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ORIGINAL RESEARCH

Patient and Advanced Practitioner Perspectives on Symptom Burden and Symptom Management in Indolent Systemic Mastocytosis

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Authors' disclosures of conflicts of interest are found at the end of this article.

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Abstract

Background: Symptom burden is the primary driver for patients with indolent systemic mastocytosis (ISM) to seek medical care, whether or not they are diagnosed. **Purpose:** This descriptive study aimed to describe the advanced practitioner (AP) and ISM patient perspective relative to the symptom burden of ISM, multidisciplinary diagnosis and management of ISM, barriers to symptom management, strategies for collaborative management of ISM, and communicative health literacy in patients with ISM. **Methods:** An ISM patient survey and AP survey were developed by an AP-led steering committee incorporating validated tools to measure symptom burden, symptom burden impact, barriers, and strategies for improving symptom burden. Surveys were embedded in Qualtrics and were deployed by Conexiant to a convenience sample of AP members of the Advanced Practitioner Society for Hematology/Oncology (APSHO), AP members of the American Initiative in Mast Cell Diseases, and patients affiliated with The Mast Cell Disease Society between December 22, 2024, and February 3, 2025. **Findings:** 50 APs and 53 ISM patients completed 100% of the questions on the corresponding surveys. The symptom burden described using the Indolent Systemic Mastocytosis Symptom Assessment Form (ISM-SAF) to identify the symptoms that are most common, most challenging, and have the greatest impact on quality of life aligns with published data for patients in this survey. Only 24% (*n* = 13) of ISM patients indicated their disease was well controlled, while 76% of APs indicated greater than 50% of their ISM patients had well-controlled disease (*n* = 38). Most APs (68%) in the survey indicated they saw one to five ISM patients per year but were comfortable with managing ISM-related symptoms (54%, *n* = 27). Practice patterns for triage,

multidisciplinary management, and shared decision-making are described. **Conclusions:** This is the first ISM symptom burden–focused survey to provide a direct comparison of patient responses to those of APs in hematology/oncology and allergy and immunology. Indolent systemic mastocytosis symptom burden measurement and symptom burden reduction remain challenging, with several barriers and gaps identified in this study. The APSHO Toolkit for Systemic Mastocytosis, developed in parallel to this study, provides an AP-focused resource for overcoming some of the barriers and gaps identified in this study.

Indolent systemic mastocytosis (ISM) is a rare clonal mast cell neoplasm with highly heterogenous presentation that is characterized by chronic, often debilitating, and at times life-threatening symptom burden (Pardanani, 2023). The symptoms associated with ISM are largely related to mast cell activation and the subsequent release of mast cell mediators. Mast cells are embedded in multiple organ systems, including the bone marrow, gastrointestinal tract, liver, lungs, and skin (Valent et al., 2023). Symptom burden is the primary driver for ISM patients, either diagnosed or undiagnosed, to seek medical care. The heterogeneity of symptoms in ISM, the changes in symptom burden over time, and the number of health-care providers involved in the evaluation of symptoms contribute to the delay in confirming a diagnosis (Gotlib et al., 2024). Presenting signs and symptoms in patients living with ISM often mimic other disorders, contributing to the delay in reaching a diagnosis. Patients living with rare diseases, including ISM, see an average of 7.3 providers across multiple specialties before receiving an accurate diagnosis (Jennings et al., 2018). The median time from symptom onset to diagnosis of ISM is 3 years but may take as long as 9 years (Jennings et al., 2018).

Symptoms and clinical findings are at the core of the differential diagnosis. Clinicians, including advanced practitioners (APs), rely on patients to describe their symptoms, including onset, triggers, duration, intensity, and any measures to mitigate them. For patients with ISM, triggers for mast cell activation are central to symptom onset, severity, and overall symptom burden (Gotlib et al., 2024). Patient-centered communication, shared decision-making, and strategies to empower patients in self-management, including communicative health literacy, are critical to effective symptom management (Bylund et al., 2023; LeBlanc et al., 2019). Communicative health literacy implies the synthesis and application of information to engage in health self-management. Symptom burden reduction is a primary goal in the management of ISM and continues to be a primary endpoint across clinical trials for investigational agents for ISM (Gotlib et al., 2024). Uncontrolled symptom burden is associated with inferior health-related quality of life in patients with ISM (Zeiger et al., 2025).

