Patient Diagnostic Journey of Systemic Mastocytosis in Europe: Results From the PRISM 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 that can affect quality of life[1,2]
- 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
- Data from PRISM on both patient and HCP experiences of SM diagnosis are reported

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 health care services
- Satisfaction with treatments, physician communication

HCP Survey

- 103 questions
- Provider approaches to SM diagnosis, care, and management
- Perceived time to patient diagnosis
- Perception of the effect of SM of patients' lives
- Treatment goals

- 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 recruited
- Study participants provided consent, enrolled in the study, and completed the relevant survey
- Descriptive statistics were generated

Results

Patient Survey

- 540 patients participated in the PRISM patient survey; 22% self-reported as having advanced SM (AdvSM) and 44% indolent systemic mastocytosis (**Figure 1A**)
- Patients reported visiting an average of 3.8 HCPs before receiving a diagnosis (**Figure 2**)
- Overall, 52% of patients reported time from symptom onset to diagnosis more than 1 year, with approximately 1 in 5 patients reporting that diagnosis took more than 5 years (**Figure 3**)
- Across countries, the most common providers to diagnose SM were hematologists/oncologists (32%), dermatologists (23%), and allergists/immunologists (18%; **Figure 4A**)
- A variety of healthcare specialties were reported to manage patients with SM (**Figure 4B**)

HCP survey

- A total of 618 HCPs responded to the PRISM survey; the majority of whom were hematologists/oncologists (n=167) and general practitioners (n=178; **Figure 1B**)
- HCP survey respondents from multiple specialties reported managing advanced and non-advanced SM patients in their clinical practices (**Figure 5**)
- Among all HCP responders, the perceived time from symptom onset to diagnosis of SM for patients was 15.5 months and ranged by specialty from approximately 10 to 20 months, with additional variation across countries (**Figure 6**)
- Overall, HCPs reported that their primary treatment goals were improving survival and quality of life (**Figure 7**)

References

1. Pardanani A. *Am J Hematol*. 2019;94(3):363-77.
2. Mesa RA. *Cancer*. 2022;128(20):3700-8.

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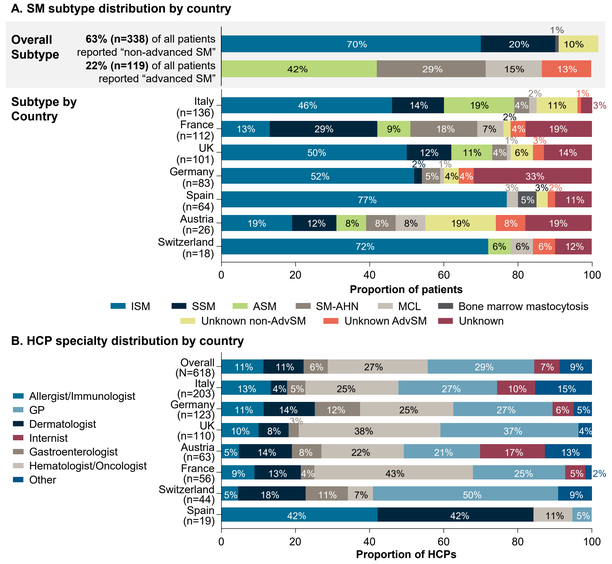
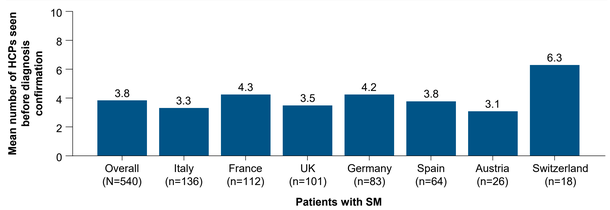
Results

Figure 1. Demographics: Patients with a variety of SM subtypes (A) and HCPs from diverse specialties (B) responded to the PRISM surveys



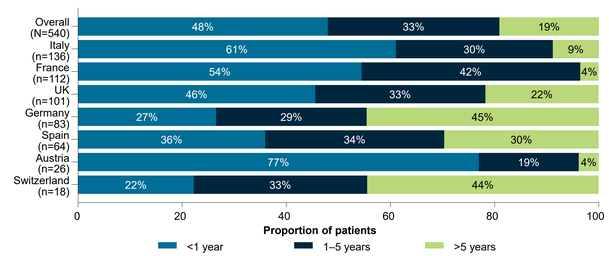
AdvSM, advanced SM; ASM, aggressive SM; GP, general practitioner; HCP, healthcare provider; ISM, indolent SM; MCL, mast cell leukemia; PRISM, Perceptions Realities and Insights on Systemic Mastocytosis; SM, systemic mastocytosis; SM-AHN, SM with associated hematologic neoplasm; SSM, smoldering SM; UK, United Kingdom.

