

Disease Symptomology, Quality of Life (QoL), and Employment Status Among Patients with Systemic Mastocytosis (SM)

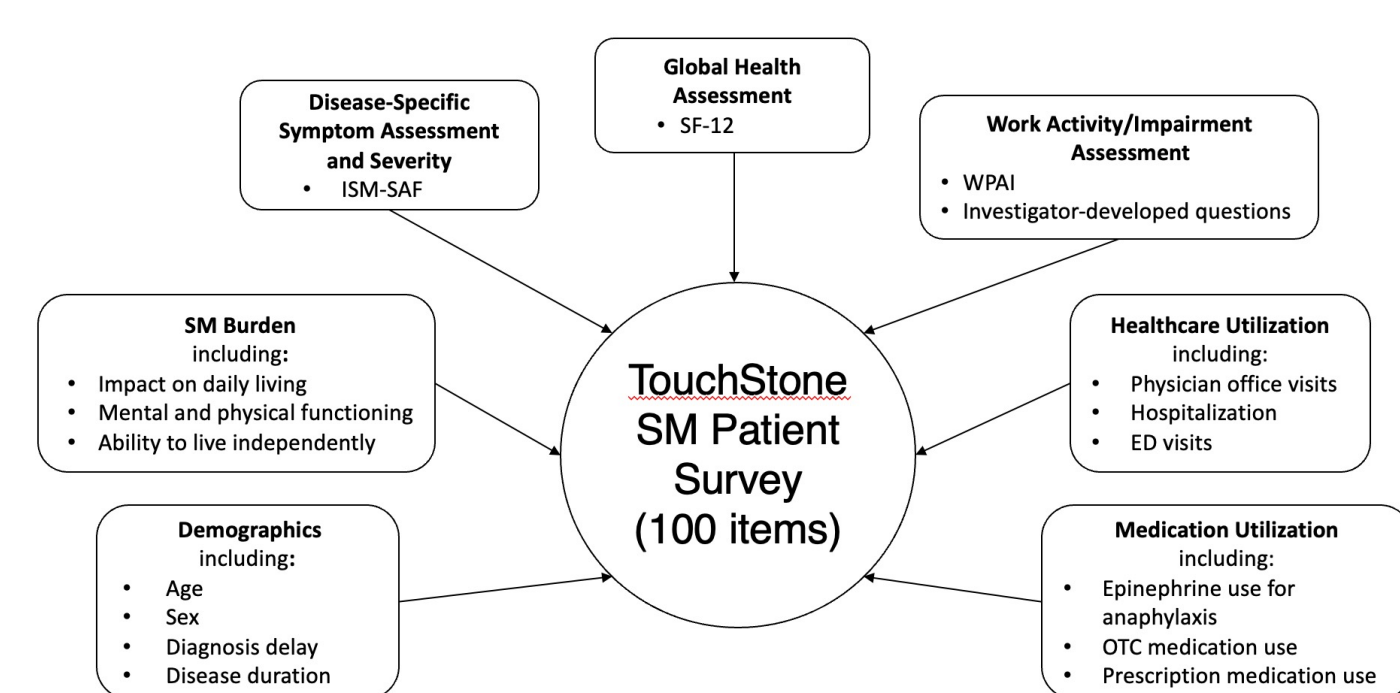
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Background

- The TouchStone SM Patient Survey was a cross-sectional study conducted in 2019 of adults ≥18 years of age who self-reported a diagnosis of systemic mastocytosis (SM) and lived in the US.
- Recruitment was conducted via the Mast Cell Connect Registry (MC Connect), a patient registry owned and managed by Blueprint Medicines Corporation (BPMC) that was initiated in 2015 to advance understanding of mastocytosis and its impact on patients.^{1,2}
- To date, there is a lack of understanding of how severity of SM symptoms affects other areas of patient functioning and well-being.
- The TouchStone study sought insights into the relationship between the severity of SM symptoms and patients' ability to work, healthcare utilization patterns, and patient reported outcome measures (Figure 1).

