

Healthcare Provider Perspectives on Management of European Patients With Systemic Mastocytosis

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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 symptoms^{1,2}
- SM consists of two distinct groups of variants, advanced and non-advanced; AdvSM includes aggressive SM, SM with associated hematologic neoplasm, and mast cell leukemia; non-AdvSM includes indolent SM and smoldering SM³
- Previously, a United States (US)-based survey study (Touchstone) reported insights from health care professionals (HCPs) treating patients with SM. In that survey, responses from 111 HCPs revealed that SM diagnosis in the US is challenging and often delayed, involving a variety of specialists, notably allergists/immunologists (A/Is) and hematologists/oncologists (H/Os)²
- There is limited insight regarding HCP profiles and their perceptions on the diagnosis and management of patients with SM in Europe and the UK
- The Perceptions Realities and Insights on Systemic Mastocytosis (PRISM) survey study examined experiences of patients with SM and perspectives from HCPs treating SM in Europe and the UK
- Here, we report data from PRISM on the impact of SM on patients from an HCP perspective

Methods

- PRISM was designed by an international steering committee of SM clinical experts and patient advocates
- PRISM included two independent surveys:
 - A patient survey (119 questions) on patient diagnostic experiences, symptom burden (using the ISM Symptom Assessment Form [ISM-SAF] which generates a Total Symptom Score [TSS]), and quality of life (QoL) using the 12-item Short-Form Health Survey (SF-12), the Euro-QoL Five-Dimension (EQ-5D), Euro-QoL Visual Analogue Scale (EQ-VAS), and the Work Productivity and Activity Impairment Questionnaire (WPAI)
 - An HCP survey (103 questions) on provider approaches to SM diagnosis, care, and management (reported here)
- Ethics committees for the participating countries (Italy, Germany, the UK, Austria, France, Switzerland, and Spain) 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

- A total of 618 HCP responses were collected from Italy, Germany, the UK, Austria, France, Switzerland, and Spain (Table 1)
- While a variety of HCP types reported treating patients with SM, likely due to the heterogeneous nature of the disease, the majority of respondents were H/Os (n=167) and general practitioners (n=178; Table 1)
- Across countries, A/Is and H/Os reported managing the most patients with SM (Figure 1)
- The HCP-perceived time from SM symptom onset to SM diagnosis varied by HCP type; H/Os typically reported the shortest time to diagnosis and A/Is and dermatologists the longest (Figure 2)
- At the time of diagnosis, HCPs reported patients presented with an average of 17.6 symptoms and reported the use of numerous medications, with antihistamines prescribed to more than two-thirds of patients⁴
- Most HCPs perceived that patients experience a significant impact of SM on their QoL and employment opportunities, with 65% reporting SM affected patients' lives "quite a bit" or "a great deal," and 58% reporting patients lost employment opportunities (Figure 3)
- Overall, HCPs reported their primary treatment goals for SM were improving QoL and survival (Figure 4), but almost half of HCPs (43%) reported feeling neutral or dissatisfied with available treatment options for patients⁴

References

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Disclosures

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Table 1. HCP specialty and office setting

| | All (N=618) | Italy (n=203) | Germany (n=123) | UK (n=110) | Austria (n=63) | France (n=56) | Switzerland (n=44) | Spain (n=19) |
|------------------------------|-------------|---------------|-----------------|------------|----------------|---------------|--------------------|--------------|
| Specialty, n (%) | | | | | | | | |
| Allergist/Immunologist | 70 (11) | 27 (13) | 14 (11) | 11 (10) | 3 (5) | 5 (9) | 2 (5) | 8 (42) |
| Dermatologist | 67 (11) | 9 (4) | 17 (14) | 9 (8) | 9 (14) | 7 (13) | 8 (18) | 8 (42) |
| Gastroenterologist | 40 (6) | 10 (5) | 15 (12) | 3 (3) | 5 (8) | 2 (4) | 5 (11) | 0 |
| Hematologist | 65 (11) | 17 (8) | 2 (2) | 27 (25) | 2 (3) | 12 (21) | 3 (7) | 2 (11) |
| Hematologist/Oncologist | 102 (17) | 34 (17) | 29 (24) | 15 (14) | 12 (19) | 12 (21) | 0 | 0 |
| General practitioner | 178 (29) | 54 (27) | 33 (27) | 41 (37) | 13 (21) | 14 (25) | 22 (50) | 1 (5) |
| Internist | 42 (7) | 21 (10) | 7 (6) | 0 | 11 (17) | 3 (5) | 0 | 0 |
| Other | 54 (9) | 31 (15) | 6 (5) | 4 (4) | 8 (13) | 1 (2) | 4 (9) | 0 |
| Office Setting, n (%) | | | | | | | | |
| Academic Hospital | 275 (44) | 100 (49) | 39 (32) | 56 (51) | 31 (49) | 23 (41) | 10 (23) | 16 (84) |
| Community Hospital | 130 (21) | 62 (31) | 10 (8) | 27 (25) | 11 (17) | 16 (29) | 1 (2) | 3 (16) |
| Solo Private Practice | 181 (29) | 36 (18) | 65 (53) | 18 (16) | 20 (32) | 15 (27) | 27 (61) | 0 |
| Other | 32 (5) | 5 (2) | 9 (7) | 9 (8) | 1 (2) | 2 (4) | 6 (14) | 0 |

