Original Article

Perceptions of patient disease burden and management approaches in systemic mastocytosis: Results of the TouchStone Healthcare Provider Survey

Ruben A. Mesa, MD[1]; Erin M. Sullivan, PhD, MPH[2]; David Dubinski, MBA, BS[2]; Brittany Carroll, MBA[2]; Valerie M. Slee, RN, BSN[3]; Susan V. Jennings, PhD[3]; Celeste C. Finnerty, PhD[3]; Linda S. Bohannon, RN, BSN, MSM[4]; Susan D. Mathias, MPH[5]; Betsy J. Lahue, MPH [6]; and Mariana C. Castells, MD, PhD[7]

BACKGROUND: Systemic mastocytosis (SM) is a rare clonal neoplasm driven by the *KIT* D816V mutation and has a broad range of debilitating symptoms. In this study, the authors evaluated SM disease perceptions and management strategies among US health care providers (HCPs). **METHODS:** Hematologist/oncologist (H/O) HCPs and allergist/immunologist (A/I) HCPs who were treating four or more patients with SM completed an online, 51-item TouchStone HCP Survey, which queried provider characteristics, perceptions of disease burden, and current management. Descriptive analyses by specialty and SM subtype were performed. **RESULTS:** Of 304 HCPs contacted, 111 (37%) met eligibility criteria, including 51% A/I specialists and 49% H/O specialists. On average, the HCPs had 14 years of practice experience and cared for 20 patients with SM. A/I HCPs saw more patients with nonadvanced SM (78%) compared with H/O HCPs, who saw similar proportions of patients with nonadvanced SM (54%) and advanced SM (46%). HCPs reported testing 75% of patients for the *KIT* D816V mutation and found an estimated prevalence of 47%. On average, HCPs estimated 8 months between symptom onset and SM diagnosis. HCPs reported that 62% of patients with indolent SM felt depressed or discouraged because of symptoms. In terms of treatment goals for SM, both types of specialists prioritized symptom improvement for nonadvanced SM and improved survival for advanced SM while also prioritizing improving patient quality of life. **CONCLUSIONS:** Both A/I and H/O specialists highlighted unmet needs for patients with SM. The HCPs surveyed reported a lower rate of *KIT* D816V mutations and a perceived shorter time between symptom onset and SM diagnosis compared with published estimates. ***Cancer* 2022;128:3700-3708**.

LAY SUMMARY:

- Specialists treating systemic mastocytosis (SM) completed a 51-item questionnaire about their clinical practices and perceptions of disease impact.
- The study included 111 hematology, oncology, allergy, and immunology physicians.
- Physicians reported that most patients had nonadvanced disease, yet SM symptoms significantly disrupted their patients' lives.
- Physicians estimated that SM is diagnosed within months of symptom onset, in contrast with published reports of years' long delays reported by patients with SM.
- This study identified unmet needs that can inform educational and patient management priorities in this rare disease.

KEYWORDS: cross-sectional studies, health care provider (HCP) perceptions, *KIT* D816V, myeloid neoplasm, myeloproliferative neoplasm, practice patterns, systemic mastocytosis.

INTRODUCTION

Systemic mastocytosis (SM) encompasses a collection of rare neoplasms characterized by clonal proliferation, accumulation, and activation of aberrant mast cells (MCs).[1,2] Up to 95% of SM cases are driven by the *KIT* D816V mutation, which constitutively activates KIT receptor tyrosine kinase, driving MC proliferation.[3–5] Mastocytosis includes several phenotypes, from cutaneous mastocytosis (skin manifestations only) to SM (systemic involvement) and MC sarcoma.[6] Nonadvanced SM includes indolent SM (ISM) and smoldering SM (SSM), whereas advanced SM includes aggressive SM, MC leukemia (MCL), and SM with an associated hematologic neoplasm.[7] It is estimated that SM affects 32,000 adults in the United States, and 95% of these cases are categorized as nonadvanced.[8] ISM is the most common subtype of nonadvanced SM, accounting for approximately 82% of all related cases.[8]

Corresponding Author: Ruben A. Mesa, Mays Cancer Center at the University of Texas T Health San Antonio, The University of Texas MD Anderson Cancer Center, Hematology, 7979 Wurzbach Road, San Antonio, TX 78229, USA (mesar@uthscsa.edu).

