

The Burden of Systemic Mastocytosis in the EU: Evidence From the PRISM Patient Survey

Ruben Mesa¹, Massimo Triggiani², Jessica Hobart³, Teresa Green⁴, Dakota Powell⁴, Coralie Gressier-Sayag⁴, Wesley Baisley⁵, Betsy J. Lahue⁵, Franziska Rueff⁶, Waltraud Schinhofen⁷, Nicole Hegmann⁸, Cristina Bulai Livideanu⁹, Amélie Beaux¹⁰, Patrizia Marcis¹⁰, Iván Alvarez-Twose¹¹, Eugenia Ribada¹², Deepti Radia¹³, Celeste C. Finnerty¹⁴

¹Atrium Health Wake Forest Baptist Comprehensive Cancer Center, Winston-Salem, NC, USA; ²Università degli Studi di Salerno, Division of Allergy and Clinical Immunology, Fisciano, Italy; ³The UK Mastocytosis Support Group, London, United Kingdom; ⁴Blueprint Medicines, Cambridge, MA, USA; ⁵Alkermi LLC, Manchester Center, VT, USA; ⁶Department of Dermatology and Allergy, LMU University Hospital, Munich, Germany; ⁷Association Mastozytose e.V., Toniworst, Germany; ⁸Patient Advocacy Group (Selbsthilfe Mastozytose e.V.), Odenthal, Germany; ⁹CHU de Toulouse – Hôpital Larrey, Toulouse, France; ¹⁰Patient Advocacy Group (ASSOMAST), Paris, France; ¹¹Hospital Virgen Del Valle, Toledo, Spain; ¹²Patient Advocacy Group (AEDM), Madrid, Spain; ¹³Guy's and St. Thomas' NHS Foundation Trust, London, United Kingdom; ¹⁴The Mast Cell Disease Society, Sterling, MA, USA

Introduction

- Systemic mastocytosis (SM), which includes both advanced and non-advanced versions of the disease, is a rare, clonal mast cell disease, primarily driven by the *KIT* D816V mutation
- The majority of SM patients have indolent SM (ISM), a subtype of the non-advanced disease, which is characterized by unpredictable, severe, and debilitating skin, gastrointestinal, and systemic symptoms^{1,2}
- Previously, a small 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, and reduced work status and productivity.² However, the burden of SM in Europe is not well characterized
- The Perceptions Realities and Insights on Systemic Mastocytosis (PRISM) study seeks to examine the experiences of advanced and non-advanced SM patients, as well as gain perspectives from healthcare providers (HCPs) treating SM in 7 countries in Europe
- Here, we report interim data from PRISM on the impact of ISM on patients from the United Kingdom (UK), Germany, and Austria