HCP, healthcare provider.

Figure 1. HCP-reported mean number of patients with SM seen by specialty across countries

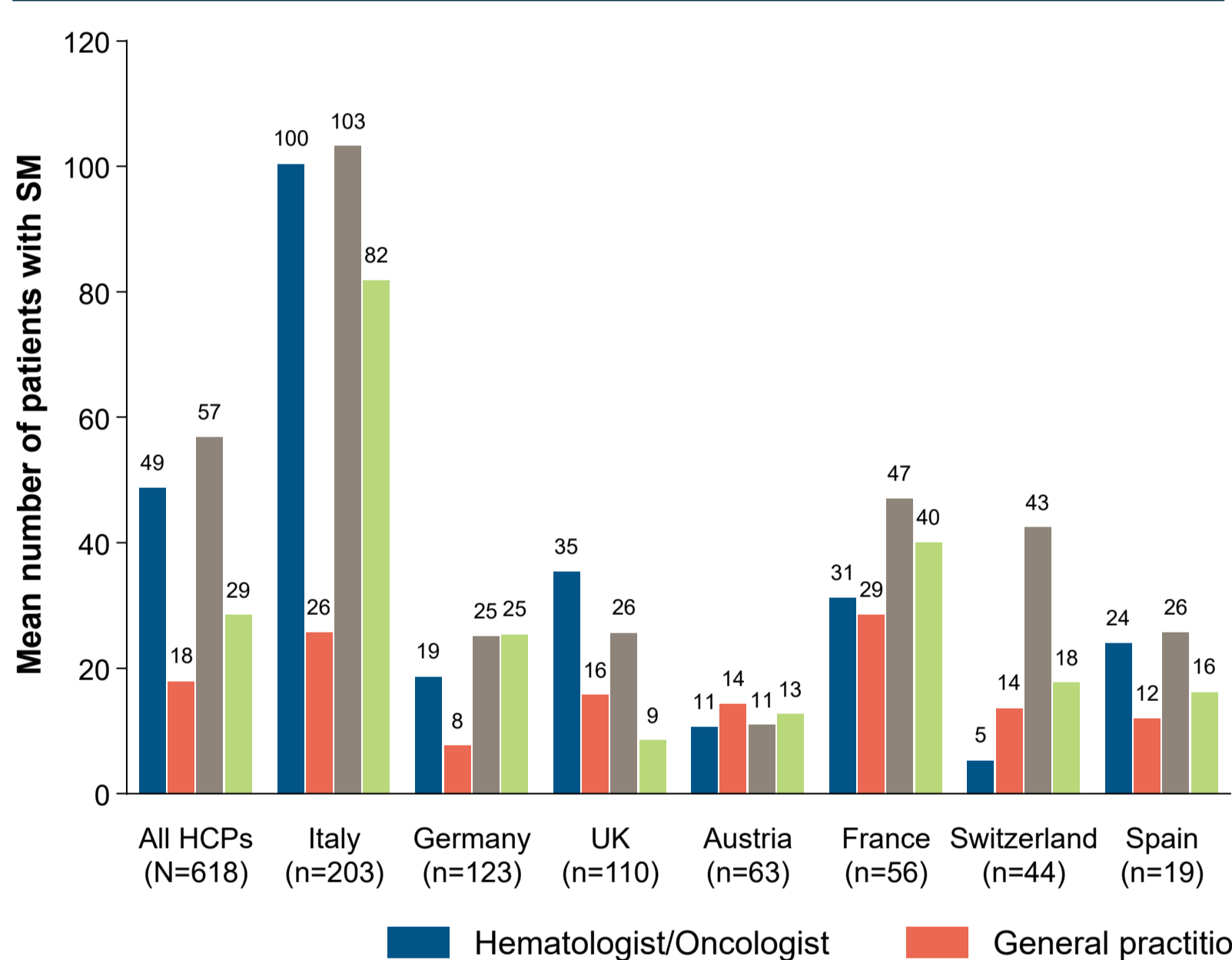
