



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

The TouchStone SM Survey Working Group: Ruben A. Mesa¹, Erin M. Sullivan², David Dubinski²,
Brittany Carroll², Valerie M. Slee³,
Susan Jennings³, Celeste Finnerty³, Linda Bohannon⁴ Susan Mathias⁵, Mariana Castells⁶

¹Mays Cancer Center at UT Health San Antonio, San Antonio, Texas, USA; ²Blueprint Medicines Corporation, Cambridge, Massachusetts, USA; ³The Mast Cell Disease Society, Sterling, Massachusetts, USA; ⁴Cancer Support Community, Washington DC, USA; ⁵Health Outcomes Solutions, Winter Park, Florida, USA;

⁶Brigham and Women's Hospital, Boston, Massachusetts, USA

Corresponding author: MesaR@uthscsa.edu

Disclosures

Dr. Mesa has received funding from AbbVie, Celgene, CTI Biopharma, Genentech, Incyte, Promedior and Samus, and has received consultancy fees from LaJolla Pharma, Novartis and Sierra Oncology.

Study sponsored by Blueprint Medicines Corporation.



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 patients⁵
- Patients with SM are often misdiagnosed or have delayed diagnosis⁶
- 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



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 registry¹
- 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	<ul style="list-style-type: none"> • 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	<ul style="list-style-type: none"> • 5-point Likert scale (responses range from ‘Not at all’ to ‘Extremely’)
Role-physical	
Bodily pain	<ul style="list-style-type: none"> • 3-point verbal rating scales
General health	
Vitality	<ul style="list-style-type: none"> • Physical and mental component scores range from 0 to 100 (lowest and highest level of health, respectively)
Social functioning	
Role-emotional	
Mental health	<ul style="list-style-type: none"> • 4-week recall period



TouchStone patient survey participants

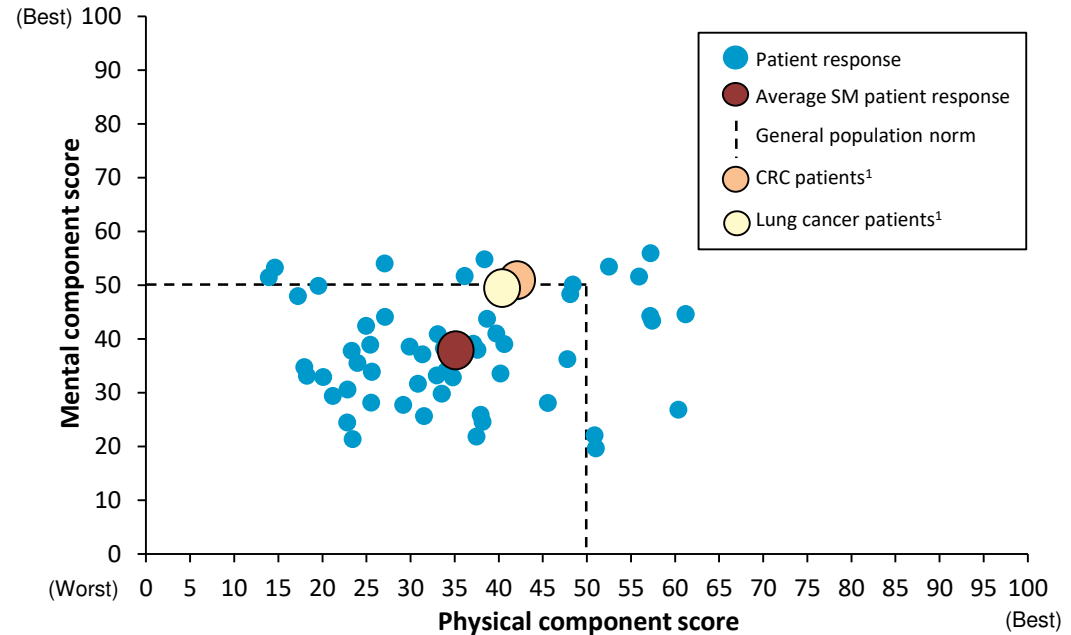
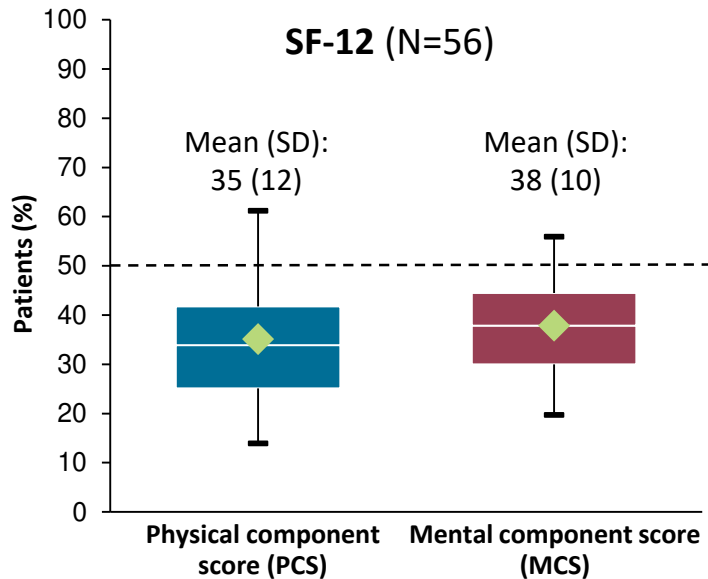
Patient characteristics	N=56
Median age (range), years	48 (20–76)
Female, n (%)	50 (89)
Mean time since receiving SM diagnosis, years (range)	7 (1–20)
SM subtype, n (%)	
ISM	37 (66)
ASM	5 (9)
SSM	3 (5)
SM-AHN	1 (2)
Unknown	10 (18)
Mean time from symptom onset to receiving physician diagnosis, years (range)	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	6 (11)