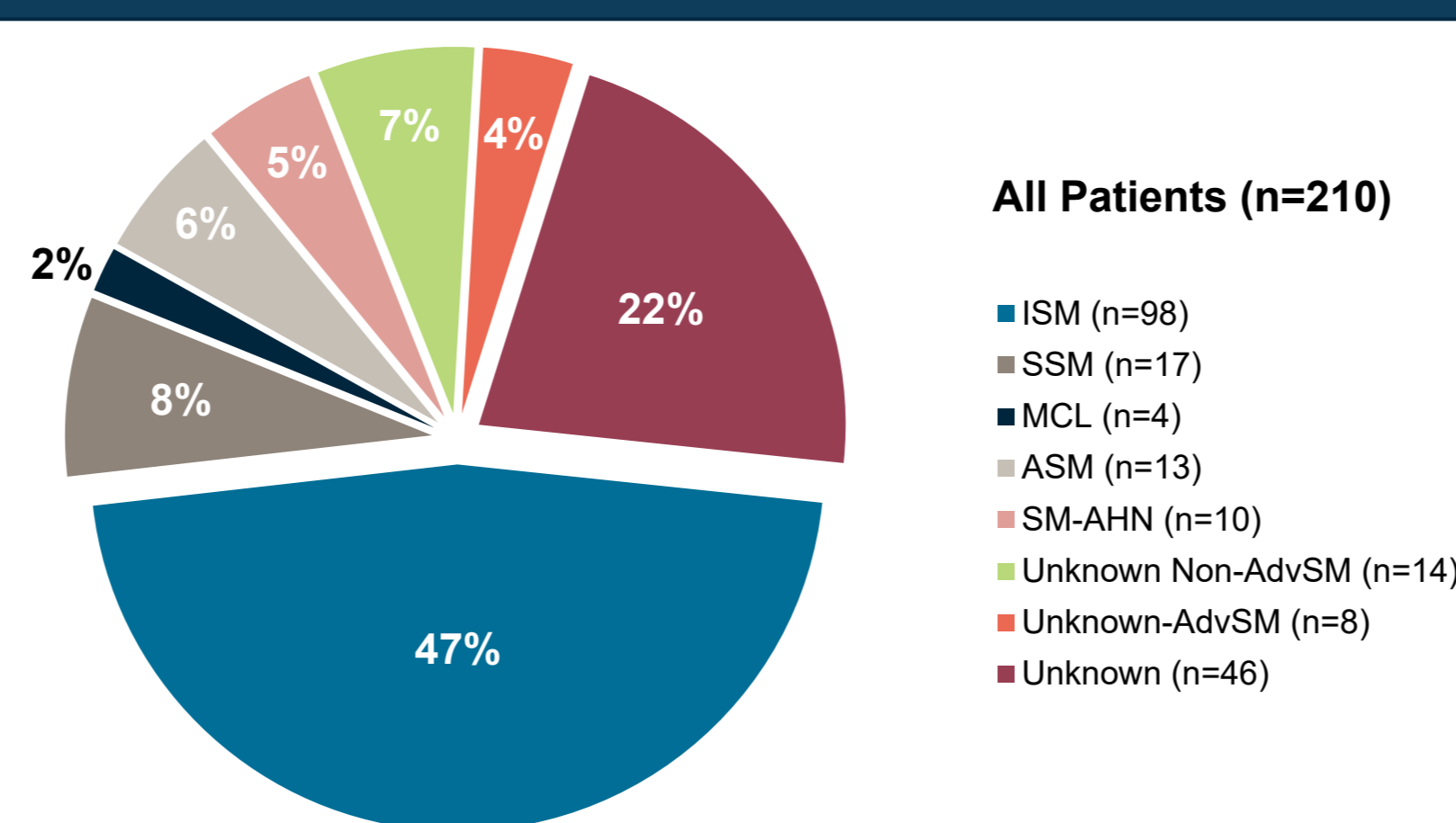
Methods

- PRISM was designed by an international steering committee of SM clinical experts and patient advocates
- The study was composed of 2 independent surveys:
 - A Patient Survey (119 questions) on patient diagnostic experiences, symptom burden (via a validated ISM symptom assessment form [ISM-SAF] generating a Total Symptom Score [TSS]), quality of life (QoL) via the 12-item short form survey (SF-12), Euro-QoL five-dimension (EQ-5D), Euro-QoL visual analogue scale (EQ-VAS), and work productivity and activity impairment questionnaire (WPAI)
 - An HCP Survey (103 questions) on provider approaches to SM diagnosis, care, and management
- Study materials were vetted by independent scientific review and approved by designated ethics committees for the 7 participating countries: France, Switzerland, Spain, Italy, Austria, Germany, and the UK
- From November 2022 through August 2023, patients ≥18 years of age with an SM diagnosis and HCPs who treated patients with SM were recruited via social media, advocacy groups, and word of mouth in the study countries
- Recruited participants provided consent, were enrolled in the study, and completed the relevant survey
- This analysis presents interim patient results available as of March 2023 for 3 countries (UK, Germany, and Austria)

Results

- From a total of 210 SM patient responses from the UK, Germany, and Austria collected as part of the PRISM survey, data from 98 ISM patients are included
- The largest self-reported SM subtype was ISM (47%); about 1 in 5 patient respondents (22%) were unsure of their SM subtype (Figure 1)
- Most ISM patients were female, and the mean age of all patients was 52.7 years (Table 1)
- Mean TSS (41.2) indicated high patient symptom burden consistent with moderate-to-severe ISM (TSS >28) (Figure 2)
- Patients consistently reported reduced physical functioning and mental health due to ISM (Figure 3A and B), with SF-12 scores well below average for healthy individuals (~50).³ The most bothersome symptoms were skin- and gastrointestinal (GI)-related, and a large subset of ISM patients experienced anaphylaxis (Figure 4)
- Polypharmacy was high, with ISM patients reporting that they take an average of 5.3 (over-the-counter [OTC] + prescription [Rx]) medications to manage symptoms (Figure 5); H1 antihistamines were the most commonly used nonprescription medication (Figure 6)
- Patients reported that ISM impacted their ability to work, with a large subset having to reduce their hours (31.6%) or discontinue work entirely (12.2%) (Figure 7)

Figure 1. Patient-Reported SM Subtypes



Abbreviations: AdvSM, advanced SM; ASM, aggressive SM; ISM, indolent SM; MCL, mast cell leukemia; Non-AdvSM, non-advanced SM; SM-AHN, SM with an associated hematologic neoplasm; SSM, smoldering SM.