Although presenting signs and symptoms may vary widely across ISM patients and change over time, strategies to elicit hallmark symptoms across all subspecialties may aid in early diagnosis. Recognizing the challenge most APs face in managing a broad range of diagnoses, the Advanced Practitioner Society for Hematology and Oncology (APSHO) convened an AP-led multidisciplinary steering committee to evaluate the role of APs in the diagnosis, management, and support of patients living with ISM. Additionally, the steering committee included a patient living with ISM and a research scientist from a mast cell disease advocacy organization to simultaneously evaluate the patient perspective.

This descriptive study aimed to describe the AP and ISM patient perspective relative to the symptom burden of ISM, multidisciplinary diagnosis and management of ISM, barriers to symptom management, strategies for collaborative management of ISM, and communicative health literacy in patients with ISM. The results of online surveys simultaneously distributed to hematology/oncology AP members of APSHO, allergy and immunology APs, and patients living with ISM will be described.

METHODS

This descriptive study explored AP and ISM patient perspectives. A systematic review of extant literature and the culmination of a series of

multidisciplinary and patient meetings (Kurtin et al., 2025), provided the foundation for the development of an AP-focused online survey and an ISM patient–focused online survey. The AP survey aimed to evaluate the AP perspective of ISM symptom burden, practice patterns, tools and strategies for the multidisciplinary management of ISM, and barriers to evaluating and managing ISM symptom burden.

The ISM patient survey aimed to evaluate the patient perspective relative to ISM symptom burden, multispecialty health-care provider interactions prior to and following an ISM diagnosis, barriers to managing symptom burden, and elements of communicative health literacy. Questions eliciting input about symptom burden were developed based on items in the Indolent Systemic Mastocytosis Symptom Assessment Form (ISM-SAF), a validated tool used across multiple clinical trials to measure patient-reported symptom burden (Padilla et al., 2021; Shields et al., 2023; Taylor et al., 2021). The ISM-SAF includes questions about the severity of symptoms across four domains (gastrointestinal, dermatological, neurocognitive, and systemic), encompassing 11 symptoms. Symptoms are rated as mild, moderate or severe. Additional symptoms were added to the survey based on input from the steering committee and a review of the literature. The AP surveys mirrored the patient survey to capture perspectives about symptom burden but also included questions about practice profile, multidisciplinary management, polypharmacy, triage, and communication of symptom burden across providers. Elements of shared decision-making were also included in the AP survey.

Communicative health literacy was measured using items from the Health Literacy Questionnaire (HLQ; Osborne et al., 2013). The HLQ is a 43-item scale that includes nine conceptually distinct areas of health literacy to assess the needs and challenges of a wide range of people and organizations. Each subscale can be used independently without compromising the reliability and validity of the tool. Three additional questions to elicit information about health technology engagement were added to the survey based on the lead author's previous work (Kurtin, 2016).

Both surveys were embedded in the Qualtrics platform. Members of the steering committee tested the online surveys prior to deploying the surveys to the target sample for functionality, time to completion, and content. A convenience sample of patients living with ISM was recruited through The Mast Cell Disease Society. A convenience sample of APs was recruited through the APSHO member directory designating experience in hematologic malignancies and AP members of the American Initiative in Mast Cell Diseases. Surveys were collected between December 22, 2024, and February 3, 2025. Qualtrics data were sorted for frequencies, coded, and loaded to SPSS software for additional analysis.

The survey questions in this exploratory study posed no harm to either the patients or the APs. Given little to no risk, no institutional review board approval was required or obtained. Respondents were provided with a disclaimer and could choose to participate. Patients were not required to provide any personal identifying data to complete the survey. Advanced practitioner members of APSHO or the American Initiative in Mast Cell Diseases were provided with the purpose of the survey and submitted their name and email address for follow-up notification at the end of the survey at their request.