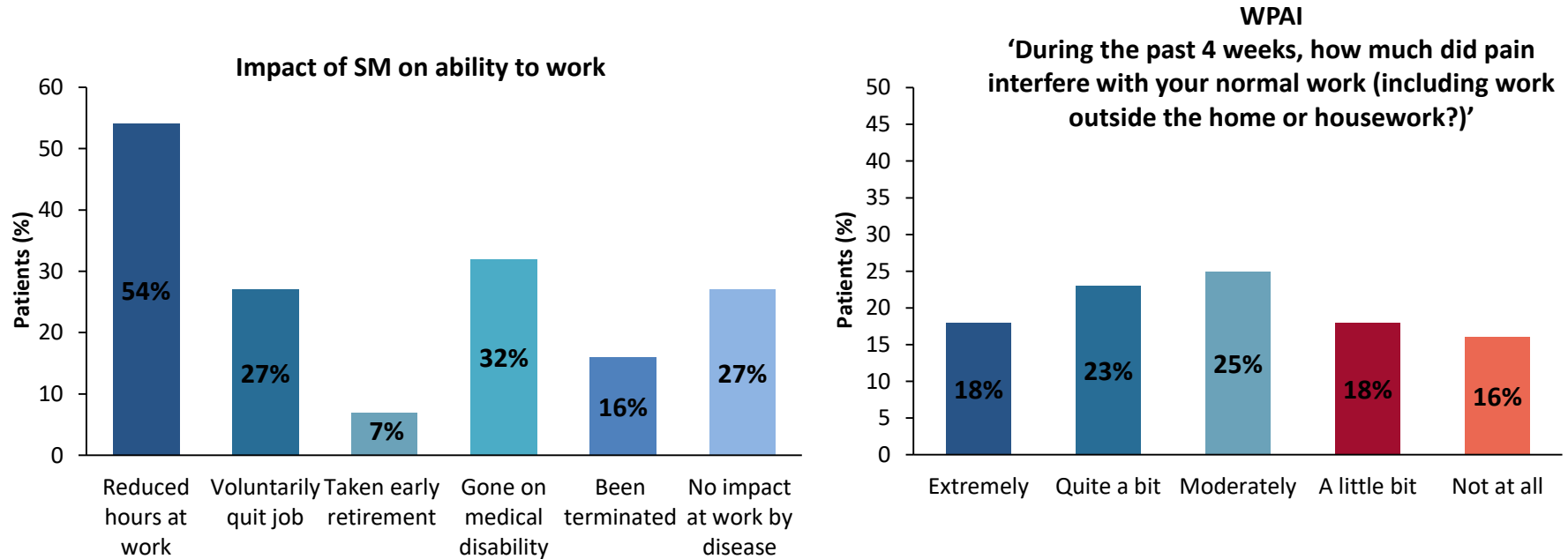


Participants reported reduced physical functioning and mental health

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



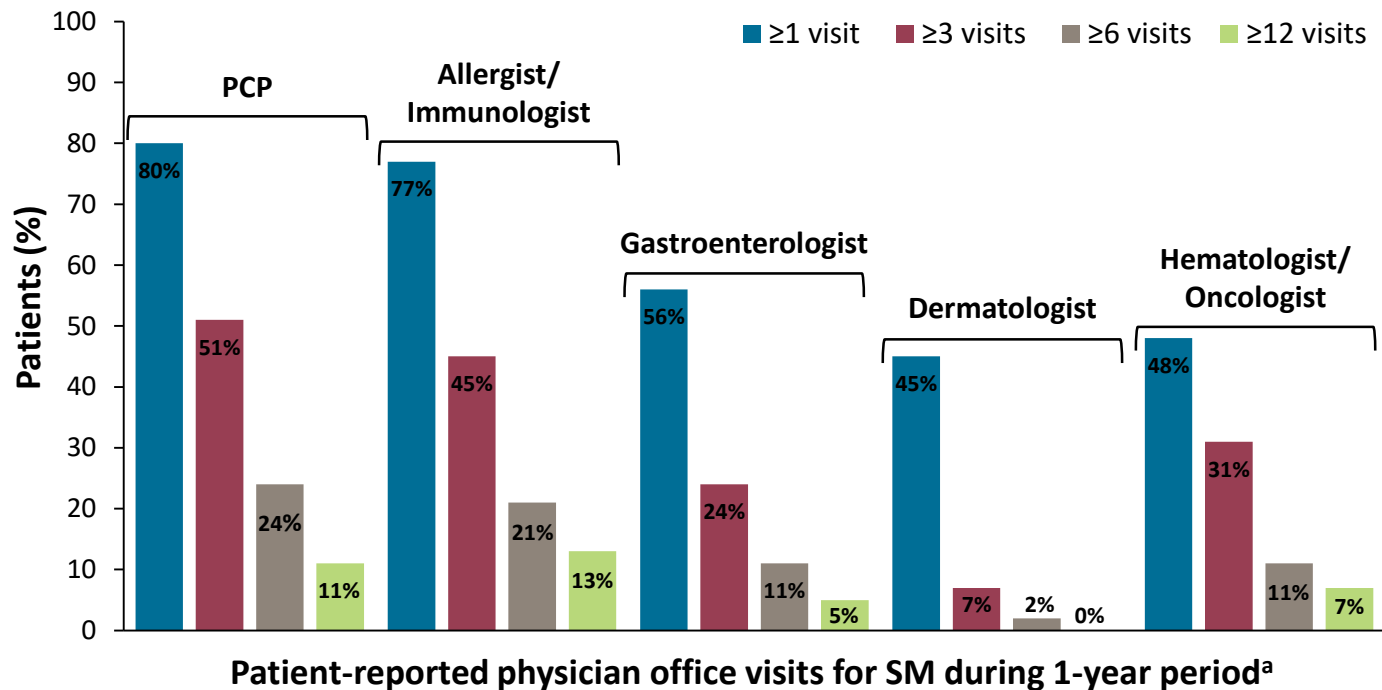
Participants reported SM symptoms have significant impact on ability to work and perform usual activities



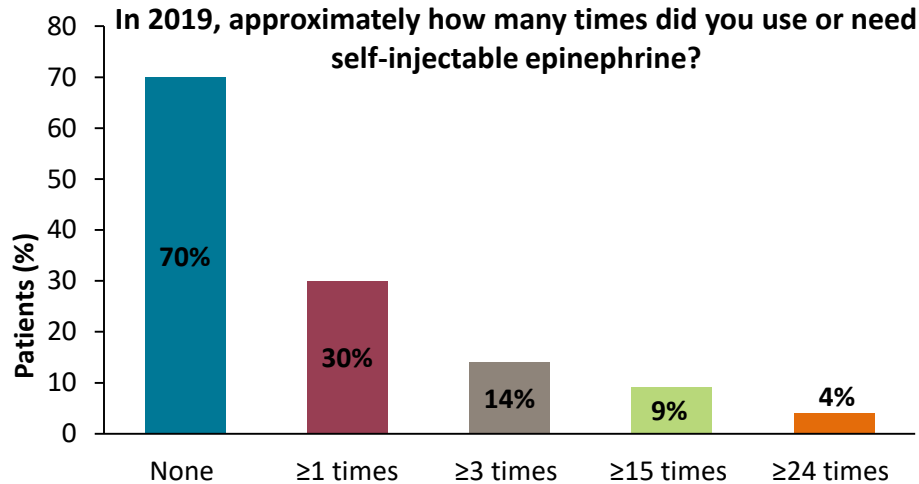
