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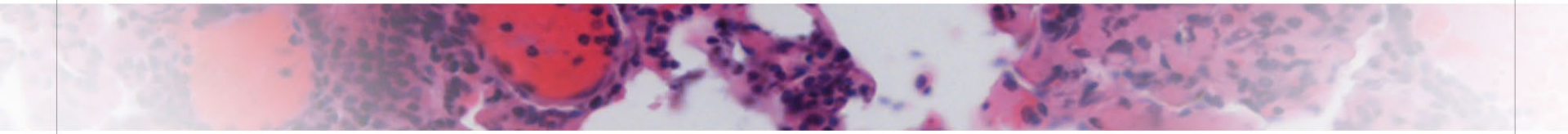

Patient Reported Outcomes among systemic mastocytosis (SM) patients in routine clinical practice: results from the TouchStone Survey

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Background

- SM is a rare, clonal MC neoplasm driven by the *KIT* D816V mutation characterized by unpredictable, severe, and debilitating skin, gastrointestinal, and systemic symptoms
- SM symptoms are caused by MC hyperactivation and uncontrolled proliferation, degranulation, and mediator release[1,2]
- As many as 50% of patients with SM, the majority of whom have the ISM subtype, report experiencing life-threatening anaphylaxis[3,4]
- Patients with SSM and AdvSM subtypes have increased risk of progression and lower OS compared with ISM patients[5]
- Patients with SM are often misdiagnosed or have delayed diagnosis[6]
- The objective of this study was to assess the impact of SM on patients' daily functioning, work status, use of healthcare services, and medication use in a real-world setting in the US

AdvSM, advanced systemic mastocytosis; MC, mast cells; ISM, indolent systemic mastocytosis; OS, overall survival; SM, systemic mastocytosis; SSM, smoldering SM.

1. Gulen T et al. *J Intern Med*. 2016;279:211–228; 2. Pardanani A. *Am J Hematol*. 2016;91:1146–1159; 3. Valent P et al. *Clin Exp Allergy*. 2014;44:914–920; 4. Hartmann K et al. *J Allergy Clin Immunol*. 2016.137:35–34; 5. Shomali W and Gotlib J. *Hematology Am Soc Hematol Educ Program*. 2018;1:127–136; 6. Jennings SV et al. *Immunol Allergy Clin North Am*. 2018;38:505–525.

TouchStone patient survey: methods

- Patients ≥18 years residing in the United States with self-reported diagnosis of SM who provided informed consent were recruited to participate in this survey through the Mast Cell Connect patient registry[1]
- Patients completed a 100-item online survey that included the **ISM-SAF** (symptom assessment), **SF-12** (global health assessment) and **WPAI** (work/activity impairment measure) questionnaires
- The online survey also included questions related to the following[a]:
  - SM diagnosis, symptoms, and impact on daily functioning, ability to work, and quality of life
  - Use of OTC and prescription medications for SM, use of epinephrine for anaphylaxis, and frequency of physician and emergency department (ED) visits during 2019 (one-year prior to COVID-19 pandemic)
- Descriptive statistics on survey answers

ISM-SAF[2]

Symptoms	Description
GI (0–30): Abdominal pain, diarrhea, nausea	• Each symptom scored 0–10 • 0 is no symptoms, 10 is the worst imaginable • 24-hour recall period
Skin (0–30): Spots, itching, flushing	
Neurocognitive (0–30): Brain fog, headache, dizziness	
Bone pain	
Fatigue	

SF-12

Assessment	Description
Physical functioning	• 5-point Likert scale (responses range from 'Not at all' to 'Extremely') • 3-point verbal rating scales • Physical and mental component scores range from 0 to 100 (lowest and highest level of health, respectively) • 4-week recall period
Role-physical	
Bodily pain	
General health	
Vitality	
Social functioning	
Role-emotional	
Mental health	

[a]Established PRO measures were scored using established scoring algorithms; GI, gastrointestinal; ISM-SAF, Indolent Systemic Mastocytosis Symptom Assessment Form; OTC, over-the-counter; PRO, patient-reported outcome; SF-12, 12-Item Short Form survey; WPAI, Work Productivity and Activity Impairment. 1. . Mast Cell Connect is an electronic online patient registry owned and managed by Blueprint Medicines: https://www.mastcellconnect.org; 2. Shields A et al. ISPOR Europe Congress 2019, November 2–6, Copenhagen, Denmark. Poster #PRO142.