FIGURE 1. COMPONENTS OF TOUCHSTONE SM PATIENT SURVEY



Abbreviations: SM=systemic mastocytosis; ISM-SAF=Indolent Systemic Mastocytosis Symptom Assessment Form; SF-12=12-Item Short Form Survey; WPAI=Work Productivity and Activity Impairment Questionnaire

ISM-SAF Background

- 12-item questionnaire completed by patients that assesses severity of 11 ISM-specific symptoms.
- Designed with input from disease experts, patients and regulatory authorities to support regulatory approval.¹
- A reduction of 30% in ISM-SAF TSS is considered clinically meaningful based on its correlation to a 1-2 point change in PGIS.²
- TSS and domain scores calculated as 14 day moving average or one time score.

FIGURE 2. ISM-SAF SYMPTOMS ASSESSED, DOMAINS, AND SCORING

Symptom	Domains	Score
Abdominal pain	GI (0 – 30)	Scored 0 – 10 daily (24-hour recall) on a handheld device 0 is no symptoms 10 is worst imaginable
Diarrhea		
Nausea		
Spots	Skin (0 – 30)	
Itching		
Flushing		
Brain Fog	Neurocognitive (0 – 30)	
Headache		
Dizziness		
Bone pain		
Fatigue		
Total Symptom Score (0-110)		

Results

- Of the 56 survey respondents (89% female; median age 48 years), 84% (n=47) had moderate/severe symptoms as delineated by a TSS ≥28; 16% (n=9) had mild symptoms (TSS <28) (Table 1).
- Patients with moderate/severe ISM have a higher level of severity across all measured ISM symptoms (Figure 3).
- Mean physical and mental component SF-12 scores were lower (worse) for patients with moderate to severe SM symptoms compared with patients with mild SM symptoms (Table 2, Figures 4 and 5).
- Patients with moderate to severe disease reported not working due to SM at a much higher rate than those with mild symptoms (32% vs. 0%) (Figure 6).
- Patients with moderate to severe symptoms were more likely to take early retirement (8.5% vs 0%), voluntarily quit (29% vs 11%), reduce work hours (57% vs 33%), or be terminated from their employment (17% vs 11%) due to their SM than patients with mild symptoms (Figure 7).
- Patients with mild disease reported pain interfering with work in and outside the home 'A little bit' (44%) or 'Not at all' (33%), while 49% of those with moderate to severe disease reported pain interfering with work 'Extremely' or 'Quite a bit' (Figure 8).
- Patients with moderate/severe SM visited the emergency room at a much higher rate of 1.38 visits/patient, compared to patients with mild SM (0.33 visits/patient) (Table 3).

TABLE 3. PATIENT DEMOGRAPHICS

	TSS Score Range		
	Overall n=56	TSS <28 n=9	TSS ≥28 n=47
Patients, n	56	9	47
Median age (range), years	48 (20 – 76)	54 (26-74)	46 (20-76)
Female, n (%)	50 (89)	7 (78)	43 (92)
Married, n (%)	33 (59)	7 (78)	26 (55)
Mean time from symptom onset to diagnosis, years	6.0	6.2	5.5
Mean time since initial SM diagnosis, years	6.7	8.7	6.2
SM subtype n (%)			
ISM	37 (66)	5 (56)	32 (68)
ASM	5 (9)	1 (11)	4 (9)
SSM	3 (5)	0	3 (6)
SM-AHN	1 (2)	1 (11)	0 (0)
Unknown	10 (18)	2 (22)	8 (17)
Mean number of physicians seen for SM prior to diagnosis	5.8	5.1	6.0
Diagnosing Physician Type, n (%)			
Allergist/Immunologist	24 (43)	4 (44)	20 (43)
Dermatologist	13 (23)	1 (11)	12 (26)
Hematologist/Oncologist	12 (21)	3 (33)	9 (19)
Gastroenterologist	3 (5)	0 (0)	3 (6)
Other	4 (7)	1 (11)	3 (6)
Primary physician who manages SM, n (%)			
Allergist/Immunologist	33 (59)	4 (44)	29 (61)
Hematologist/Oncologist	12 (21)	4 (44)	8 (17)
General Practitioner/PCP	9 (16)	1 (11)	8 (17)
Other	2 (4)	0 (0)	2 (4)
Setting of care for primary SM physician n (%)			
Academic hospital	18 (32)	4 (44)	14 (30)
Multi-specialty group/HMO	16 (29)	2 (22)	14 (30)
Single specialty group	5 (9)	0 (0)	5 (10)
Solo practice	9 (16)	2 (22)	7 (15)
Community hospital	2 (4)	0 (0)	2 (4)
Other	4 (7)	1 (11)	3 (6)
Not sure	2 (4)	0 (0)	2 (4)

