennings, S., ... & Castells, M. (2020). Patient reported outcomes among systemic mastocytosis (SM) patients in routine clinical practice: Results from the TouchStone survey. *Blood*, *136*, 37.

# Study Design

## Cohort Identification /

- Patients with newly diagnosed AdvSM were identified using a claims-based algorithm applied to CMS-sourced 100% Medicare FFS claims (Parts A/B/D).
  - Required ≥1 medical claim with diagnosis of SM and ≥1 medical claim with a diagnosis of ASM, SM-AHN, or MCL between 01/01/2017 and 12/31/2018. The index date was the date of first observed SM diagnosis code.
  - Continuously enrolled in Medicare with medical and pharmacy coverage for 12 months pre- and post-index and no diagnosis of SM in the 12 months prior to index.
- Patients with AdvSM were direct matched (1:1) to a non-SM control cohort on age, sex, race, index year, Medicare-Medicaid dual eligibility, and CCI score.

## Outcomes /

- Total all-cause HCRU and costs (based on Medicare payments [2021 USD]) were assessed pre- and post-index. Chi-square/t-tests evaluated differences between AdvSM and non-SM patients.

AdvSM: advanced Systemic Mastocytosis; SM: Systemic Mastocytosis; CMS: Centers for Medicare and Medicaid Services; ASM: aggressive SM; SM-AHN: SM with associated hematological neoplasm; MCL: mast cell leukemia; CCI: Charlson Comorbidity Index; HCRU: healthcare resource utilization; USD: US dollar. ICD-10-CM diagnosis codes: SM : D47.02; ASM: C96.21, SM-AHN: C92.X, C93.X, D45, D46.X, D47.1, D47.3, D47.4, D47.5, D75.81, D94.6; MCL: C94.30, C94.31, C94.32

# Key Findings

**Table 1: Characteristics of Matched Cohorts of AdvSM and Non-SM Medicare Beneficiaries**

| Baseline Characteristics | AdvSM Cohort (N=339) % | Non-SM Cohort (N = 339) % | P-Value |
|---|---|---|---|
| Mean (SD) age at index* | 68.2 (13.2) | 68.5 (13.9) | 0.71 |
| Female* | 59.0% | 59.0% | 1.00 |
| White* | 90.9% | 90.9% | 1.00 |
| Dual eligible for Medicare and Medicaid* | 27.7% | 27.7% | 1.00 |
| Eligible for Part D Low Income Subsidy | 30.7% | 33.9% | 0.23 |
| CCI Score, Mean (SD)* | 4.32 (3.09) | 4.32 (3.09) | 1.00 |

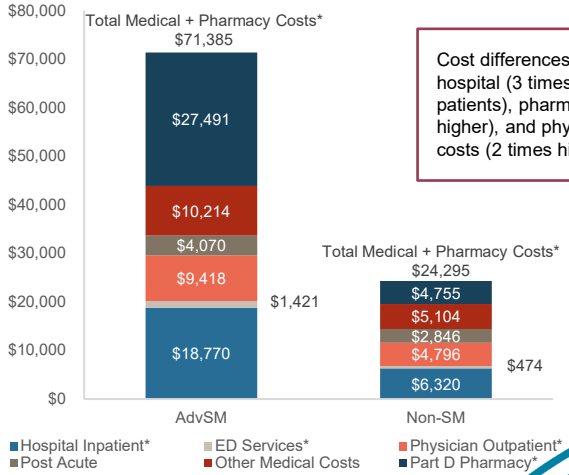
**Table 2: Healthcare Resource Utilization During The 12-Month Post-Index Period Among AdvSM and Non-SM Patients**