TouchStone patient survey participants

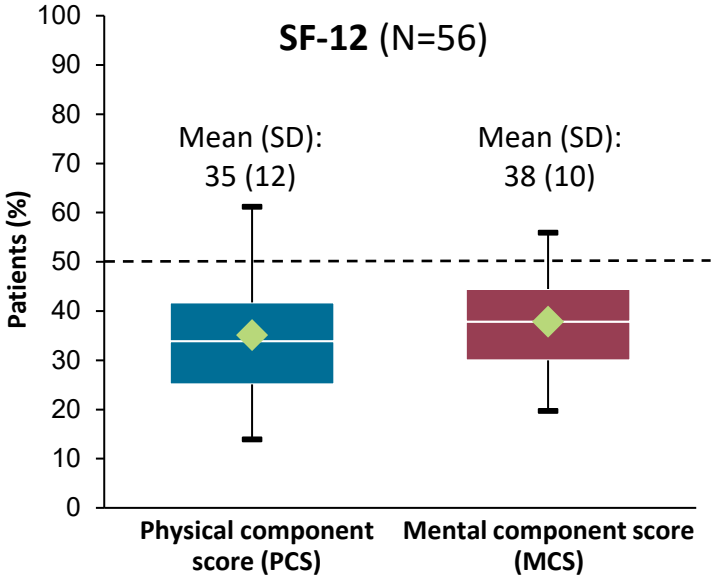
Patient characteristics	N=56
Median age (range), years	48 (20–76)
Female, n (%)	50 (89)
Mean time since receiving SM diagnosis, years (range)	7 (1–20)
SM subtype, n (%)	
ISM	37 (66)
ASM	5 (9)
SSM	3 (5)
SM-AHN	1 (2)
Unknown	10 (18)
Mean time from symptom onset to receiving physician diagnosis, years (range)	6 (1–10)
Type of physician who diagnosed SM, n (%)	
Allergist/Immunologist	24 (43)
Dermatologist	13 (23)
Hematologist/Oncologist	12 (21)
Gastroenterologist	3 (5)
Other	4 (7)

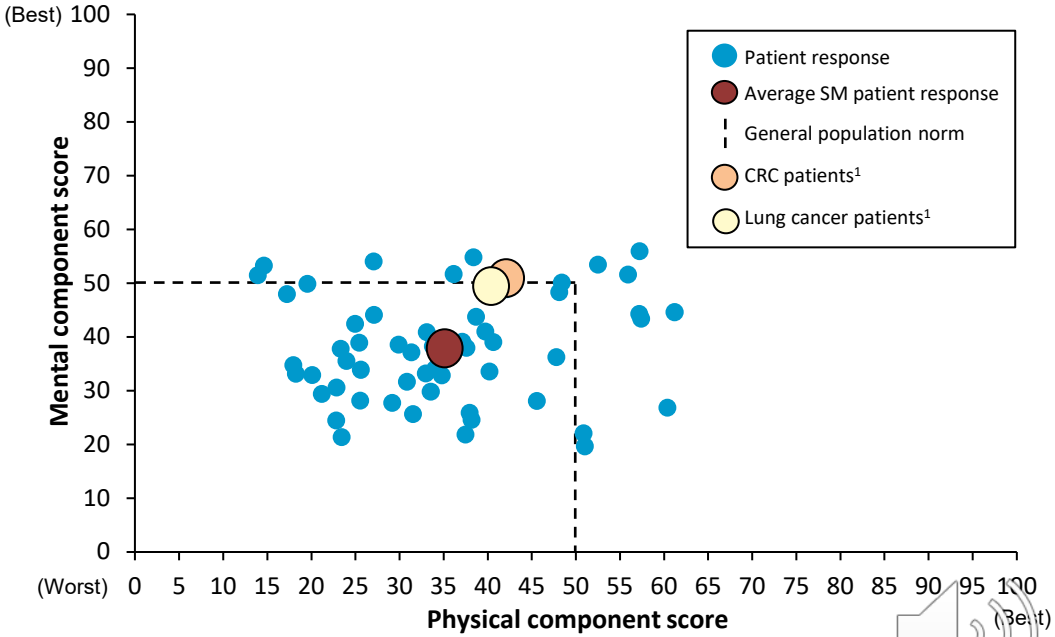
Primary physician who manages SM, n (%)	
Allergist/Immunologist	33 (59)
Hematologist/Oncologist	12 (21)
General practitioner/PCP	9 (16)
Other	2 (4)
Setting of care for primary SM physician, n (%)	
Academic hospital	18 (32)
Multi-specialty group/HMO	16 (29)
Single specialty group	5 (9)
Solo practice	9 (16)
Community hospital	2 (4)
Other	4 (7)
Not sure	2 (4)
Symptoms reported during the past year, n (%)	
Patients reporting ≥10 symptoms	56 (100)
Most bothersome symptom	
Anaphylactic episodes	10 (18)
Abdominal/stomach pain	9 (16)
Diarrhea	7 (13)
Fatigue	[illegible]