ASM: Aggressive Systemic Mastocytosis; SSM: Smoldering Systemic Mastocytosis; SM-AHN: Systemic Mastocytosis with Associated Hematological Neoplasm; PCP: Primary Care Provider; HMO: Health Maintenance Organization

TABLE 4. RELATIONSHIP BETWEEN TSS AND SF-12 PCS AND MCS

	TSS Score Range		
	Overall n=56	TSS <28 n=9	TSS ≥28 n=47
TSS mean [median]	52.6 [56]	19.4 [22]	59.0 [59]
SF-12 PCS mean (SD)	35.1 (12.5)	43.3 (11.8)	33.9 (12.0)
SF-12 MCS mean (SD)	37.8 (9.9)	46.8 (7.5)	37.8 (9.5)

SF-12: 12-item short form survey; PCS: Physical Composite Score; MCS: Mental Composite Score

TABLE 5. HCRU AND POLYPHARMACY BY TSS SCORE

	TSS Score Range		
	Overall n=56	TSS <28 n=9	TSS ≥28 n=47
Mean OTC Medications Taken for SM (median)	3 (3)	3 (3)	3.1 (3)
Mean prescription Medications Taken for SM (median)	3.1 (3)	2.11 (2)	3.3 (3)
Number of HCP Visits in 1 year n (%)			
<7 visits	19 (34)	6 (67)	13 (28)
7-13 visits	21 (38)	3 (33)	18 (38)
>13 visits	16 (29)	0 (0)	16 (34)
Mean emergency room visits for anaphylaxis, 2019 (n)	1.2 (68)	0.3 (3)	1.4 (65)
Patient visits to physician for SM related inquiry over 1 year, n (%)			
Primary Care			
0 visits	12 (21)	4 (44)	8 (17)
≥ 1 visit	44 (80)	5 (55)	39 (83)
≥ 3 visits	28 (51)	2 (22)	26 (55)
≥ 6 visits	13 (24)	1 (11)	12 (26)
≥ 12 visits	6 (11)	0 (0)	6 (13)
Epinephrine Self-Injectable Uses, n (%)			
Patients reporting 0-1 uses	48 (86)	8 (88)	40 (85)
Patients reporting ≥ 2 Uses	8 (14)	1 (11)	7 (15)
Mean injections in 2019	2.1	0.3	2.5