[1]Mays Cancer Center at The University of Texas Health San Antonio MD Anderson Cancer Center, San Antonio, Texas, USA; [2]Blueprint Medicines Corporation, Cambridge, Massachusetts, USA; [3]The Mast Cell Disease Society, Inc., Sterling, Massachusetts, USA; [4]Cancer Support Community, Washington, District of Columbia, USA; [5]Health Outcomes Solutions, Winter Park, Florida, USA; [6]Alkemi LLC, Manchester Center, Vermont, USA; [7]Division of Allergy and Clinical Immunology, Brigham and Women's Hospital, Boston, Massachusetts, USA

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Although advanced SM is associated with a worse prognosis, characterized by organ damage and shortened life expectancy compared with nonadvanced disease,[1,7,9] patients with all subtypes may present acute and/or chronic symptoms, which can be severe and unpredictable, affecting activities of daily living and negatively affecting quality of life (QoL).[5,10] Symptoms reported by patients with SM include skin, gastrointestinal, neurocognitive, and life-threatening anaphylactic events.[11,12] Despite severe symptoms, patients have reported a median of 7 years from symptom onset to SM diagnosis, and approximately 50% of patients consult between three and six different physicians when seeking diagnosis and treatment for SM.[10]

Surveys on patient experiences with MC disease[10] and myeloproliferative neoplasms are reported in the literature,[13,14] including a recent study[15] linking perceptions of providers to a corresponding survey of their patients with myeloproliferative neoplasms; however, there is a dearth of information specifically on SM practice patterns from the clinician perspective. The purpose of this study was to gain insight into the practice patterns of clinicians caring for patients with SM, their perceptions of unmet patient needs and disease burden, and their satisfaction with current management strategies.

MATERIALS AND METHODS

Recruitment and study population

Investigators used double-blinded market research survey panels to contact 304 physician health care providers (HCPs). Before accessing survey items, HCPs reviewed the risks, benefits, and strict confidentiality of the survey tool. Consenting HCPs completed three screening questions; HCPs who specialized as allergists/immunologists (A/I) or hematologists/oncologists (H/O), had at least four patients with SM in their practice, and had a minimum of 3 years of post-fellowship practice experience were eligible to participate. Data collection for this online survey took place between June 26, 2020, and July 14, 2020. Participating HCPs received a nominal honorarium.

Survey instrument design

The TouchStone HCP Survey contained 51 items, required approximately 15–20 minutes to complete, and was organized into four key domains: provider characteristics, disease assessment, patient burden, and current management. *Provider characteristics* (11 items) covered practice setting, practice characteristics, and information about how HCPs diagnose SM, including *KIT* D816V mutation testing and time to diagnosis. *Disease assessment* (15 items) captured the assessment of symptoms HCPs commonly encountered in practice and asked HCPs which guidelines they referenced for the treatment of patients with SM. *Patient burden* (14 items) used scaled responses (ranging from *not at all* to *a great deal*) to collect HCP perceptions of the impact of SM symptoms on their patients' activities of daily living and potential limitations. Finally, *current management* (11 items) assessed how HCPs were treating patients with SM and queried their satisfaction with overall treatment and management options. For instance, HCPs were asked to estimate the time spent with each patient in the clinic, the percentage of patients with ISM who were treated aggressively (and how aggressive treatment was defined), and how both over-the-counter and prescribed medications were used to manage patient symptoms. HCPs were also asked to assess treatment side effects on their patients' QoL, to designate the first and second most important treatment goals for ISM and advanced SM.

Statistical analysis and reporting

Statistical data analysis was performed using SAS software, version 9.4 (SAS Institute Inc.). Results were reported for each item across the four survey domains and stratified by physician specialty (A/I and H/O). Measures of central tendency and dispersion (e.g., mean, median, interquartile range, and standard deviation) were computed for all survey items. The reported annual numbers of per-patient appointments (two survey questions) were examined for reasonableness, and outlier responses of >100 visits annually were censored (four HCP responses for appointments with patients who had nonadvanced SM appointments and three HCP responses for appointments with patients who had advanced SM). Analyses of responses related to advanced SM were limited to HCPs who had these types of patients in their practice. Findings were further evaluated for trends related to practice setting and volume of patients with SM. For survey items reported as continuous variables, a Student *t*-test was used to assess whether a statistically significant difference in the mean response existed across comparisons. Categorical variables were evaluated for independence using the Fisher exact test or the χ^2 test. The significance level was predefined as .05 for all analyses.

RESULTS

Of the 304 HCPs contacted, 39% ($n = 119$) consented to participate. Eight HCPs did not meet eligibility criteria based on their responses to screening questions and were excluded. Completed surveys for 111 physicians were analyzed.