ASM, aggressive SM; HMO, health maintenance organizations; PCP, primary care physician; SM, systemic mastocytosis; SM-AHN, systemic mastocytosis with an associated hematologic neoplasm.; SSM, smoldering SM.

Participants reported reduced physical functioning and mental health

Compared to CRC and lung cancer patients, SM patients on average report lower (worse) PCS and MCS scores



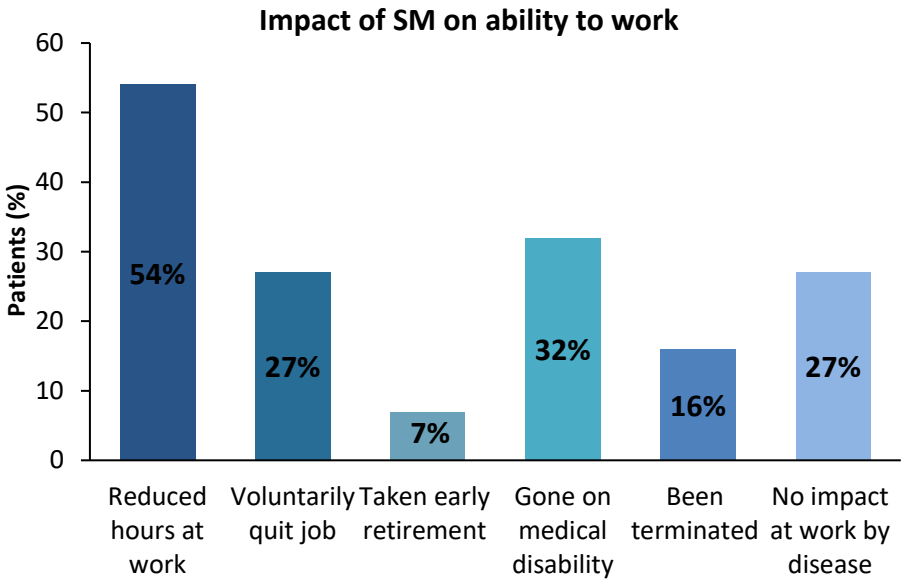


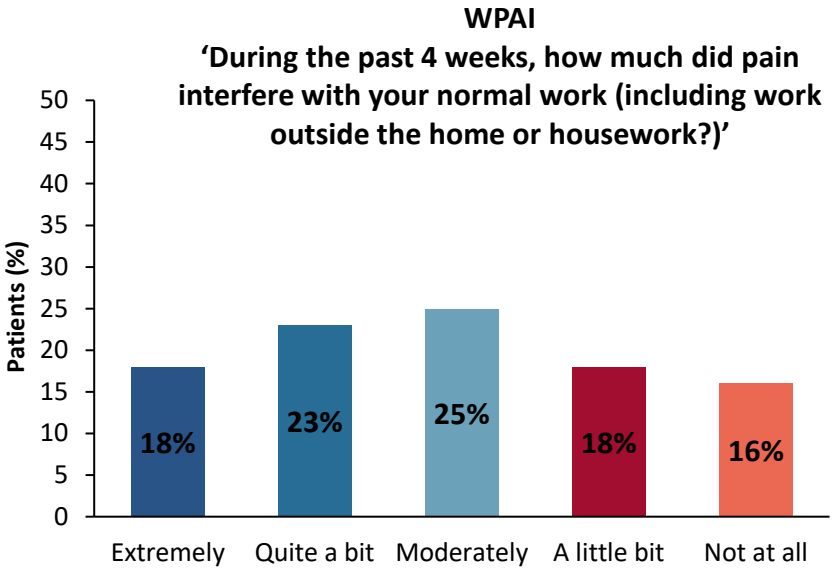
Blue and red box plots on the SF-12 figure represent the interquartile range and the median.