OTC: Over The Counter; HCP: Healthcare Providers

FIGURE 3. INDIVIDUAL SYMPTOM SCORES FOR TOUCHSTONE SM PATIENTS ON ISM-SAF

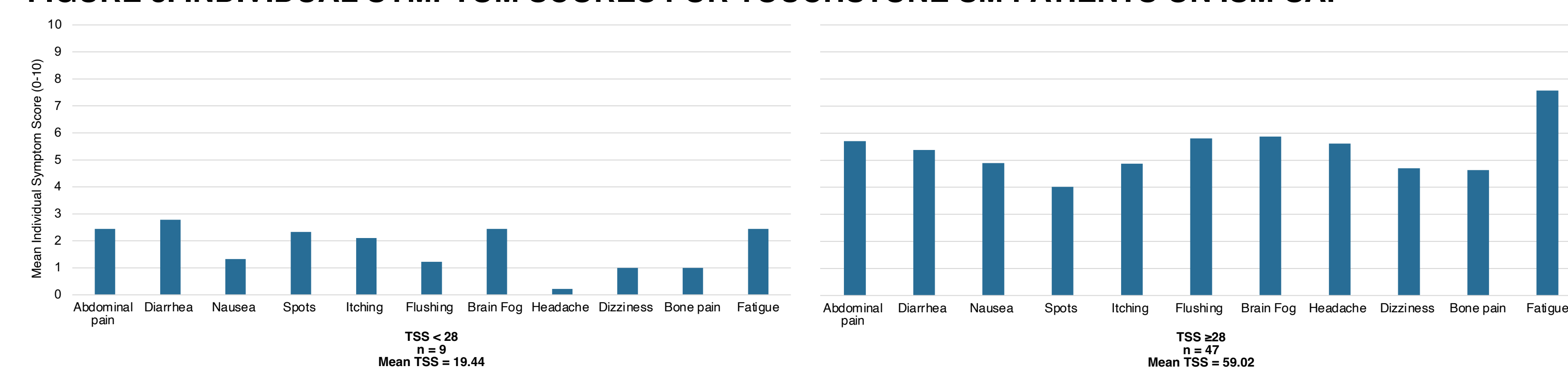


FIGURE 4. CORRELATION BETWEEN TSS AND SF-12 PCS AMONG TOUCHSTONE SM PATIENTS⁺

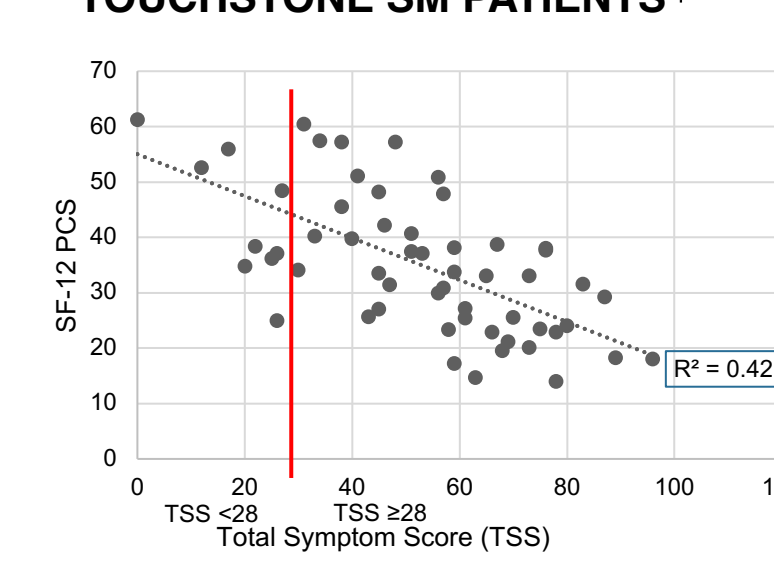
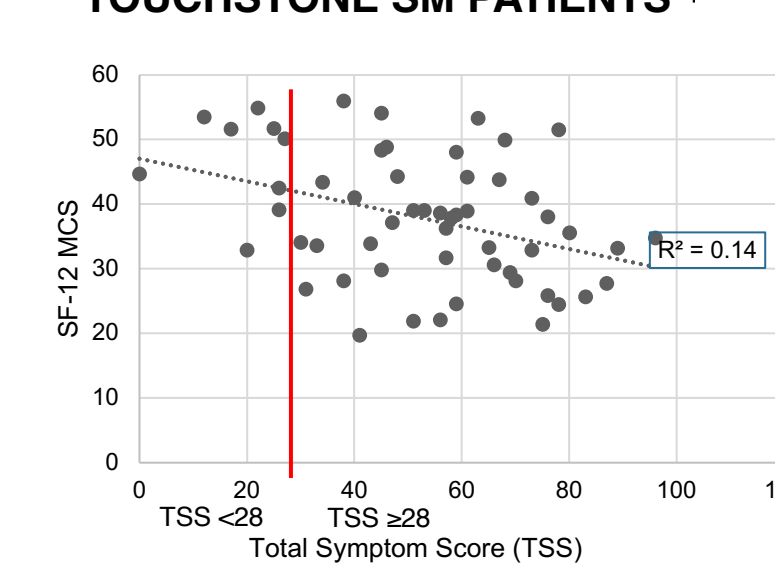


FIGURE 5. CORRELATION BETWEEN TSS AND SF-12 MCS AMONG TOUCHSTONE SM PATIENTS⁺



⁺Negative correlation is expected due to different scoring systems for SF-12 and ISM-SAF.

FIGURE 7. AS A RESULT OF YOUR SM, HAVE YOU EVER...?