RESULTS

Fifty APs completed 100% of the survey questions. The average time required to complete the survey was 15.65 minutes (range: 7.5–62 minutes). Demographic questions aimed to create a practice and experiential profile for APs. Most APs identified as nurse practitioners (56%, n = 28), with fewer physician assistants (PA; 36%, n = 18), pharmacists (6%, n = 3), and a single clinical nurse specialist (CNS, 2%, n =1). Thirty-two (66%) APs reported working in hematology/oncology, with a mix of practice types, including 43% practicing in academic or comprehensive cancer centers, and 57% working in community-based hospitals, office settings, or remote telemedicine practices. Sixteen percent of APs had 5 years or less experience as an AP. The remainder of APs had more experience (30% with 6–10 years; 18% with 11–15 years; 14% with 16–20 years; 20% with > 20 years). Forty-one percent (n = 13) of hematology/oncology APs reported working in the specialty for 11 to more than 20 years, compared with 11%

(*n* = 2) of allergy and immunology APs reporting 11 to 15 years of experience in the specialty.

Fifty-three ISM patients completed 100% of the survey questions. The average time required to complete the survey was 22.57 minutes (range: 8–88 minutes). Demographic data focused on the lived ISM experience. The mean age at diagnosis of ISM patients in this survey was 44.5 years (range: 24–69 years). Years since diagnosis ranged from 43 to less than 1 year (mean: 8 years). The time between the onset of symptoms and having a confirmed diagnosis of ISM ranged between less than 1 year (15%, *n* = 8) and more than 20 years (38%, *n* = 20). Patients in this survey indicated they had never had a bone marrow biopsy (62%, *n* = 33), had a single bone marrow biopsy (25%, *n* = 13), or had three or more biopsies (13%, *n* = 7). Interestingly, APs indicated most of their patients (76%, *n* = 37) had two or more bone marrow biopsies.

Symptom Burden in SM

Symptom burden reduction remains a primary endpoint for most clinical trials investigating treatments for ISM. Improving or controlling symptoms, or "making them livable" is a common goal for patients living with ISM. When asked "On your current treatment regimen, do you consider your disease…", responses were mixed, with only 24% (*n* = 13) indicating their disease was well controlled, and most indicating their disease was either moderately well controlled (*n* = 29, 55%) or not well controlled (*n* = 11, 21%). When asked, "What percentage of patients with ISM in your practice are currently well controlled on their current treatment," most APs indicated that greater than 50% of patients were well controlled (76%, *n* = 38). Most APs (68%) in the survey indicated they saw one to five ISM patients per year but were comfortable in managing ISM-related symptoms (54%, *n* = 27). The discordance in the patient and AP perspective is not surprising given the limited experience APs in this survey have managing patients with ISM.

The 10 most common symptoms reported by patients (ranked on a scale of 1 to 3) included fatigue (70%), spots (57%), brain fog (53%), diarrhea (49%), shortness of breath or wheezing (38%), tachycardia (34%), blood pressure instability (25%), itching (25%), abdominal pain (23%), and bone pain (21%; Table 1). Patients ranked fatigue as the most common, most challenging and most unpredictable symptom with the greatest impact on quality of life (QOL), although it was not identified as the most severe symptom in the 60 days prior to the survey. Among the other most common symptoms, brain fog is common, most challenging to manage, unpredictable, and has a high impact on QOL. Diarrhea is common, challenging to manage, and has a significant impact on QOL. Abdominal pain, although not as common, was reported as the most severe symptom in this patient group in the 60 days preceding the survey and has a significant impact on QOL. Spots were reported as common, recurring, and unpredictable, but rated to have less impact on QOL. As would be expected, anaphylaxis is unpredictable and has a moderate impact on QOL. Interestingly, many of the symptoms reported as severe in the 60 days prior to the survey were not listed as the most common or challenging symptoms to manage.

A reduction in symptom burden and improvement in QOL is a universal goal when managing patients with ISM. Advanced practitioners identified fatigue as the most common symptom (71%), the fourth most challenging symptom to manage (45%), and the second most likely to impact QOL (57%; Table 2). Brain fog (74%), blood pressure instability (57%), abdominal pain (47%), fatigue (45%), and itching (38%) were identified as the five most challenging symptoms to manage by APs. Itching (63%), fatigue (57%), brain fog (55%), diarrhea (45%), and anaphylaxis (43%) were identified as having the greatest impact on quality of life by APs.