Provider characteristics

Respondents included 57 (51%) A/I specialists and 54 (49%) H/O specialists who had an average of 14.1 years of post-fellowship practice experience. These HCPs reported having an average of 19.6 patients with SM in their practice, with H/O providers reporting significantly more patients compared with A/I providers ($p < .01$). Nonadvanced SM accounted for the majority of patients with SM for all HCP practices, and A/I physicians treated a significantly higher proportion of patients with nonadvanced disease than H/O physicians ($p < .0001$; Table 1). On average, physicians estimated that it took 8.5 months for a typical patient with SM in their practice to receive an SM diagnosis from the time of symptom onset and estimated that 47.3% of patients had the *KIT* D816V driver mutation. Further details on provider characteristics, perceptions of diagnosis timing, and reported testing practices are depicted in Table 1.

HCP respondents represented a wide array of practice settings. Academic hospital outpatient clinics were the most commonly reported setting overall, followed by single specialty group practices. Together, 76% of HCPs reported academic and/or specialty practice settings, and the majority of HCPs (64%) reported that they routinely discussed their patients who had SM with a multidisciplinary team. A breakdown of respondent practice setting by specialty is provided in Table 2.

Disease assessment

Table 3 reports physician perceptions of SM symptoms and disease severity by physician specialty. All survey responses on symptom severity were in the moderate-to-severe range (on a scale from 0 [absent] to 10 [very severe]). A/I physicians reported significantly higher mean symptom severity than H/O physicians for patients who had ISM and advanced SM ($p < .05$). A/I respondents also reported that a significantly greater proportion of their patients who had ISM recognized their symptoms as related to this condition (ISM) compared with patients of H/O respondents ($p < .05$). No significant differences between specialists were observed in the proportions of patients who had no symptoms, mild symptoms, severe symptoms, or very severe symptoms. However, A/I physicians reported a significantly higher proportion of patients with ISM who had moderate symptoms compared with their H/O counterparts ($p < .01$). Specialists reported referencing different guidelines for SM treatment; *National Comprehensive Cancer Network* guidelines were referenced by 85% of H/O HCPs versus 26% of A/I HCPs; whereas American Academy of Allergy, Asthma, and Immunology guidelines were referenced by 68% of A/I HCPs versus 11% of H/O HCPs.

Patient burden

HCP respondents indicated that the feelings and lives of their patients with ISM were significantly disrupted by the disease and reported that patients with ISM felt at least somewhat anxious or worried, isolated, and depressed or discouraged because of their condition (Fig. 1). Greater than 50% of HCPs surveyed perceived limitations related to ISM symptoms across all aspects of

TABLE 1. Provider Characteristics and Perceptions on Systemic Mastocytosis Diagnosis

Survey item	All HCPs: Mean ± SD $n = 111$	Mean		p[a]
		A/I HCPs, $n = 57$	H/O HCPs, $n = 54$	
No. of patients with SM in practice	19.6 ± 17.87	15.2	24.2	.007
Years in practice as an attending physician	14.1 ± 7.04	13.4	14.9	.286
Proportion (%) of patients with nonadvanced SM	62.3 ± 25.75	77.7	46.1	< .0001
Proportion (%) of patients with advanced SM	37.7 ± 25.75	22.3	53.9	< .0001
Annual no. of appointments per patient for a typical patient with nonadvanced SM[b]	9.11 ± 14.96	3.8	14.0	.001
Annual no. of appointments per patient for a typical patient with advanced SM[c]	10.23 ± 11.05	5.6	14.1	.001
Time from initial presentation to SM diagnosis, months	5.4 ± 9.63	6.4	4.4	.286
Time from symptom onset to SM diagnosis, months	8.5 ± 9.19	9.8	7.1	.112
No. of physicians seen prediagnosis by a typical patient with SM	3.5 ± 1.78	3.8	3.1	.026
Percentage of patients with SM tested for the *KIT* D816V mutation	74.9 ± 31.35	80.6	68.8	.047
Percentage of patients with SM harboring *KIT* D816V mutation	47.3 ± 29.89	45.0	49.7	.408

Abbreviations: A/I, allergist/immunologist; HCPs, health care providers; H/O, hematologist/oncologist; SD, standard deviation; SM, systemic mastocytosis.
[a]Calculated using Student *t*-test.
[b]For this category, 50 H/O responses and 57 A/I responses were analyzed ($n = 107$).
[c]For this category, 51 H/O responses and 42 A/I responses were analyzed ($n = 93$).

