The Burden of Systemic Mastocytosis in Europe: Results From the PRISM Patient Survey

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Introduction

- Systemic mastocytosis (SM) is a clonal mast cell disease primarily driven by the *KIT* D816V mutation and characterized by unpredictable and debilitating skin, gastrointestinal, and systemic symptoms[1,2]
- Previously, a US-based survey (Touchstone) reported insights from 56 SM patients and showed that patients (both advanced and non-advanced) experienced a high symptom burden, poor quality of life (QoL), and reduced work status and productivity[2]; however, the burden of SM in Europe from a patient perspective is not well characterized
- The Perceptions Realities and Insights on Systemic Mastocytosis (PRISM) survey study sought to understand the experiences of patients with SM as well as gain perspectives from healthcare providers (HCPs) treating SM in Europe
- Here, we describe the self-reported impact of SM on patients from the PRISM study

Methods

- PRISM was designed by an international steering committee composed of SM clinical experts and patient advocates

Patient Survey

- 119 item questionnaire
- SM diagnostic journey
- ISM-SAF (symptom assessment), SF-12 (QoL), EQ-5D (global health assessment), and WPAI (work/activity impairment measure)
- Use of medications and healthcare services
- Satisfaction with treatments and physician communication

- Ethics committees for the participating countries (Austria, France, Germany, Italy, Spain, Switzerland, and the UK) vetted and approved the study materials
- From November 2022 through August 2023, patients ≥18 years of age with an SM diagnosis and HCPs who self-reported management of patients with SM were eligible to participate
- Study participants provided consent, enrolled in the study, and completed the relevant survey
- Descriptive statistics were generated

Results

- A total of 540 patient respondents from Italy, France, the UK, Germany, Austria, Spain, and Switzerland were included; respondents were primarily female (59.6%) with an average age of 44.1 years, and were diagnosed with SM over 7 years ago (**Table 1**)
- The largest self-reported SM subtype was indolent SM (44%), and 15% were unsure of their SM subtype (**Figure 1**)
- Overall, mean total symptom score (TSS; 46.8) indicated high patient symptom burden, with patients reporting moderate (TSS ≥28) to severe (TSS ≥42) symptoms (**Figure 2**)
- The most bothersome symptoms reported by patients with SM were skin- and gastrointestinal-related, with a large subset of patients reporting anaphylaxis symptoms (**Figure 3**)
- Patients with SM reported substantially impaired physical and mental functioning (**Figure 4;** mean SF-12 values as reported in the literature for healthy individuals and lung cancer patients are indicated as benchmarks)
- There was high patient-reported current and lifetime use of symptom-directed medications (**Figure 5**)
- Overall, 51% of patients reported experiencing anaphylactic episodes during the prior year. Of the patients experiencing anaphylaxis, 27% of patients reported experiencing 5 or more episodes
- Approximately 59% of patients reported that SM had meaningful impact on their ability to work, with 34% reducing hours and 18% going on medical disability (**Figure 6**)

References

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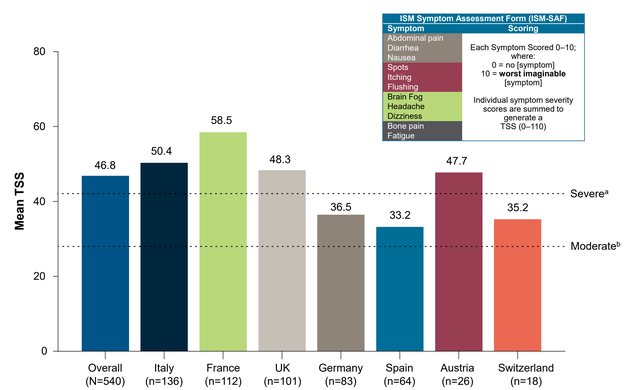
Table 1. Patient demographics

	All patients (n=540)	Italy (n=136)	France (n=112)	UK (n=101)	Germany (n=83)	Spain (n=64)	Austria (n=26)	Switzerland (n=18)
Age, years, mean (SD)	44.1 (13.8)	42.3 (12.0)	35.9 (9.2)	43.8 (16.0)	56.7 (10.5)	49.5 (11.1)	32.2 (10.0)	50.5 (10.2)
Sex, female, n (%)	322 (59.6)	68 (50.0)	39 (34.8)	78 (77.2)	57 (68.7)	52 (81.3)	13 (50.0)	15 (83.3)
Time since SM diagnosis, months, mean (SD)	91.6 (105.1)	56.9 (70.9)	55.3 (94.9)	73.7 (103.9)	196.6 (103.6)	137.1 (94.3)	22.7 (24.1)	133.6 (107.7)

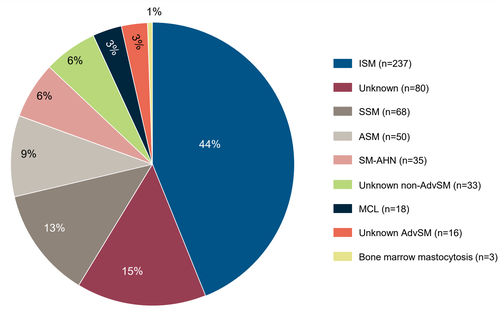
Data presented as n (%).
SD, standard deviation; SM, systemic mastocytosis; UK, United Kingdom.