Measuring symptoms and communicating symptom burden measures over time is critical to the effective management and evaluation of the efficacy of interventions. The ISM-SAF has been the primary tool used to date for ISM-focused clinical trials (Shields et al., 2023). In this survey, only 26% (*n* = 14) of patients indicated they were familiar with the ISM-SAF, and only 11% (*n* = 6) indicated it was used consistently in their visits with healthcare providers.

Some APs in this study (40%, *n* = 20) indicated they used the ISM-SAF inconsistently or not at all (10%, *n* = 5). Twenty percent of APs (*n* = 10) did not know what the ISM-SAF was. Only

Table 1. **Systemic Mastocytosis Patient-Reported Symptom Burden**

Symptom	Impact on QOL	Most common, %	Most challenging to manage, %	Recurring over the last 60 days, %	Severe over the last 60 days, %	Unexplained in the last 60 days, %	Unpredictable in the last 60 days, %
Fatigue	77	70	55	8	19	26	44
Brain fog	53	53	55	25	23	30	21
Diarrhea	49	49	53	23	23	13	15
Abdominal pain	37	23	15	23	38	17	8
Itching	36	25	28	28	26	21	13
Flushing	34	19	28	19	32	25	13
Bone pain	30	21	19	22	32	17	11
Anaphylaxis	30	2	10	21	11	4	10
Spots	26	57	41	30	24	8	17
Headaches	25	19	19	26	32	19	7
Tachycardia	23	34	32	31	23	12	6
Shortness of breath/wheezing	19	38	42	34	21	8	10
Blood pressure instability	17	25	22	26	30	9	2
Nausea/vomiting	15	19	19	22	34	9	4
Dizziness	11	19	21	28	28	9	6
Sweating	4	6	15	28	25	15	2

Note. QOL = quality of life.

Table 2. Advanced Practitioner Estimates of Systemic Mastocytosis Symptom Burden

Symptom	Impact on QOL	Most common, %	Most challenging to manage, %
Itching	63	65	38
Fatigue	57	71	45
Brain fog	55	63	74
Diarrhea	45	29	20
Anaphylaxis	43	14	24
Abdominal pain	35	50	47
Blood pressure instability	29	18	57
Flushing	29	23	35
Nausea/vomiting	26	26	24
Shortness of breath/wheezing	22	35	27
Spots	18	12	26
Dizziness	18	26	25
Headaches	14	10	2
Tachycardia	12	47	16
Bone pain	12	6	12
Sweating	6	8	18

31% (n = 15), indicated they used it consistently in practice. Consistent documentation of symptom burden can be hindered by the lack of integrated tools in the electronic medical record (EMR). Of the APs indicating they used the form consistently, only 1 (2%) indicated it was embedded in the EMR. The others scanned the paper document into the EMR. Scanned documents are often placed in erroneous sections of the EMR and do not allow clinicians to track trends over time. Advanced practitioners indicated they used the ISM-SAF items to structure their review of symptoms during patient visits (48%, n = 49), with fewer indicating they consistently documented symptom burden in the clinic visit note (34%, n = 17). When asked if they found measuring symptom burden over time difficult, 54% (n = 27) strongly agreed or agreed, 34% were neutral (n = 17), and 12% (n = 6) disagreed.

Understanding symptom burden in ISM requires consideration of triggers. Triggers are well described as the primary cause of mast cell activation and mediator release and the primary source of ISM symptoms. Individual triggers vary by individual patient and may change over time. Documenting and tracking triggers are critical to avoiding them and reducing symptom frequency and severity, particularly in the case of anaphylaxis. The most common triggers in this patient group were emotional stress (45) and physical stress (42; Table 3). Food or beverage (41), heat (40), temperature change (39), alcohol (36), environmental stress (35), fatigue (34), chemical odors (33), and sun/sunlight (33) round out the top 10 triggers. For 28 patients, triggers have not been clearly identified. Considering the symptom burden profile and difficulty in avoiding triggers that are a natural part of most people's day-to-day life, it is not surprising that ISM patients become isolated and, in some cases, homebound due to fear of triggers and poorly controlled symptoms.