TABLE 2. Respondent Practice Setting by Specialty

Practice setting	Percentage of Respondents (No.)			p[a]
	All HCPs, $n = 111$	A/I HCPs, $n = 57$	H/O HCPs, $n = 54$	
Academic hospital inpatient only	4.5 (5)	0.0 (0)	9.3 (5)	< .0001
Academic hospital outpatient clinic	30.6 (34)	22.8 (13)	38.9 (21)	
Community hospital	9.0 (10)	5.3 (3)	13.0 (7)	
Community hospital inpatient only	3.6 (4)	0.0 (0)	7.4 (4)	
Multispecialty group/HMO	15.3 (17)	24.6 (14)	5.6 (3)	
Single specialty group	25.2 (28)	26.3 (15)	24.1 (13)	
Solo practice	11.7 (13)	21.1 (12)	1.9 (1)	

Abbreviations: A/I, allergist/immunologist; HCPs, health care providers; HMO, health maintenance organization; H/O, hematologist/oncologist.
[a]Calculated using the Fisher exact test.

TABLE 3. Physician Perceptions of Systemic Mastocytosis Symptoms and Severity by Specialty

Survey item	All HCPs, $n = 111$	A/I HCPs, $n = 57$	H/O HCPs, $n = 54$	p[a]
Mean severity of symptoms for patients with nonadvanced ISM or SSM on a scale from 0 (absent) to 10 (very severe)	4.96	5.28	4.63	.043
Mean severity of symptoms for patients with advanced SM on a scale from 0 (absent) to 10 (very severe)[b]	7.49	7.93	7.15	.024
Proportion (%) of patients with ISM able to recognize their symptoms as related to SM	51.4	57.2	45.2	.018
Mean time spent treating a typical patient with ISM per visit, minutes	38.5	36.8	40.2	.768
Proportion (%) of patients with ISM and no symptoms	11.7	9.7	13.9	.146
Proportion (%) of patients with ISM and mild symptoms	30.9	28.0	33.9	.122
Proportion (%) of patients with ISM and moderate symptoms	32.8	37.9	27.4	.002
Proportion (%) of patients with ISM and severe symptoms	15.6	15.5	15.7	.915
Proportion (%) of patients with ISM and very severe symptoms	9.0	8.9	9.1	.930

Abbreviations: A/I, allergist/immunologist; HCPs, health care providers; H/O, hematologist/oncologist; ISM, indolent systemic mastocytosis; SM, systemic mastocytosis; SSM, smoldering systemic mastocytosis.
[a]Calculated using Student *t*-test.
[b]The analysis of this survey item was restricted to respondents who treated advanced SM ($n = 96$), including 51 H/O specialists and 45 A/I specialists.

daily life for their patients, including impact on school/university/work, sports/physical activity, sleep, sexual activity, leisure time, relationships, and ability to care for children.

Disease management

A breakdown of HCP rankings of the most important and second most important treatment goals for both ISM and advanced SM is provided in Figure 2. Overall, 41% of HCPs rated improving QoL as the most important treatment goal for patients with ISM, and 25% noted improvement of symptoms as the second most important treatment goal in this population. For HCPs who were caring for patients with advanced SM (HCPs, $n = 96$) and evaluating treatment goals, the most frequent choice for *most important* was *improved progression-free survival/overall survival*, and the top choice for *second most important* was *delay disease progression and reduce the risk of organ damage*. Although there was no significant difference in the *most important* treatment goals for ISM between A/I and H/O physicians, distribution of the *second most important* treatment goal differed significantly ($p = .0018$) between specialists. (Note: Data by HCP type are not included in the figures.)

The physicians surveyed both reported and described aggressively treating an average of 52% of their patients with ISM with prescription drugs. For instance, respondents provided examples of *aggressive treatment*, such as using multiple therapies, chemotherapy, and steroids. A significantly higher proportion of A/I physicians reported a pharmaceutical management approach compared with H/O physicians (58% vs. 46%; $p = .011$). Furthermore, 85% of all respondents noted that treatment side effects affected overall QoL *at least somewhat* in patients with ISM. HCPs expressed differing levels of satisfaction with the overall treatment and management of SM by disease severity. As shown in Figure 3A, 7% of HCPs were *very satisfied* with overall treatment and management of ISM, and 18% expressed some dissatisfaction. For advanced SM, as shown in Figure 3B, 4% of HCPs were *very satisfied* with overall treatment and management, and 29% reported some dissatisfaction.