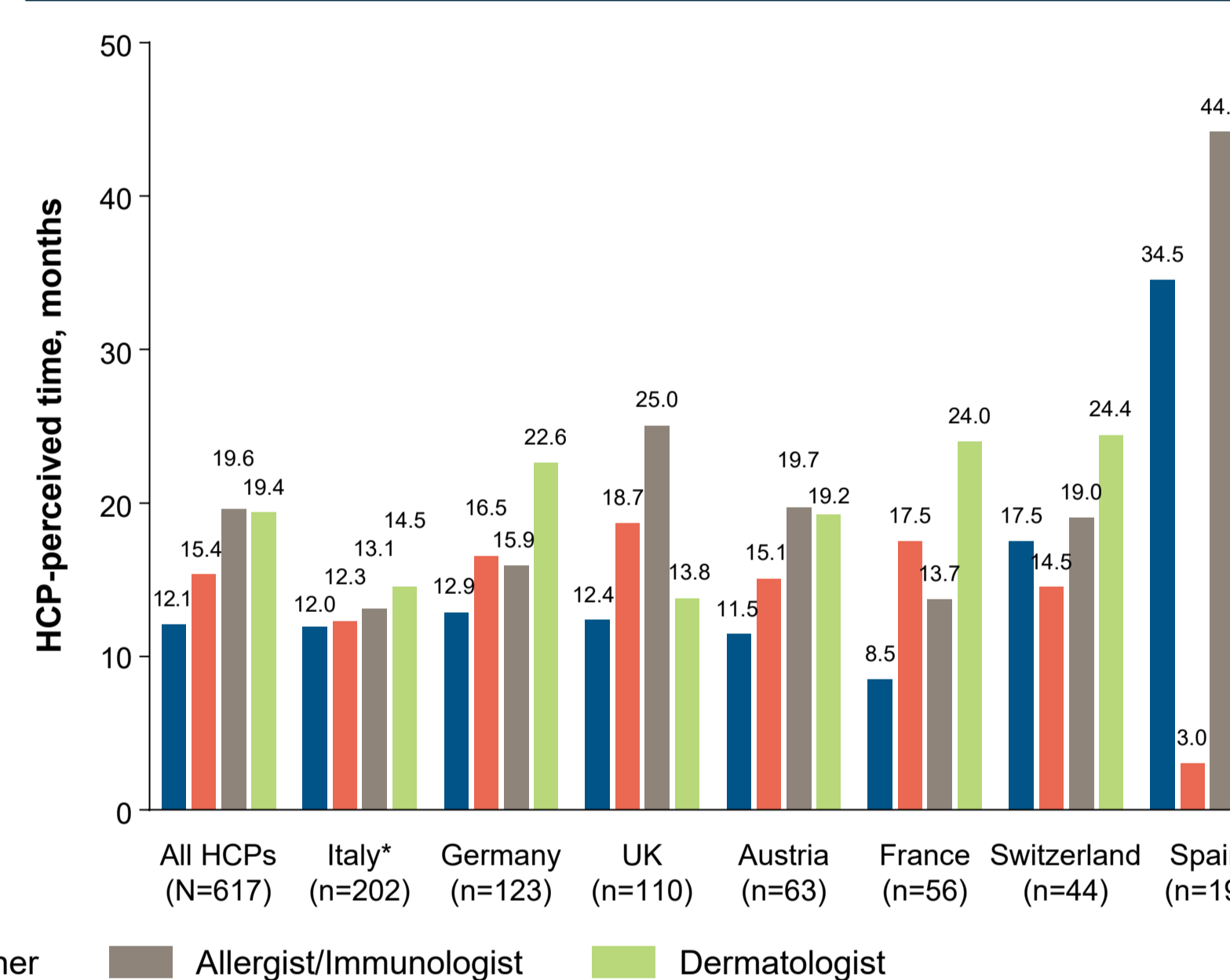