Multidisciplinary Management of ISM Symptom Burden

Most patients with ISM see multiple health-care providers across multiple specialties prior to and after their diagnosis with ISM. In this patient group, the average number of health-care providers seen prior to a diagnosis of ISM was 9 (range: 2–70). The most common health-care providers seen by this patient group prior to and after the diagnosis of SM are presented in Table 4. When

asked who they considered to be their ISM champion, patients identified a hematologist/oncologist (59%, *n* = 31), allergist/immunologist (22.6%, *n* = 12), mast cell allergy and immunology specialist (11.1%, *n* = 6), gastroenterologist (2%, *n* = 1), or general medicine practitioner (2%, *n* = 1). Two patients designated "myself" in the "Other" category.

Polypharmacy is common in ISM patients. Most medications are over the counter and are not covered by insurance. Forty-one percent of patients in this study indicated they take 1 to 5 medications, 40% indicated they take 6 to 10 medications, and 19% indicated they take more than 15 medications. When asked who helped them to manage their medications, patients listed retail specialty pharmacists (32%), retail pharmacists (26%), and multiple health-care providers across 12 specialties. Again, there were several patients that entered "myself" in the "Other" category. The implications of polypharmacy, particularly for oral medications and patients that rely on multiple over-the-counter medications, are significant. The relatively young age of the patients in this study has additional implications.

Shared decision-making and patient-centered communication are critical to eliciting symptom burden, building trust, and empowering the patient to manage their health. Items for the HLQ were included in this survey to describe communicative health literacy among patient participants (Table 5). It is not surprising that all patients in this survey had access to the internet as this was an online survey and the average age was relatively young. In addition, most patients indicated they had access to a smartphone and were able to access the patient portal for their health-care system. Although most patients indicated they had access to at least one health-care provider who knows them (91%), gaps in knowledge and access to health-care providers exist, particularly with the onset of acute symptoms. Access to health-care providers in the setting of acute symptoms is critical to avoid emergency room or urgent care visits.

Triage procedures for the APs in this survey included a variety of team members, including call centers (22%), team-based registered nurses (32%), non-licensed staff (42%), a practice-based registered nurse (34%), and specialty nurse navigators (18%). Both APs (36%) and MDs (10%) also play a role in triage. Same-day appointments were possible in some practices if the patient called in early enough and the AP had room on their schedule the same day (66%, *n* = 33). Five APs (10%) indicated their centers had designated urgent care centers.

Communicating symptoms to a health-care provider requires organization of ideas, focus, energy, and time. The average time for a return visit for APs in this study was 30 minutes (range: 15–60). Advanced practitioners indicated the complexity of symptoms (64%), time (58%), patients' difficulty in communicating symptoms (40%), the number of providers involved in patients' care (32%), confidence in managing the disease (16%), and a lack of caregivers (14%) presented the greatest barriers to shared decision-making.

Table 3. Mast Cell Activating Triggers Reported by Patients With Systemic Mastocytosis

Trigger	Yes	No
Emotional stress	45	8
Physical stress	42	11
Food or beverage	41	12
Heat	40	13
Temperature change	39	14
Alcohol	36	17
Environmental stress	35	18
Fatigue	34	19
Chemical odors	33	20
Sun/sunlight	33	20
Natural odors	32	21
Cold	30	23
Exercise	30	23
Unidentified	28	25
Mechanical irritation	26	27
Venoms/insect stings	23	30
Medications	19	34
Filler or excipients in medications	18	35
Infections	17	36
Vaccinations	15	38
Contrast media	13	40
Surgery or procedures	13	40