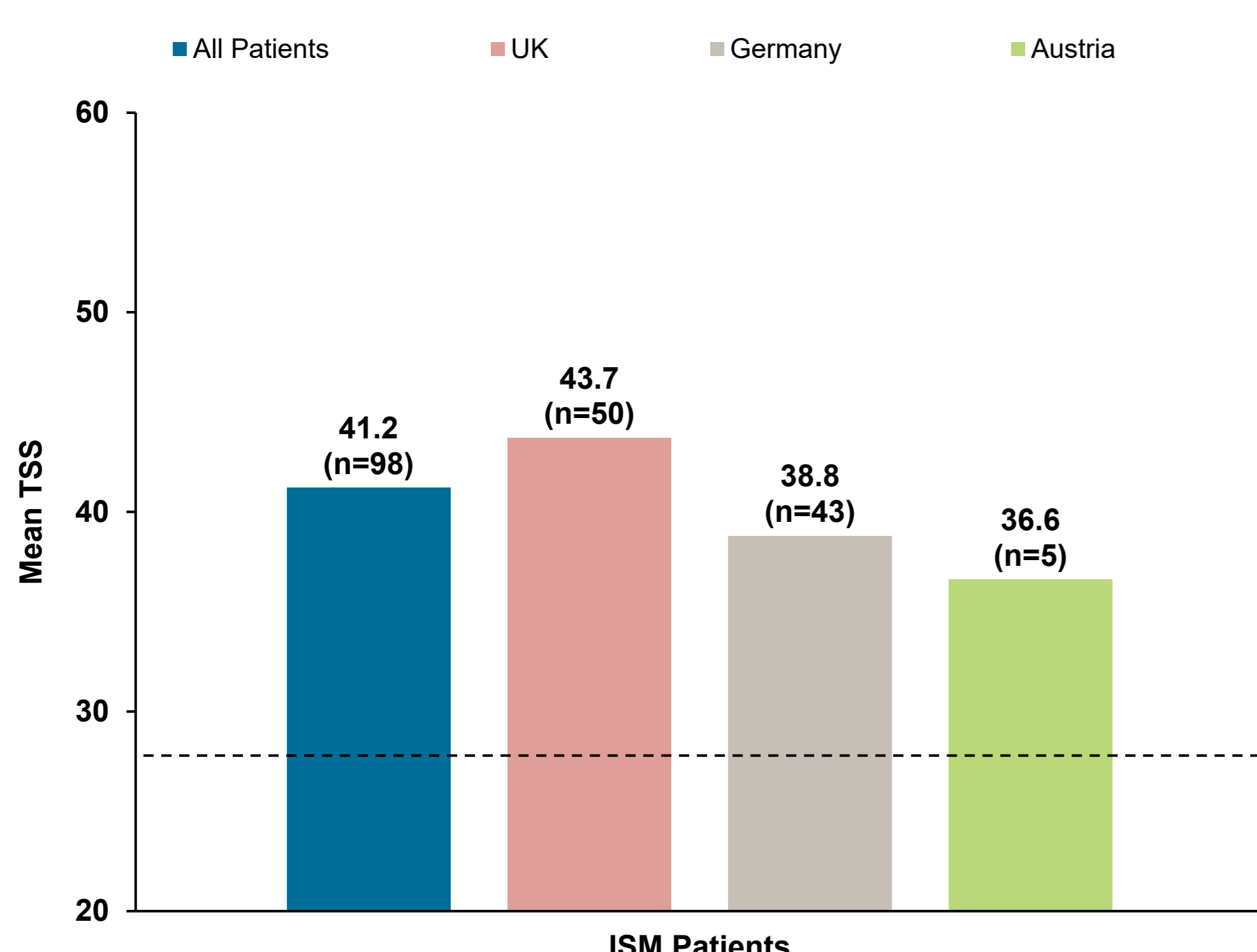
Table 1. Demographic and Clinical Characteristics of ISM Patients

	All (n=98)	UK (n=50)	Germany (n=43)	Austria (n=5)
Age, mean years (SD)	52.7 (13.3)	51.0 (14.2)	56.6 (10.7)	36.8 (8.7)
Female, n (%)	75 (76.5)	43 (86.0)	30 (69.8)	2 (40.0)
Time since SM diagnosis, mean months (SD)	128.0 (114.6)	95.8 (118.5)	170.5 (98.4)	37.6 (47.4)

Abbreviations: ISM, indolent systemic mastocytosis; SD, standard deviation; SM, systemic mastocytosis.