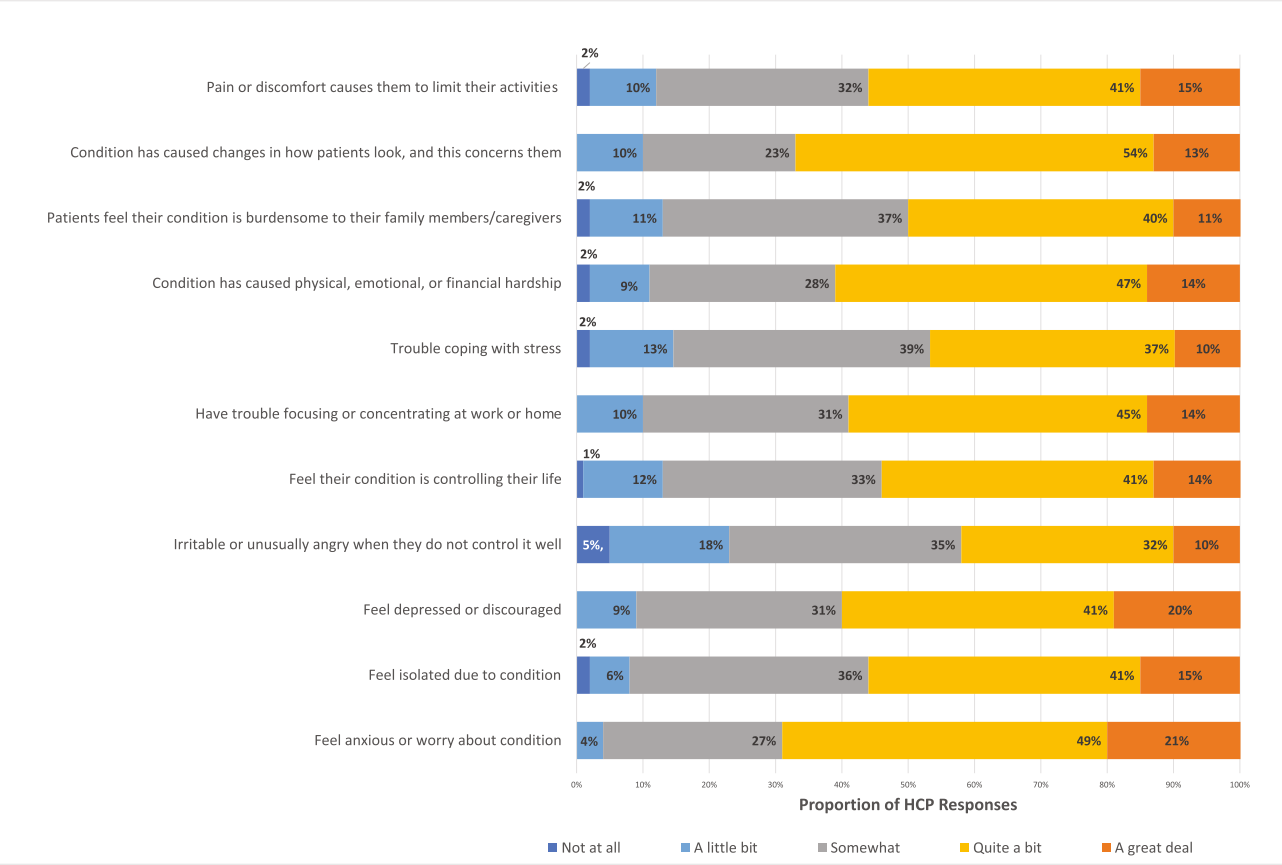

FIGURE 1. HCP perception of disease impact on feelings and daily life of patients with indolent systemic mastocytosis (n = 111). HCP indicates health care provider.

Overall, a majority (58%) of HCPs reported that they were *somewhat satisfied* or *very satisfied* with overall treatment and management of their patients who had ISM; however, fewer than one half (37%) expressed satisfaction with the overall treatment and management of their patients who had advanced SM.

Patient volume and practice setting

Survey findings on HCP perceptions and practice patterns did not differ by patient volume or practice setting, with a few exceptions. Compared with higher volume practices (>15 patients with SM), HCPs at lower volume practices (≤15 patients) reported spending more time per appointment with patients who had nonadvanced SM (34 vs. 31 minutes, $p = .038$), treating a higher proportion of their patients with either type of SM aggressively using prescription drugs ($p < .05$), and managing a lower proportion of patients with advanced SM (31% vs. 47%; $p < .0001$). Larger practice size was associated with the practice settings more frequently reported by H/O specialists; however, the majority of both specialists (73% of A/I physicians and 78% of H/O physicians; $p = .62$) reported either academic or specialty practice affiliation. Compared with community/solo practice HCPs, academic/specialty HCPs reported testing a higher proportion of patients for the *KIT* 816V mutation (80% vs. 58%; $p = .004$) and using aggressive prescription treatment in greater proportions of patients with advanced SM (68% vs. 55%; $p = .04$).