Figure 2. Patients reported moderate to severe symptoms



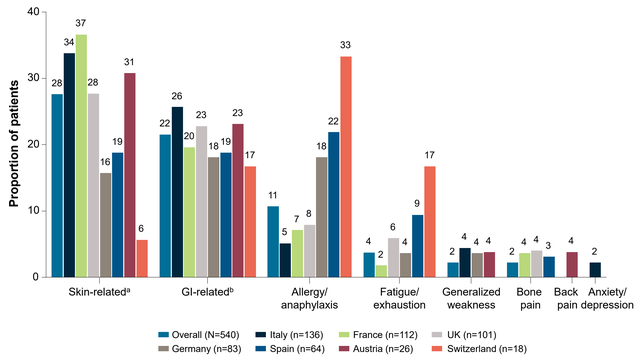
[a]TSS ≥42 indicates severe symptoms.[3]
[b]TSS ≥28 indicates moderate to severe symptom.[3]
SM, systemic mastocytosis; TSS, total symptom score; UK, United Kingdom.

Figure 1. SM subtypes overall



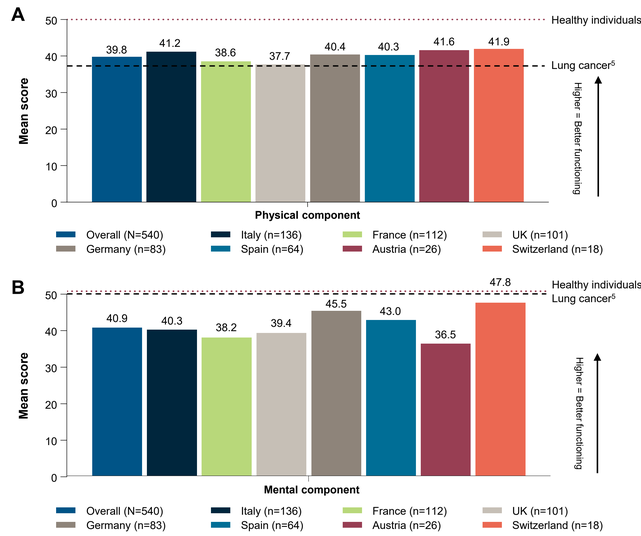
AdvSM, advanced SM; ASM, aggressive SM; ISM, indolent SM; MCL, mast cell leukemia; SM-AHN, SM with associated hematologic neoplasm; SM, systemic mastocytosis; SSM, smoldering SM.

Figure 3. The most bothersome symptoms of SM were related to skin, GI, and allergy/anaphylaxis



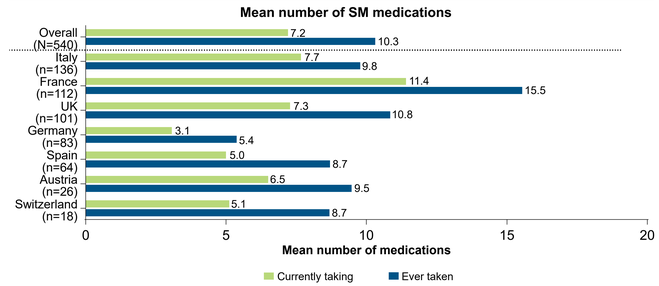
[a]Skin-related symptoms include hives, itching, spots on your skin, and flushing. [b]GI-related includes stomach bloating, nausea/vomiting, heartburn, gastroesophageal reflux, change in bowel movements, abdominal/stomach pain, and stomach cramps.
GI, gastrointestinal; SM, systemic mastocytosis; UK, United Kingdom.

Figure 4. Patients with SM reported substantially impaired physical (A) and mental (B) functioning



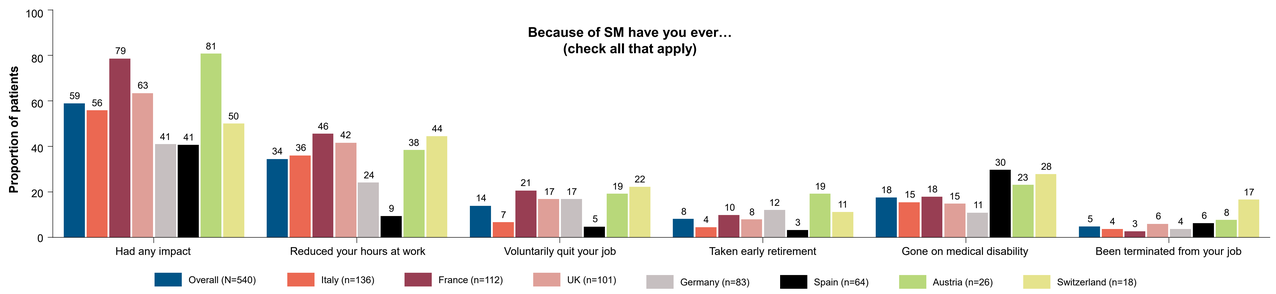
SF-12, 12-Item Short-Form Health Survey; SM, systemic mastocytosis; UK, United Kingdom.

Figure 5. High patient-reported current and lifetime use of symptom-directed medications



SM, systemic mastocytosis; United Kingdom.

Figure 6. Patients reported that SM impacted their ability to work



SM, systemic mastocytosis; UK, United Kingdom.

Conclusions

- PRISM is the largest survey to evaluate patient experiences with SM
- These data show that patients with SM report moderate to severe disease-related symptoms and impaired physical and mental functioning and frequent anaphylaxis despite receipt of multiple symptom-targeted medications
- These data highlight the disease burden and unmet needs for patients with SM

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