Results (cont)

Figure 2. Mean Total Symptom Scores (TSS*) Reported by ISM Patients Were Moderate or Severe (ISM-SAF)



*TSS≥28 indicates moderate to severe symptom profile. Median (range) TSS: All patients, 39 (0–95); UK, 39 (4–93); Germany, 34 (0–95); Austria, 42 (18–53).

Figure 3. ISM Patients Reported Reduced Physical Functioning and Mental Health (SF-12)

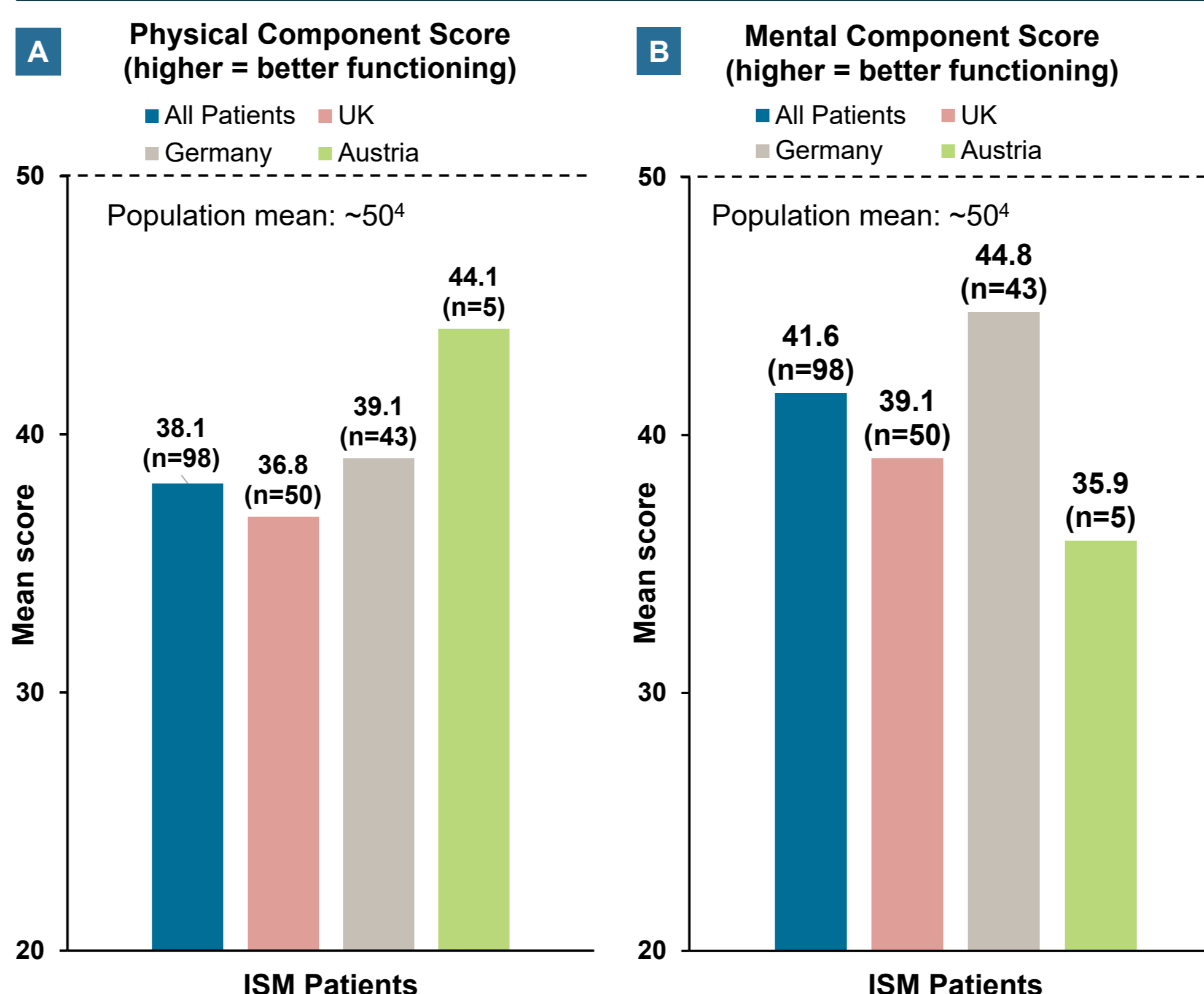


Figure 4. The Most Commonly Reported Primary Signs and Symptoms of ISM Were Skin- and GI-Related