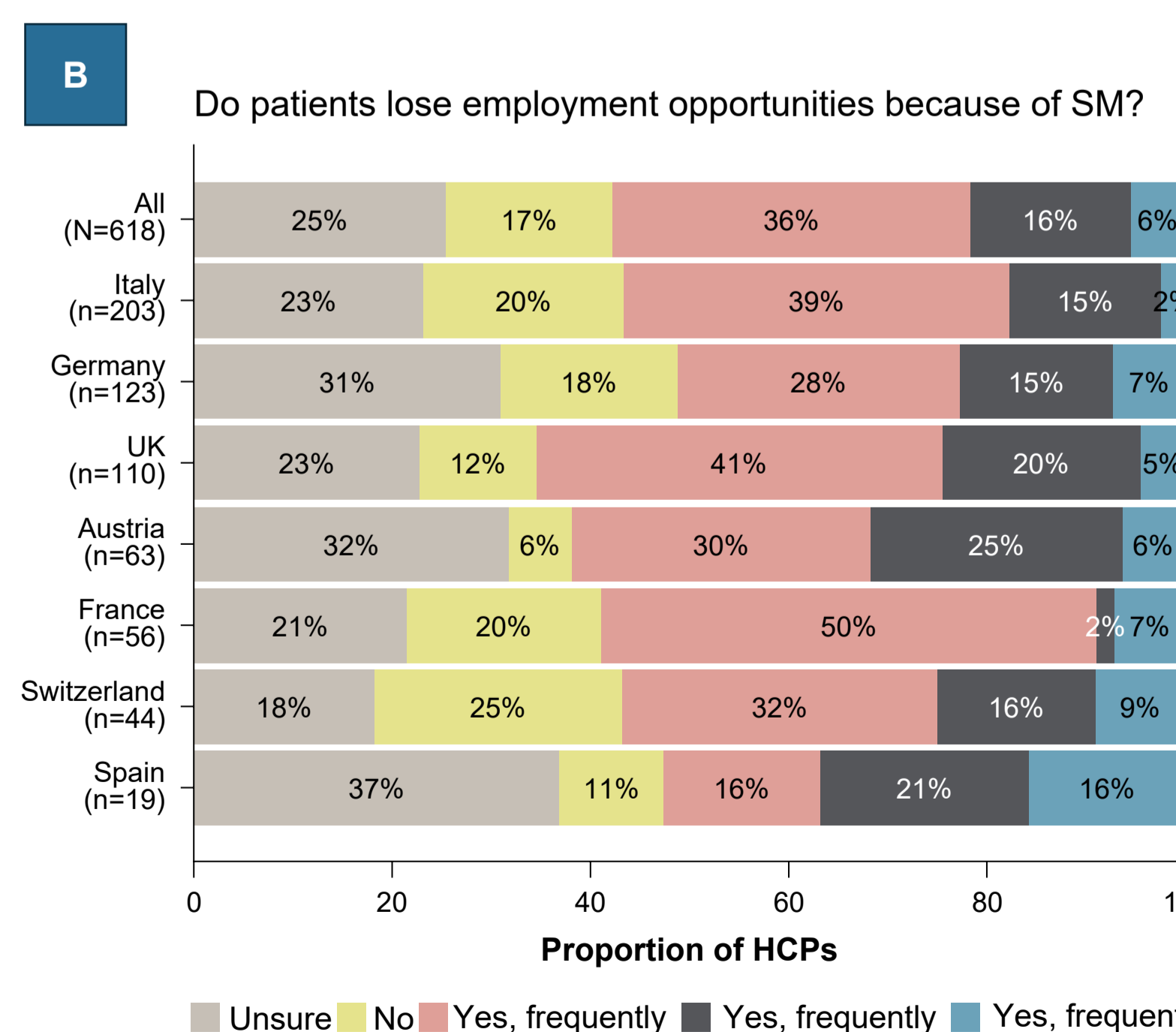
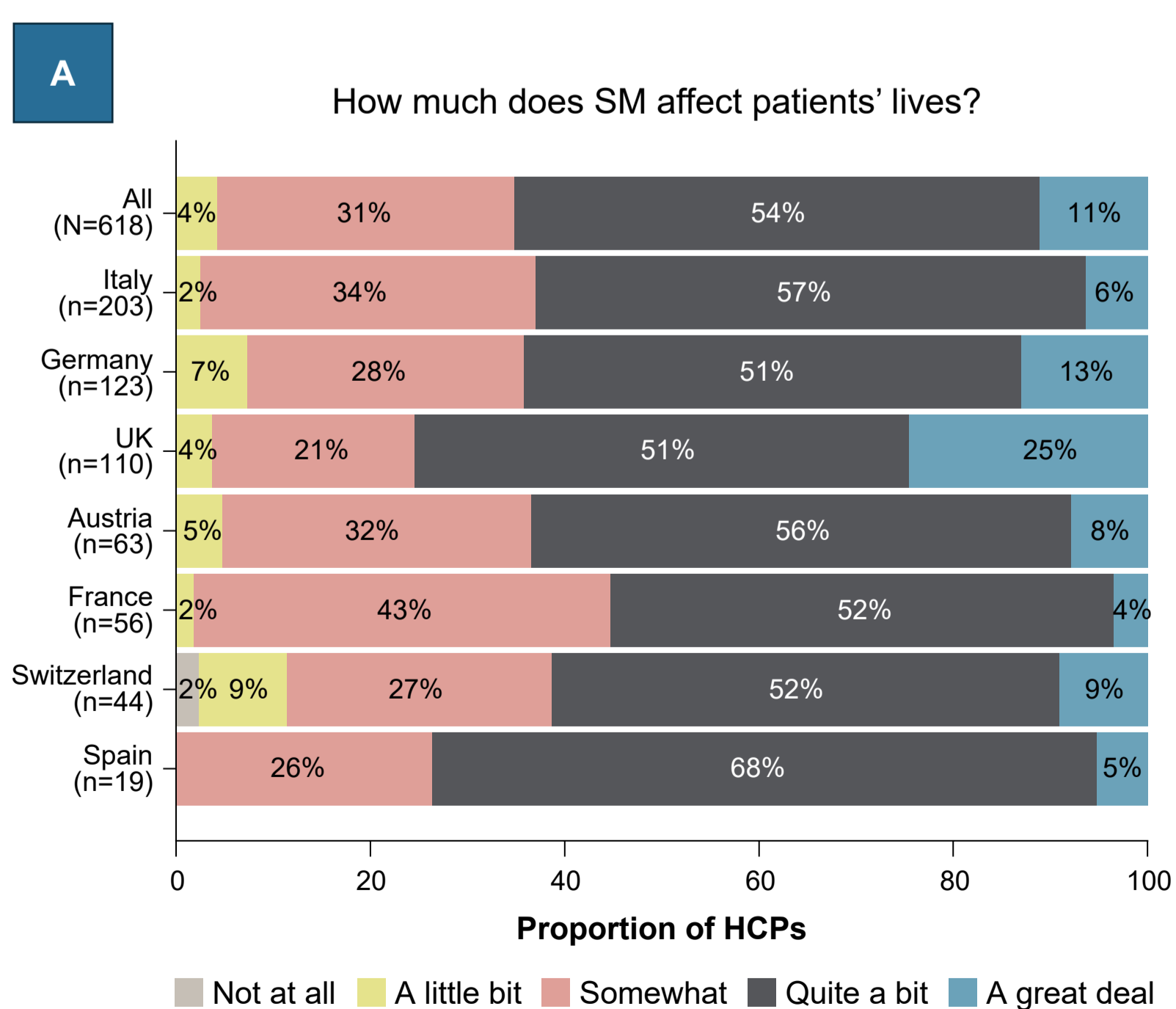