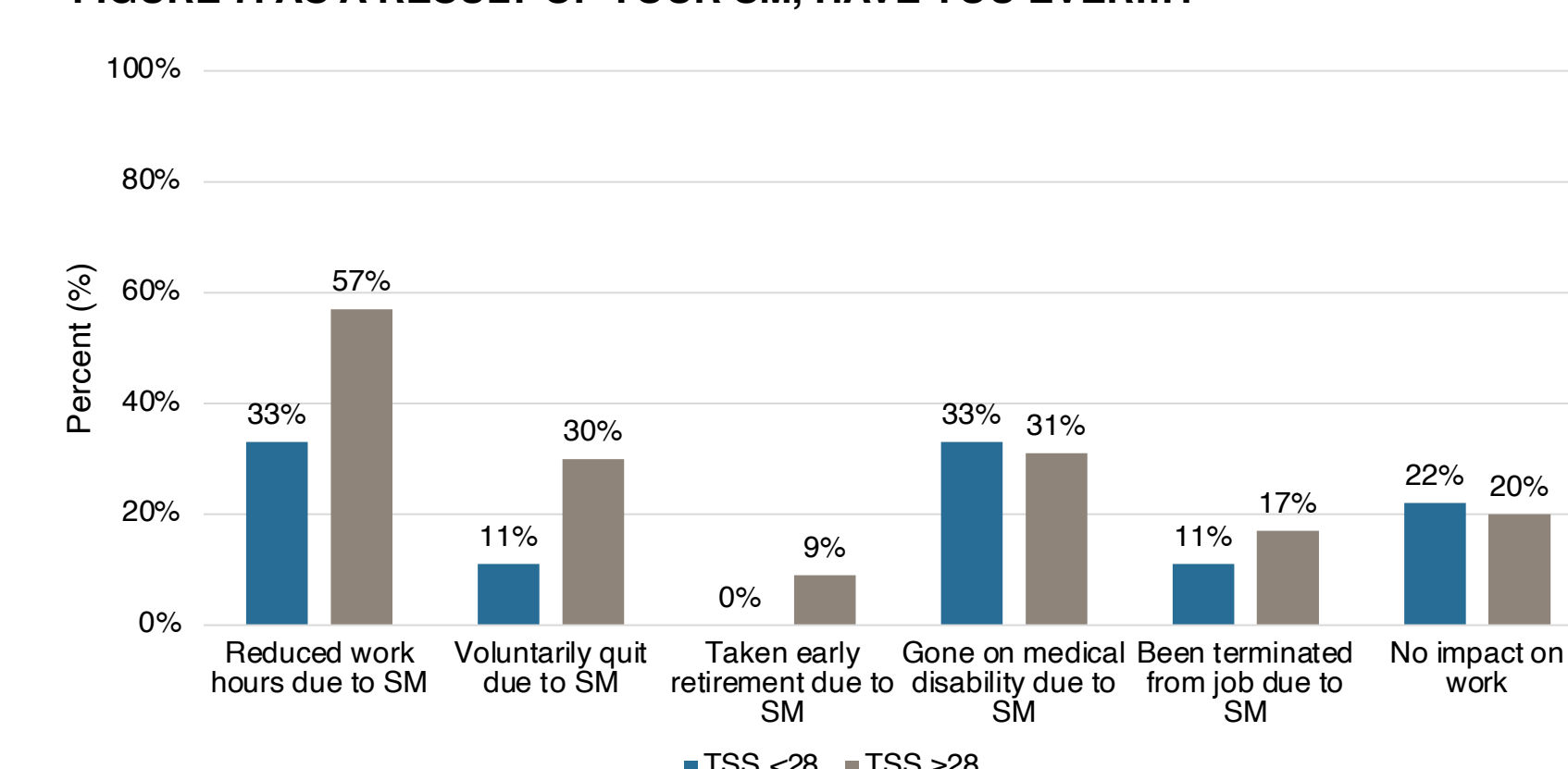


FIGURE 6. EMPLOYMENT STATUS OF TOUCHSTONE SM PATIENTS BY TSS SCORE