DISCUSSION

The survey captured a balance of A/I and H/O specialists among respondents and reflects real-world practice because patients with SM routinely require the care of multiple specialists.[10] In comparing the SM populations seen by A/I specialists versus H/O specialists, we observed that A/I physicians care for a significantly higher proportion of patients with nonadvanced SM but, on average, manage fewer patients with SM; whereas H/O

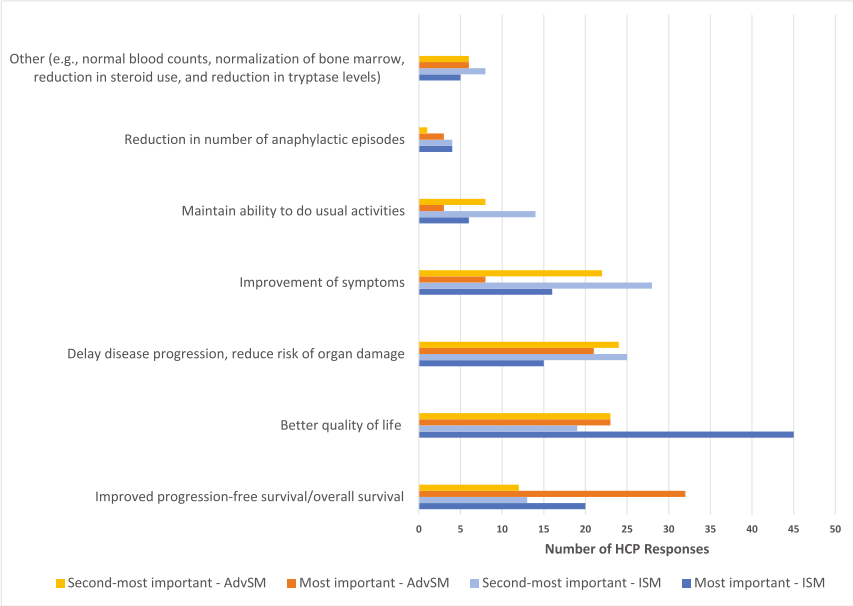

FIGURE 2. HCP-selected treatment goals for patients with indolent systemic mastocytosis ($n = 111$) and advanced systemic mastocytosis ($n = 96$). AdvSM indicates advanced systemic mastocytosis; HCP, health care provider; ISM, indolent systemic mastocytosis.

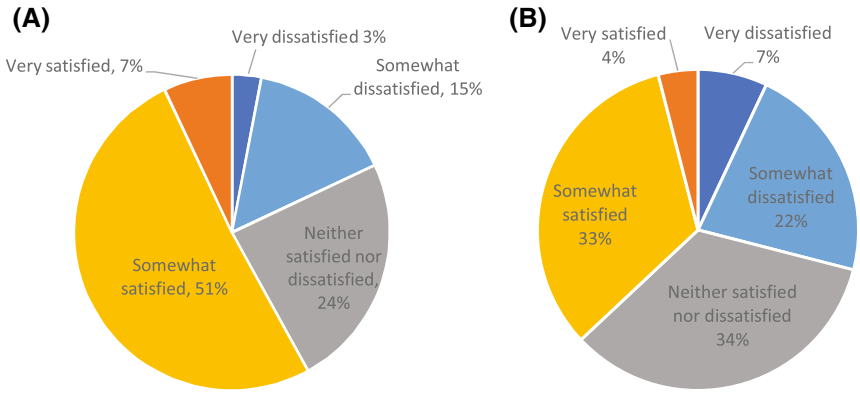

FIGURE 3. HCP satisfaction with overall treatment and management for (A) indolent systemic mastocytosis ($n = 111$) and (B) advanced systemic mastocytosis ($n = 96$). HCP indicates health care provider.

physicians care for a significantly higher proportion of those with advanced SM and report managing a larger caseload of patients with SM. H/O specialty, higher volume practice, and academic/specialty practice setting were each associated with greater proportions of patients who had advanced SM and more frequent use of prescription treatments in this patient group, whereas A/I specialty and a lower volume practice or a community/solo practice setting were associated with higher proportions of patients who had ISM and the use of aggressive treatments across both types of patients. Notably, compared with H/O physicians, A/I physicians reported a higher proportion of moderate symptoms in patients with ISM and indicated that their patients with non-advanced SM had a higher mean severity of symptoms. These factors, along with preferences for distinct clinical guidelines, may explain the differences in pharmaceutical management approaches reported by each specialty.