Table 4. Collaborative Management of Patients With Systemic Mastocytosis

Medical/surgical specialty	Providers seen before diagnosis	Providers seen after diagnosis	Referrals made by APs for collaborative management
General Medicine	64% MD; 25% MD/AP	62% MD; 10% MD/AP	26% MD; 50% MD/AP
Emergency Medicine	44% MD; 14% MD/AP	30% MD; 4% MD/AP	18% MD; 22% MD/AP
Gastroenterology	53% MD; 6% MD/AP	47% MD; 4% MD/AP	22% MD; 58% MD/AP
Pulmonary	23% MD	26% MD; 2% MD/AP	24% MD; 42% MD/AP
Psychology/Psychiatry	15% MD; 15% MD/AP	17% MD; 8% MD/AP	26% MD; 20% MD/AP
Cardiology	38% MD, 8% MD/AP	38% MD; 4% MD/AP	36% MD; 38% MD/AP
Dermatology	55% MD; 17% MD/AP	51% MD; 6% MD/AP	24% MD; 58% MD/AP
Endocrinology	28% MD; 5% MD/AP	21% MD; 6% MD/AP	26% MD; 26% MD/AP
Rheumatology	36% MD; 6% MD/AP	23% MD; 4% MD/AP	34% MD; 20% MD/AP
Neurology	34% MD; 6% MD/AP	34% MD; 2% MD/AP	30% MD; 24% MD/AP
Integrative Medicine	13% MD; 4% MD/AP	11% MD	16% MD; 20% MD/AP
Hematology	47% MD; 8% MD/AP	68% MD; 2% MD/AP	24% MD; 50% MD/AP
Oncology	30% MD; 6% MD/AP	45% MD; 4% MD/AP	22% MD; 50% MD/AP
Allergy and Immunology	59% MD; 12% MD/AP	68% MD; 6% MD/AP	48% MD; 44% MD/AP
Mast Cell Allergy and Immunology Specialist	26% MD; 4% MD/AP	42% MD; 2% MD/AP	34% MD; 26% MD/AP
Mast Cell Hematology Specialist	32% MD; 4% MD/AP	53% MD; 2% MD/AP	44% MD; 20% MD/AP
Obstetrics and Gynecology	8% MD	6% MD	0
Orthopedics	2% MD	2% MD	0

DISCUSSION

Effectively characterizing and measuring symptom burden in patients living with systemic mastocytosis remains an elusive target. The heterogeneity of symptoms across patients and over time, the limited experience most clinicians have with this rare disease, and the numerous barriers inherent in a fragmented health-care system pose substantial challenges. Patients in this study see numerous health-care providers across multiple subspecialties as described in previous publications (Valent et al., 2023; Zeiger et al., 2025). While the ISM-SAF has been used as a validated tool to measure symptom burden over time for patients enrolled in clinical trials, uptake in practice may be limited by a lack of consistent use, not having the tool embedded in the EMR, and limited time.

Alternative tools for measuring symptom burden and the impact on quality of life for patients living with mastocytosis are used primarily in large studies. The Mastocytosis Quality of Life Questionnaire (MC-QoL), and the Mastocytosis Symptom Assessment Form (MSAF) are two well-established tools (van Anrooij et al., 2016). The length of the tools limits application in day-to-day practice. The Mastocytosis Control Test (MCT) incorporates elements of symptom assessment, impact on quality of life, and control of SM-related symptoms (Siebenhaar et al., 2018). The tool has established validity and reliability. It consists of five questions with five options each to estimate a disease control score. As there is currently no tool to measure disease control specific to ISM, this tool may offer a reasonable option for clinicians, although application may face similar barriers to those found in this study.

Advanced practitioners play a critical role in symptom assessment and management. Familiarity with the symptom profile of ISM, including triggers, will improve the AP approach to connecting those symptoms to a possible diagnosis of ISM strategies for reducing symptom burden

Table 5. Items Measuring Communicative Health Literacy and Health Technology Engagement.

Health Literacy Questionnaire (HLQ) item	Strongly agree, %	Agree, %	Neutral, %	Disagree, %	Strongly disagree, %
I have all the information I need to look after my health and my symptoms.	17	32	28	23	0
I have at least one health-care provider who knows me well and sees me consistently.	51	40	5	4	0
I have the health-care providers I need to help me work out what I need to do.	26	36	24	13	0
When I have acute symptoms, I have access to my health-care team to help me manage my symptoms.	23	23	15	32	7
I spend quite a lot of time actively managing my health.	47	40	7	2	4
When I feel ill, the people around me really understand what I am going through.	9	28	19	28	15
If I need help, I have plenty of people I can rely on.	21	21	28	22	8
I have at least one person who can come to medical appointments with me.	30	43	8	17	2
I have at least one health-care provider that I trust and would recommend to family and friends.	43	43	10	2	2
I look forward to seeing the health-care provider I rely on to manage my ISM.	40	32	23	6	0
Health Technology Engagement items					
I have consistent access to the internet.	81	17	2	0	0
I have consistent access to a smartphone.	82	15	2	2	0
I can access the patient portal at my health-care institutions.	58	23	4	7	8

(Kurtin et al., 2025). Recognizing the challenges specific to ISM and identifying red-flag symptoms that may spark an ISM-focused diagnostic workup may reduce the delay in diagnosis. The Toolkit for Systemic Mastocytosis, available at https://www.apsho.org/apsho-aptoolkit-sm, was developed in parallel to this survey and provides an AP-centric tool for understanding the ISM disease state, diagnostic workup, and strategies for management.