| | AdvSM Cohort (N=339) | Non-SM Cohort (N = 339) | P-Value |
|---|---|---|---|
| **All Cause Utilization Measure Over 12 Months Post-Index** | | | |
| *Medical Services (Mean (SD) Number of Encounters Per Patient, Total Cohort)* | | | |
| Hospitalizations | 1.3 (2.4) | 0.5 (1.0) | <0.01 |
| Hospitalizations (among patients with ≥1) | 2.9 (2.9) | 1.8 (1.3) | <0.01 |
| Length of stay (days) | 17.2 (23.0) | 10.3 (13.6) | <0.01 |
| ED Visits | 2.0 (6.4) | 0.8 (1.6) | <0.01 |
| ED Visits (among patients with ≥1) | 4.1 (8.7) | 2.3 (2.1) | 0.01 |
| Primary Care Office Visits | 9.8 (12.9) | 6.7 (7.3) | <0.01 |
| Oncologist/Hematologist Visits | 6.8 (14.7) | 1.0 (4.6) | <0.01 |
| Allergist/Immunologist Visits | 1.5 (5.6) | 0.1 (0.5) | <0.01 |
| Other Provider Visits | 55.5 (46.6) | 29.3 (28.3) | <0.01 |
| *Pharmacy Services* | | | |
| Proportion of Patients with ≥1 Prescription | | | |
| Oral corticosteroids | 42.7% | 27.1% | <0.01 |
| Systemic corticosteroids | 39.2% | 28.6% | <0.01 |
| Epinephrine | 22.7% | <3.0% | <0.01 |
| Chemotherapy | 9.1% | 5.0% | 0.04 |
| Omalizumab | 4.1% | 0.0% | <0.01 |

AdvSM: advanced systemic mastocytosis; SM: systemic mastocytosis; SD: standard deviation; CCI: Charlson Comorbidity Index; ED: emergency department
For SM patients, outcomes were measured during the 12 months following diagnosis date of ISM. Index dates were randomly assigned to patients in the non-SM cohorts. A randomly selected pharmacy or medical claim during the selection window that matches with the index date from the SM cohorts served as the "index date" for these patients.

- 339 AdvSM and 339 non-SM patients were included for analysis
- Over 25% of patients were <65 years of age at index and originally qualified for Medicare with a disability
- During the 12-month pre-index period, AdvSM patients had higher prevalence of asthma (26% vs. 16%, p=0.001) and malignancy (60% vs. 23%, p<0.0001), and more specialist and ED visits per patient (mean [SD] 17.4 [17.4] vs. 11.9 [12.0], p<0.0001; 3.8 [11.0] vs. 1.4 [2.8], p=0.010) compared with controls

**Figure 1: Direct Medical and Pharmacy Costs During 12 Months Post-Index Among Matched Cohorts of AdvSM and Non-SM Patients**




Cost differences were driven by hospital (3 times higher for AdvSM patients), pharmacy (almost 7 times higher), and physician outpatient costs (2 times higher).

*Differences between AdvSM and Non-SM cohort were statistically significant at p=0.05
Other Medical Costs include: outpatient visits other than ED, laboratory tests, radiology encounters, and other physician services

# Limitations

- Medicare beneficiaries with AdvSM were identified beginning January 1, 2017, but the ICD-10 diagnosis code for SM (D47.02) did not go into effect until October 1, 2017. Misidentification of patients may contribute to an underestimation of SM patients in the study sample.
- SM patients tend to experience a long diagnosis journey. AdvSM patients may have been initially diagnosed with SM prior to the 12-month baseline period.
- SM-AHN was identified based on treatment utilization and may underrepresent the true number of SM-AHN patients due to challenges associated with diagnosis and reporting of both the SM and AHN components in medical claims.
- Use of age and comorbidity score in the cohort matching process resulted in a larger proportion of AdvSM and non-SM patients who qualified for Medicare due to pre-existing disability rather than age (as compared to the full Medicare population). The matching process did not fully account for the reason for disability and other comorbid factors which may undercount the true difference in HCRU and costs between AdvSM and non-SM populations.
- Limited generalizability to non-Medicare populations.
- Temporality cannot be established using claims data.

AdvSM: advanced Systemic Mastocytosis; ICD-10: International Classification of Diseases, 10th Revision; SM: Systemic Mastocytosis; SM-AHN: SM with associated hematological neoplasm; MCL: HCRU: healthcare resource utilization

# Conclusions

- In this retrospective analysis, Medicare FFS beneficiaries with AdvSM were more resource intensive and nearly 3 times more costly in the 12 months following SM diagnosis compared to a matched cohort of non-SM patients.
- Costs were driven by significantly higher rates of inpatient stays, ED visits, physician outpatient visits, and higher utilization of prescription medications.
- Notably, AdvSM patients in this analysis included a larger proportion of patients <65 years old with pre-existing disability compared to the broader Medicare population (26% vs. 14%), suggesting that AdvSM patients may be more likely to qualify for Medicare due to disability rather than age compared to the overall Medicare population. Further research in this area is warranted.

FFS: Fee for Service; SM: systemic mastocytosis; AdvSM: advanced systemic mastocytosis; ED: emergency department