Regardless of specialty, setting of care, or volume of patients, the majority of HCPs reported consulting with

a multidisciplinary team about their patients with SM. Therefore, the practice patterns observed may also reflect the dynamics of SM disease progression. According to European Competence Network on Mastocytosis registry data, 4.9% of patients with ISM progress to either SSM or advanced SM, and 9.4% of patients with SSM progress to advanced SM when followed for a median of 4 years.[16] In SSM, rates of progression or leukemic transformation as high as 18% have been documented.[17–19]

The perceived average of 8.5 months between symptom onset and SM diagnosis reported by HCP survey respondents, which was consistent within a few months regardless of specialty affiliation, patient volume, or practice setting, differed markedly from data reported in the literature regarding the diagnostic odyssey of patients with SM. For instance, in a study evaluating patient perceptions in MC disorders from the Mast Cell Connect Registry (MC Connect; ClinicalTrials.gov identifier NCT02620254), which is owned and managed by Blueprint Medicines Corporation, it was reported that patients experienced a median duration of 7 years between symptom onset and an accurate diagnosis of SM.[10] Patients with advanced disease in the MC Connect study had a median delay of 3 years from symptom onset to diagnosis, whereas those with nonadvanced SM had a median delay of 9 years.[10] Finally, in a recent survey of patients with SM conducted concurrent to this provider survey, patients reported a mean of approximately 6 years between symptom onset and receipt of physician diagnosis.[20] Our HCP study suggests that there is a gap in provider knowledge regarding the challenge of diagnosing SM. Closing this gap may be especially crucial because patients with ISM report provider knowledge as a potential barrier to timely SM diagnosis.[10]

TouchStone HCP respondents largely reported testing for the *KIT* 816V mutation, and this practice was more common for HCPs who were affiliated with academic and specialty practices. HCPs estimated that 47% of their patients with SM harbored the *KIT* D816V driver mutation, whereas published estimates report >90% prevalence of this mutation.[3,11] For instance, a study[3] of 113 adults with systemic MC diseases prospectively analyzed purified bone marrow MCs. Those investigators identified the presence of the *KIT* D816V mutation in 93% of patients who had indolent or aggressive forms of SM.[3] Similarly, a retrospective study of 342 adults with SM treated at the Mayo Clinic between 1976 and 2007 detected the *KIT* D816V mutation in the bone marrow-derived DNA of a majority of patients.[9] The discordance may be related to selection bias because most published studies are from reference centers where *KIT* D816V mutation detection may have higher sensitivity in patients with low MC burden, such as those who have ISM.

In the current study, both types of providers reported that the severity of symptom burden varied greatly between patients with indolent disease. However, our findings suggest a high burden of SM-related symptoms and underscore the need to stratify patients with ISM by symptom severity to support optimal management. One notable finding related to the debilitating nature of nonadvanced SM is the extent to which HCPs reported disability among patients with ISM, which has been reported by other studies.[10] Providers in the TouchStone HCP Survey noted that most patients with ISM felt at least *quite a bit* depressed or discouraged, consistent with existing data estimating that depression occurs in up to 60% of patients with SM.[21,22] These findings were consistent when evaluated across specialty type, practice setting, and by volume of patients with SM.

Although improved survival was the most important treatment goal noted for patients with advanced SM, it remained an important consideration in nonadvanced SM and was the second most commonly selected *most important* treatment goal for patients with ISM. The emphasis on survival-based treatment goals in advanced SM is not surprising considering the historically poor prognosis of these subtypes, which are associated with a median overall survival <3 years.[23] Median overall survival is even lower in patients who have SM with an associated hematologic neoplasm or MCL (2 years and 2 months, respectively).[1,7,9] Our study indicated that the goals of delaying disease progression/reducing the risk of organ damage and improving symptoms were commonly selected as the *most important* and *second most important* treatment goals across both advanced and nonadvanced SM, suggesting that there are common considerations in managing the implications of SM on involved organ systems across the spectrum of disease severity. Furthermore, existing literature[10,20] on treatment goals reported by patients suggests an opportunity to improve alignment between HCPs and patients. Prior research reported that patients with MC diseases, including SM patients, are troubled by perceived gaps in provider disease knowledge; prefer definitive, curative-intent therapy to symptom-based, multimodality treatment; and desire greater integration of holistic care approaches.[10]