Figure 2. HCP-perceived mean time to SM diagnosis by specialty across countries



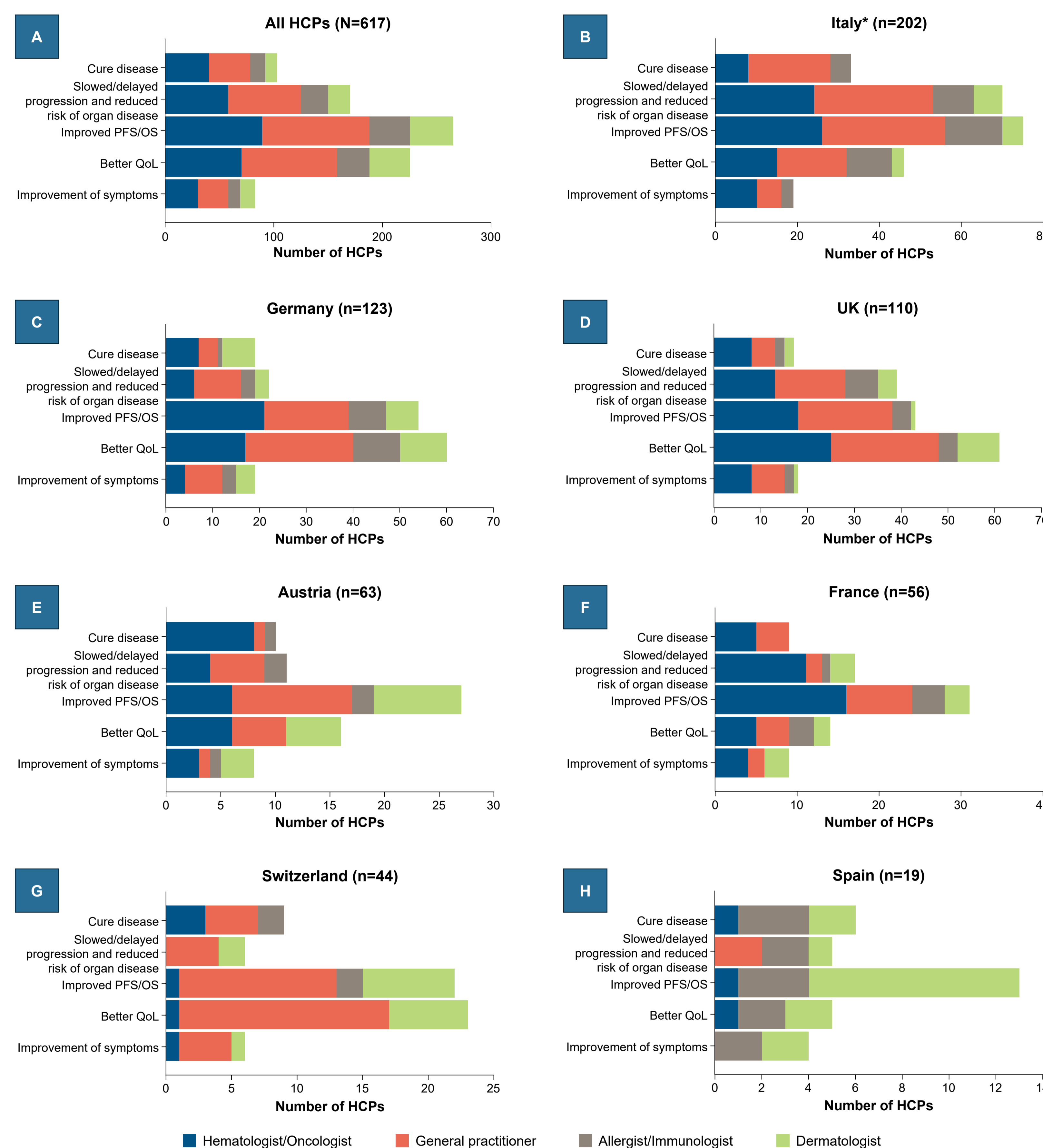
*n=202 as one HCP from Italy did not respond. HCP, healthcare provider; SM, systemic mastocytosis.

Figure 3. HCP perceptions on the effect of SM on patients' lives (A) and lost employment opportunities (B)



HCP, healthcare provider; SM, systemic mastocytosis.

Figure 4. HCP-reported treatment goals for patients with SM by HCP type for all HCPs (A) and HCPs from Italy (B), Germany (C), the UK (D), Austria (E), France (F), Switzerland (G), and Spain (H)



*n=202 as one HCP from Italy did not respond. HCP, healthcare provider; OS, overall survival; PFS, progression-free survival; QoL, quality of life; SM, systemic mastocytosis.

Conclusions

- While H/Os and A/Is reported managing the most patients with SM, HCPs from a variety of healthcare specialties reported being involved in the diagnosis and management of SM, likely due to the heterogeneous presentation of the disease
- HCP perception of time to diagnosis varied by specialty and varied substantially from previously reported patient estimates of time to diagnosis of approximately 6 years²
- HCPs perceived SM to have a considerable negative impact on patients' QoL and ability to work
- Improving QoL and survival of patients with SM were the most frequently reported goals for treatment