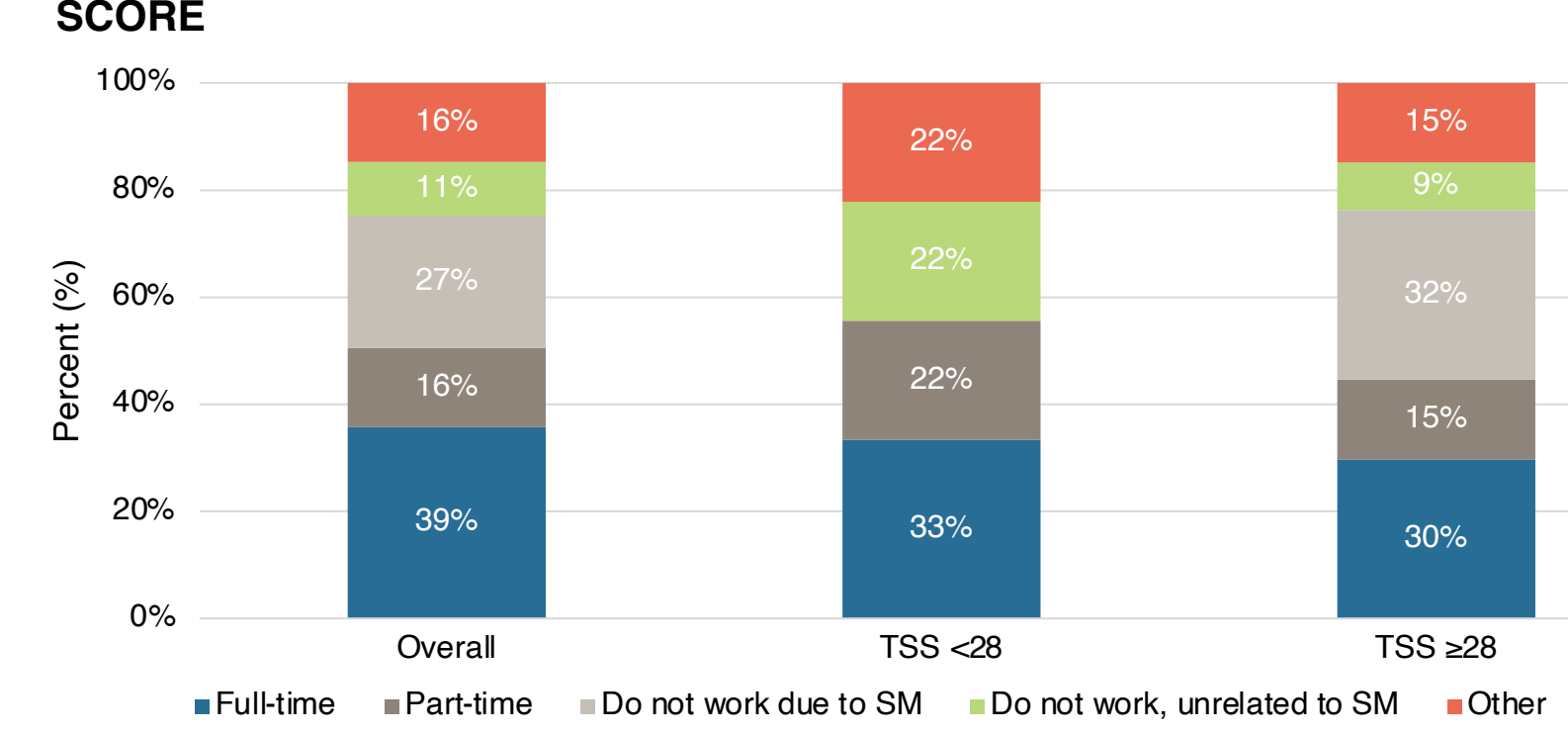
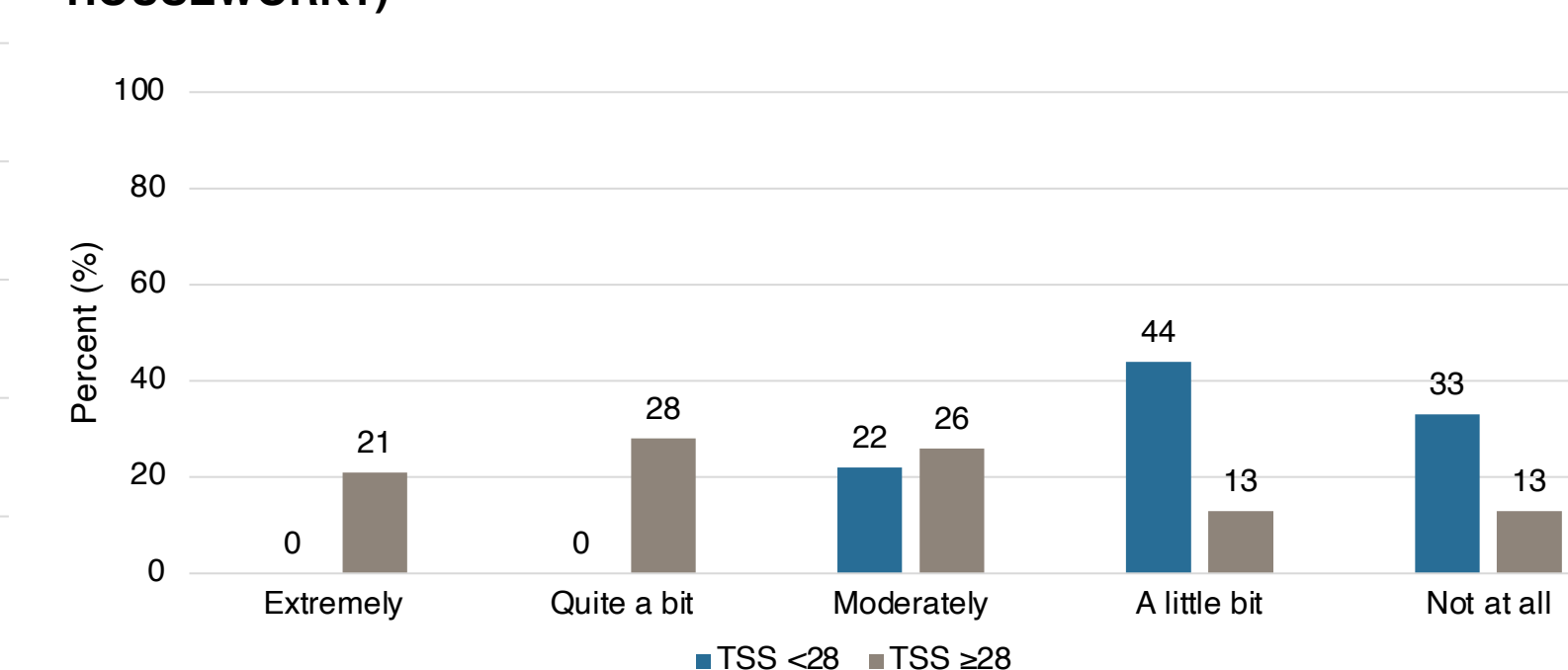