1. Kenzik KM et al. *Cancer.* 2015;121:2831–2839.

CRC, colorectal cancer; MCS, mental composite score; PCS, physical composite score; SD; standard deviation.

Participants reported SM symptoms have significant impact on ability to work and perform usual activities

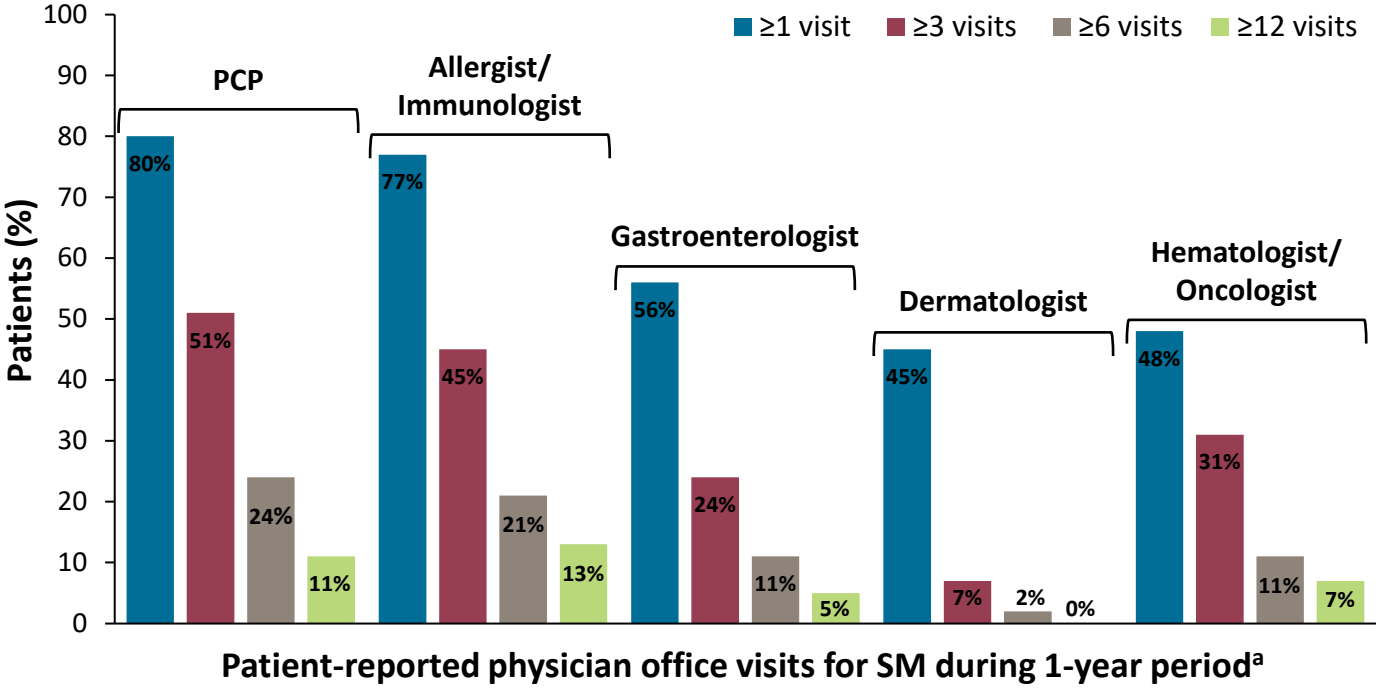




64% of respondents reported they avoid leaving their house due to SM symptoms

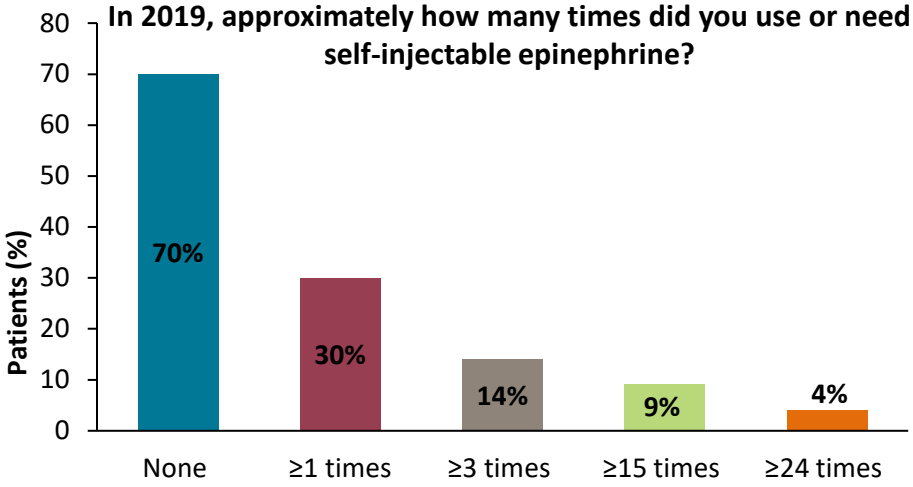
Percentages on the bar graphs have been rounded to the closest whole number and may not add up to 100.

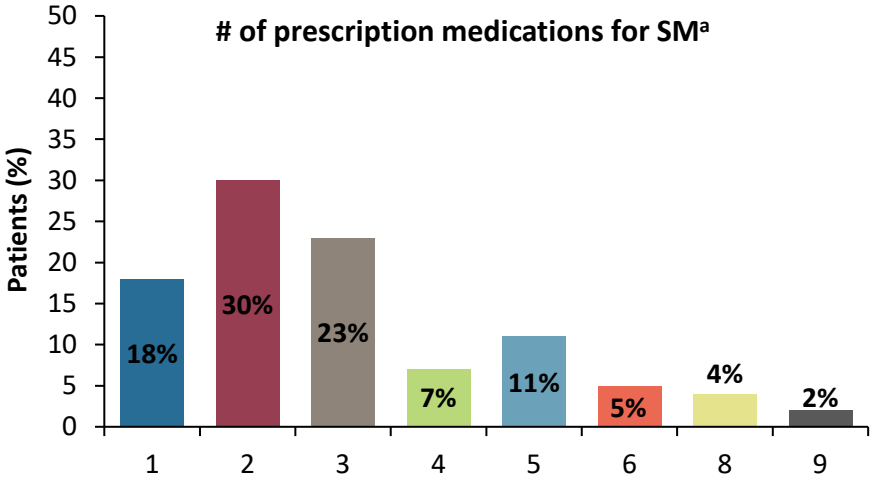
Participants reported frequent visits to multiple physician specialists for their SM symptoms



[a]Visits in 2019.
PCP, primary care physician. Percentages on the bar graphs have been rounded to the closest whole number and may not add up to 100.

Participants reported SM-related anaphylaxis, events and use of multiple OTC and prescription medications for SM





- 88% of participants reported they have epinephrine for emergency use
- 30% of participants reported going to the ED in 2019 at least one time for anaphylaxis
- 63% of participants reported having anaphylaxis but managing it at home at least one time instead of going to the ED in 2019

- 51% of participants reported taking ≥3 prescription medications to manage SM
- 61% of participants reported taking ≥3 OTC medications

[a]Over a period of one year.
Percentages on the bar graphs have been rounded to the closest whole number and may not add up to 100.

Conclusions

- These survey findings indicate that SM symptoms have a substantial negative impact on patients' ability to work and perform usual activities.
- Compared to CRC and lung cancer patients, participants in this TouchStone survey reported on average lower (worse) physical functioning and mental health (PCS and MCS SF-12 scores).
- Over a one-year period, SM patients in this study reported use of multiple OTC and prescription medications, frequent visits to physician specialists to manage their SM, and anaphylactic events.

Limitations and future research

- This study is limited by the inclusion of patients with self-reported SM. Future studies including patients with physician-verified SM should be considered.
- Additional research on the frequency and optimal management of anaphylaxis among SM patients is warranted based on these findings.

Acknowledgements

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