Although symptoms reported as common, challenging to manage, and having the greatest impact on quality of life were mostly aligned in this study, areas of discordance remain. Patient anecdotes offered during steering committee meetings, focus groups, and patient forums as a part of this study emphasized the unmet needs of patients living with ISM. "Not being seen, not being heard," "If you look okay, you must be okay," "I am basically homebound," "Trying to reach a livable symptom burden," "I know something is wrong, but I am told everything is normal," are just some of the quotes that were provided in the process of this study. In the process of developing the ISM-SAF, patients from 12 countries identified several unmet needs, including better clinician education about symptoms and mastocytosis, particularly neurologic and psychologic symptoms, and improved access to specialty centers and emergency care (Valent et al., 2021).

While multidisciplinary management of ISM is recommended as best practice (Gotlib et al., 2023), communication among providers, particularly when they are not in the same health system,

is lacking. Advanced practitioners in this study indicated the number of providers involved in the care of ISM patients, the complexity of symptoms, the difficulty patients have in communicating their symptoms, and the lack of caregivers as primary barriers to implementing shared decision-making. Limited time, demands of the EMR, complex patients with chronic health problems, and a lack of training are described as barriers to shared decision-making in the literature (Bylund et al., 2023; LeBlanc et al., 2019).

Elements of communicative health literacy indicated strengths and weaknesses for the patients in this study. Communicating their symptom burden over time and across numerous health-care providers and systems places ownership on the patient and their caregivers to maintain records, plan for visits, organize medications, and facilitate communication across providers. This level of engagement requires energy, time, and caregiver support. Given the prevalence of fatigue, brain fog, other debilitating symptoms, and the continual need to avoid stress and other triggers, ISM patients must rely on others to assist them in managing their disease. Caregiver burnout is prevalent across chronic illnesses, particularly those requiring frequent engagement with health-care systems and those that are associated with isolation (Kurtin et al., 2013; Yuen & Wilson, 2021). There is a paucity of published literature specific to caregivers of patients living with ISM.

Strengths and Limitations

The major strength of this study is that it was led by APs with a focus on the AP role in managing rare and complex diseases, in this case, ISM. The inclusion of a patient ambassador and advocacy research scientist in the development of the survey brought a real-world element to the survey design. The process of developing the questionnaire, including several discussion sessions, focus groups, steering committee meetings, and patient forums enriched the content and added a degree of realism to the process. Applying validated tools used in clinical trials and in practice in the survey questions will add to the literature and perhaps support progress in devising strategies for improved management of ISM. This is the first ISM symptom burden–focused survey to provide a direct comparison between ISM patient responses and responses of APs in hematology/oncology or allergy and immunology.

The limitations of the study were largely related to the convenience sample of patients recruited through an advocacy organization focused on supporting patients with mast cell diseases. As such, the patients in this survey were likely to be better educated about their disease and have the capacity to access information. Similarly, APs were recruited through APSHO, a professional organization supporting APs in hematology/oncology and through the American Initiative in Mast Cell Diseases that supports APs in the specialty. Given the rare nature of ISM, surveys of APs outside these organizations would likely produce different data. Use of an online survey and self-reported data are additional limitations.

FUTURE DIRECTIONS

Continued work toward improving management and control of the symptom burden associated with ISM was provided by the launch of the Toolkit for Systemic Mastocytosis on the APSHO website (https://www.apsho.org/apsho-aptoolkit-sm). Additional publications will weave data that was not included in this work specific to the challenges of polypharmacy in ISM. Continued development of resources for APs to better understand the management of treatments for mast cell mediator–driven symptoms and treatments targeting the driver mutation for SM (*KIT* D816V), will be necessary to expand the AP network for these patients. ●

Disclosures

Dr. Kurtin has served as a consultant for Agios, Blueprint Medicines, and GSK, and received honoraria from Agios and GSK. Ms. Kolb has served as a consultant for Blueprint Medicines. The remaining authors have no conflicts of interest to disclose.

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