FIGURE 8. DURING THE PAST 4 WEEKS, HOW MUCH DID PAIN INTERFERE WITH YOUR NORMAL WORK (INCLUDING WORK OUTSIDE THE HOME OR HOUSEWORK?)



Methods

- Patients ≥18 years residing in the United States with a 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 (Figure 2), SF-12 (Table 1) and WPAI* (Table 2) questionnaires.
- The online survey also included questions related to the following:
 - 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)
- This study is a sub-analysis comparing patients with mild vs. moderate to severe ISM symptom burden on measures of work impairment and quality of life.

TABLE 1. SF-12 ASSESSMENT AREAS AND DESCRIPTION

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

TABLE 2. WPAI* ASSESSMENT AREAS AND DESCRIPTION

Assessment	Description
"During the past 4 weeks, how much did pain interfere with your normal work (including work outside the home or housework?)"	5-Point Likert scale (responses range from 'Not at all' to 'Extremely') 4-week recall period
As a result of your SM, have you ever?	Check all that apply
Reduced hours at work	Check one Prior to COVID-19 pandemic
Voluntarily quit your job	
Taken early retirement	
Gone on medical disability	
Been terminated from your job	
No impact at work by disease	
How would you describe your work status in February 2020?	

*Questions include those directly from WPAI and investigator-designed work productivity questions

Conclusions

- Compared to ISM patients with mild disease, patients with moderate to severe ISM symptoms report the following:
 - Worse mental well-being and physical functioning (i.e., SF-12)
 - Inability or impaired ability to work,
 - Higher rates of physician visits,
 - Increased rates of emergency room visits for anaphylaxis, and
 - Increased use of epinephrine.
- TSS scores generated by the validated ISM-SAF tool differentiate between ISM patients with mild versus moderate/severe symptoms and may offer clinicians a useful way to better understand the impact of ISM symptoms on daily functioning and patient well-being.
- This study is limited by its small sample size and stratification of groups, and further research to assess the burden of SM disease is warranted.

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