Limitations

The cross-sectional nature of our survey limits the conclusions that can be drawn about patterns and trends among HCPs who care for patients with SM. The 39% survey response rate could also be considered a limitation but is consistent with HCP response rates documented

in the literature.[24] Although A/I and H/O physicians play crucial roles in the diagnosis and treatment of SM, the exclusion of other specialists who care for patients with SM may limit the generalizability of these results. Future surveys should collect additional details to further evaluate relationships of HCPs with specialty centers, referral patterns, and access to advanced SM testing methods. In addition, the survey methodology may introduce biases related to question order, demand characteristics, or responding bias because of the scaled responses. Desirability bias was less likely in this study given the strict confidentiality and anonymity of HCP survey responses; however, the online recruiting methods may be biased toward physicians with electronic contact details. Finally, HCP responses regarding patient experiences were not linked to a survey of the patients with SM who they treat, which limited our ability to draw conclusions about whether HCP perceptions align with actual patient experiences. However, concurrent to TouchStone HCP Survey administration, patients with SM were recruited for a TouchStone SM Patient Survey.[20] Therefore, whereas patients of surveyed HCPs were not interviewed to gauge patient-reported outcomes or patient goals for SM treatment, findings from the TouchStone SM Patient Survey[20] (reported elsewhere in this issue) shed light on their disease and health care experiences and perceptions.

CONCLUSIONS

The TouchStone HCP Survey provides valuable insights into the diagnosis and management of patients with SM by A/I and H/O physicians and documents physician perceptions around disease burden and severity. Regardless of specialty, caseload of patients with SM, or practice setting, HCPs perceived a markedly lower prevalence of the *KIT* D816V mutation and a shorter duration between symptoms and diagnosis for patients who have SM compared with estimates published in the literature, suggesting a need for greater physician awareness. Respondents noted increasing survival and improving QoL as key treatment goals in SM. HCPs also confirmed a significant need for improved SM management, with nearly one third noting at least some dissatisfaction with the overall treatment and management of patients who have advanced SM. Furthermore, a majority indicated that patients with ISM feel depressed or discouraged because of SM and experience symptom-related limitations in daily activities. Future studies could integrate HCP-reported findings with insights directly from patients who have SM to better identify treatment gaps and opportunities to improve the quality of care for this patient population.

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AUTHOR CONTRIBUTIONS

Ruben A. Mesa: Supervision (lead), conceptualization (equal), methodology (equal), writing–original draft (supporting), and review and editing (equal). **Erin M. Sullivan:** Conceptualization (equal), methodology (equal), writing–original draft (lead), formal analysis (equal), and writing–review and editing (equal). **David Dubinski:** Project administration (lead), conceptualization (lead), methodology (equal), and writing–review and editing (equal). **Brittany Carroll:** Conceptualization (supporting), methodology (equal), visualization (lead), and writing–review and editing (equal). **Valerie M. Slee:** Conceptualization (supporting), formal analysis (supporting), writing–original draft (supporting), and writing–review and editing (equal). **Susan V. Jennings:** Conceptualization (supporting), methodology (equal), formal analysis (supporting), writing–original draft (supporting), and writing–review and editing (equal). **Celeste C. Finnerty:** Conceptualization (supporting), methodology (equal), formal analysis (supporting), writing–original draft (supporting), and writing–review and editing (equal). **Linda S. Bohannon:** Conceptualization (supporting), methodology (equal), writing–original draft (supporting), and writing–review and editing (equal). **Susan D. Mathias:** Conceptualization (supporting), methodology (equal), formal analysis (supporting), writing–original draft (supporting), and writing–review and editing (equal). **Betsy J. Lahue:** Conceptualization (supporting), methodology (supporting), formal analysis (lead), writing–original draft (lead), and writing–review and editing (lead). **Mariana C. Castells:** Conceptualization (supporting), methodology (equal), writing–original draft (supporting), and writing–review and editing (equal).

CONFLICTS OF INTEREST

Erin M. Sullivan, David Dubinski, and Brittany Carroll own stock/stock options as employees of Blueprint Medicines Corporation, which develops therapies to treat systemic mastocytosis. Rubin A. Mesa, Linda S. Bohannon, Susan D. Mathias, and Mariana C. Castells received nominal research compensation for their time as part of the TouchStone Survey research team. Linda Bohannon reports grants or contracts from Labcorp, Geron, Amgen, Merck, and Alkermes outside the submitted work. Betsy J. Lahue is employed by Alkemi LLC, a firm that receives consulting fees from Blueprint Medicines Corporation.

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