64% of respondents reported they avoid leaving their house due to SM symptoms



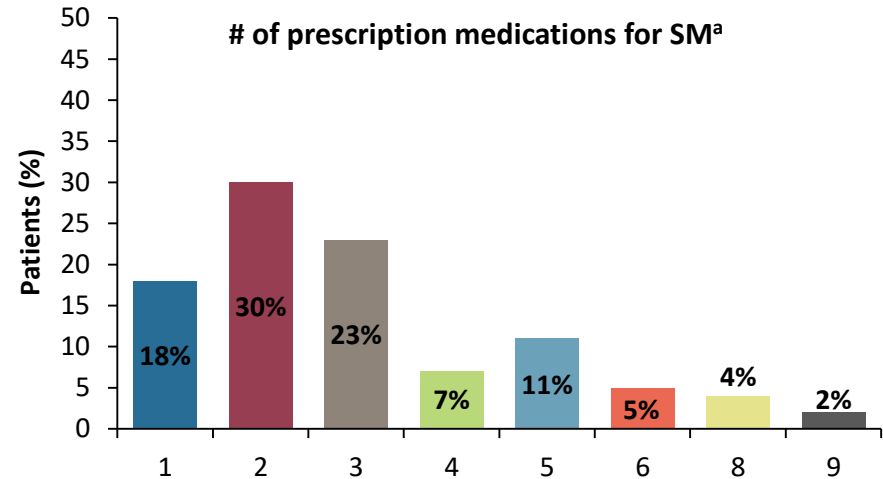
Participants reported frequent visits to multiple physician specialists for their SM symptoms



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

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

- Participating patients
- ClearView healthcare partners
- Paragon, UK