Figure 2. Mean number of HCPs seen before diagnosis



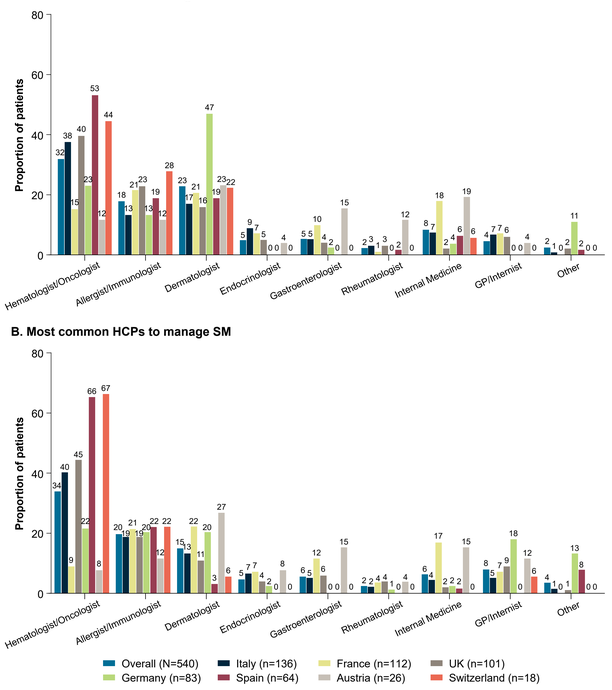
HCP, healthcare provider; SM, systemic mastocytosis; UK, United Kingdom.

Figure 3. Patient-reported time from symptom onset to diagnosis



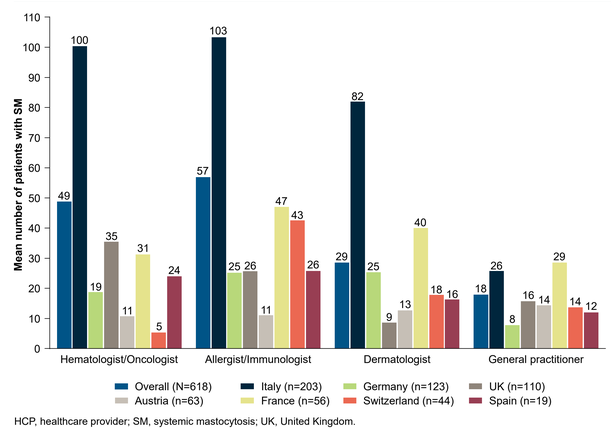
UK, United Kingdom.

Figure 4. Patient-reported diagnosis and management of SM



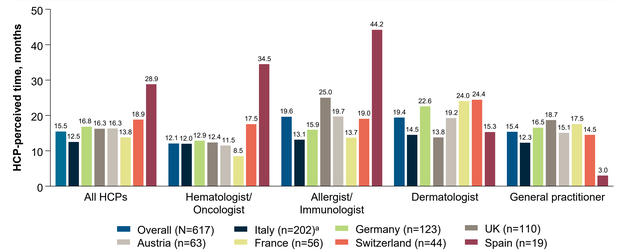
GP, general practitioner; HCP, healthcare provider; SM, systemic mastocytosis; UK, United Kingdom.

Figure 5. HCP-reported mean number of SM patients managed in clinical practice



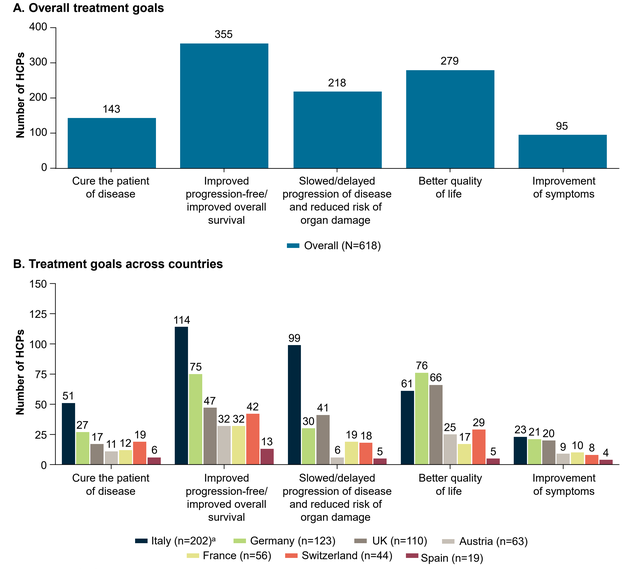
HCP, healthcare provider; SM, systemic mastocytosis; UK, United Kingdom.

Figure 6. HCP-perceived time to SM diagnosis by most common HCP type



[a]n=202 as 1 HCP from Italy did not respond.
HCP, healthcare provider; SM, systemic mastocytosis; UK, United Kingdom.

Figure 7. HCP-reported treatment goals for HCPs overall (A) and across countries (B)



[a]n=202 as 1 HCP from Italy did not respond.
HCP, healthcare provider; UK, United Kingdom.

Conclusions

- PRISM is the largest survey to evaluate patient experiences and HCP perceptions of SM
- SM patients report seeing multiple HCPs prior to their SM diagnosis, and variation in the HCP specialty who manages their care
- While half of SM patients reported time from symptom onset to SM diagnosis of more than a year and 1 in 5 patients reported a diagnostic journey greater than 5 years, HCPs perceived that time to diagnosis for patients ranged between approximately 10 to 20 months.
- Improving quality of life and prolonging survival for SM patients were the primary treatment goals reported by HCPs

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