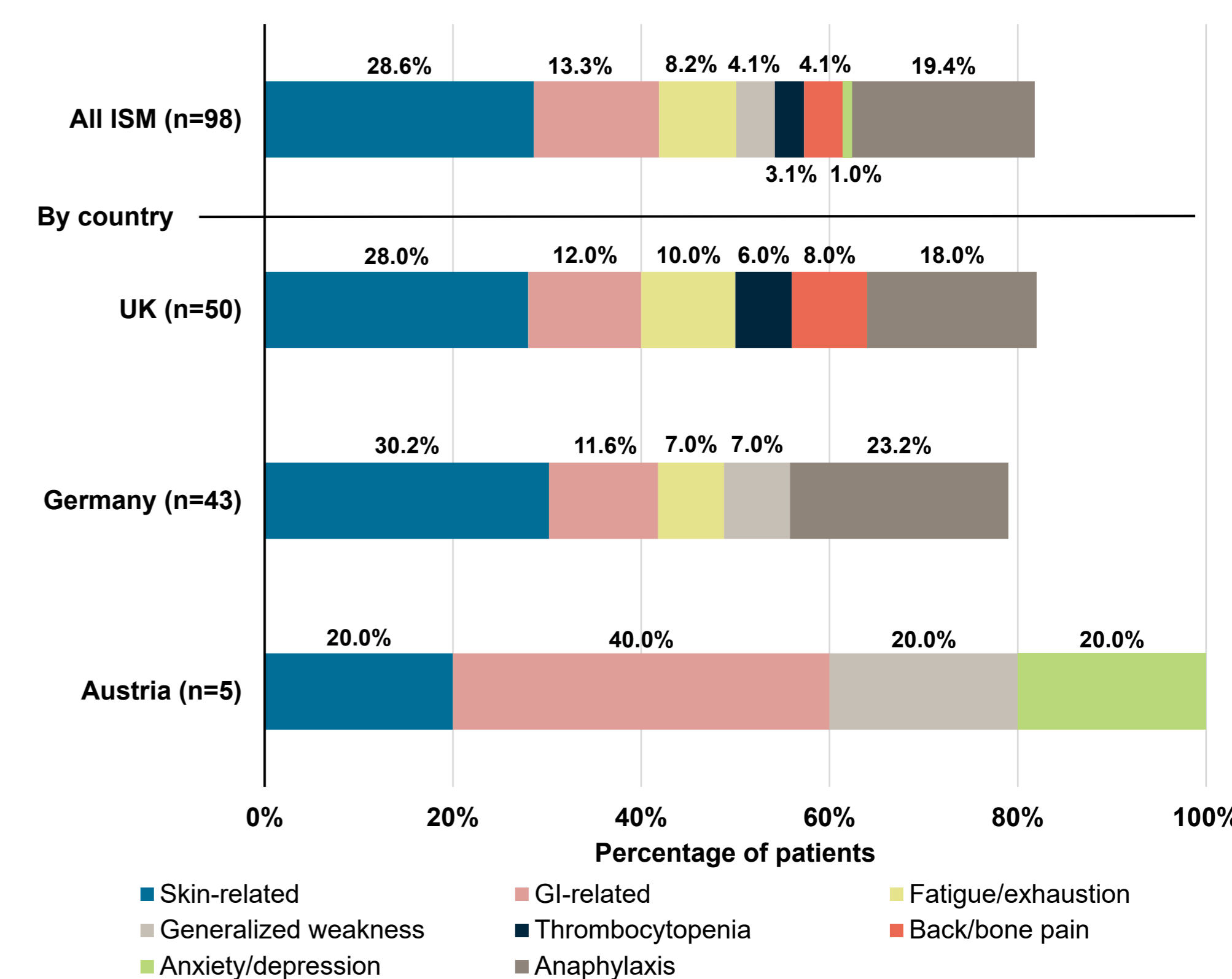
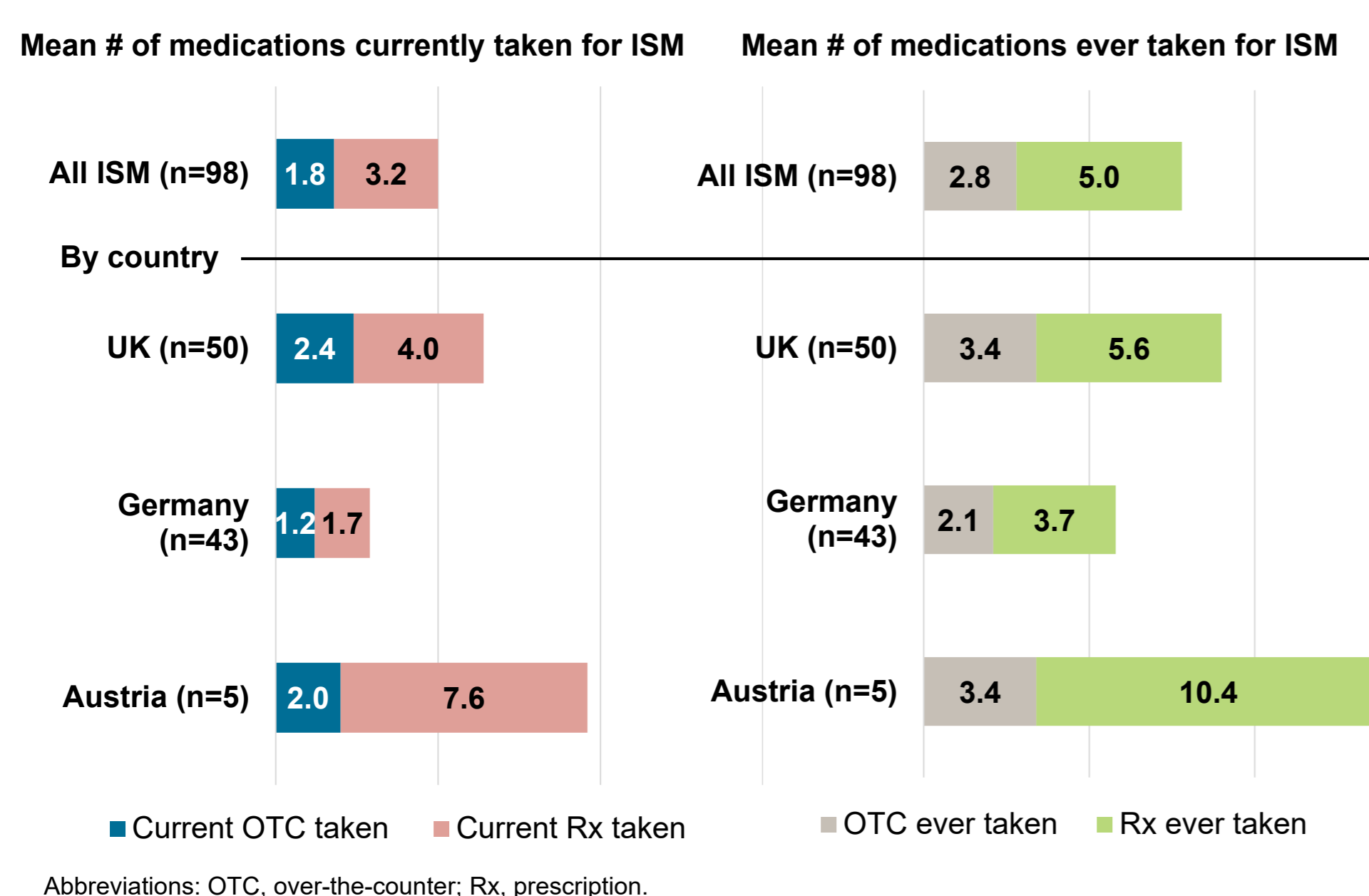


Figure 5. Polypharmacy: Patients Took Many Prescription and Nonprescription Medications to Manage Their ISM Symptoms



Abbreviations: OTC, over-the-counter; Rx, prescription.

Figure 6. Antihistamines, Proton Pump Inhibitors, and Antidiarrheals Were the Most Commonly Reported Nonprescription Medications

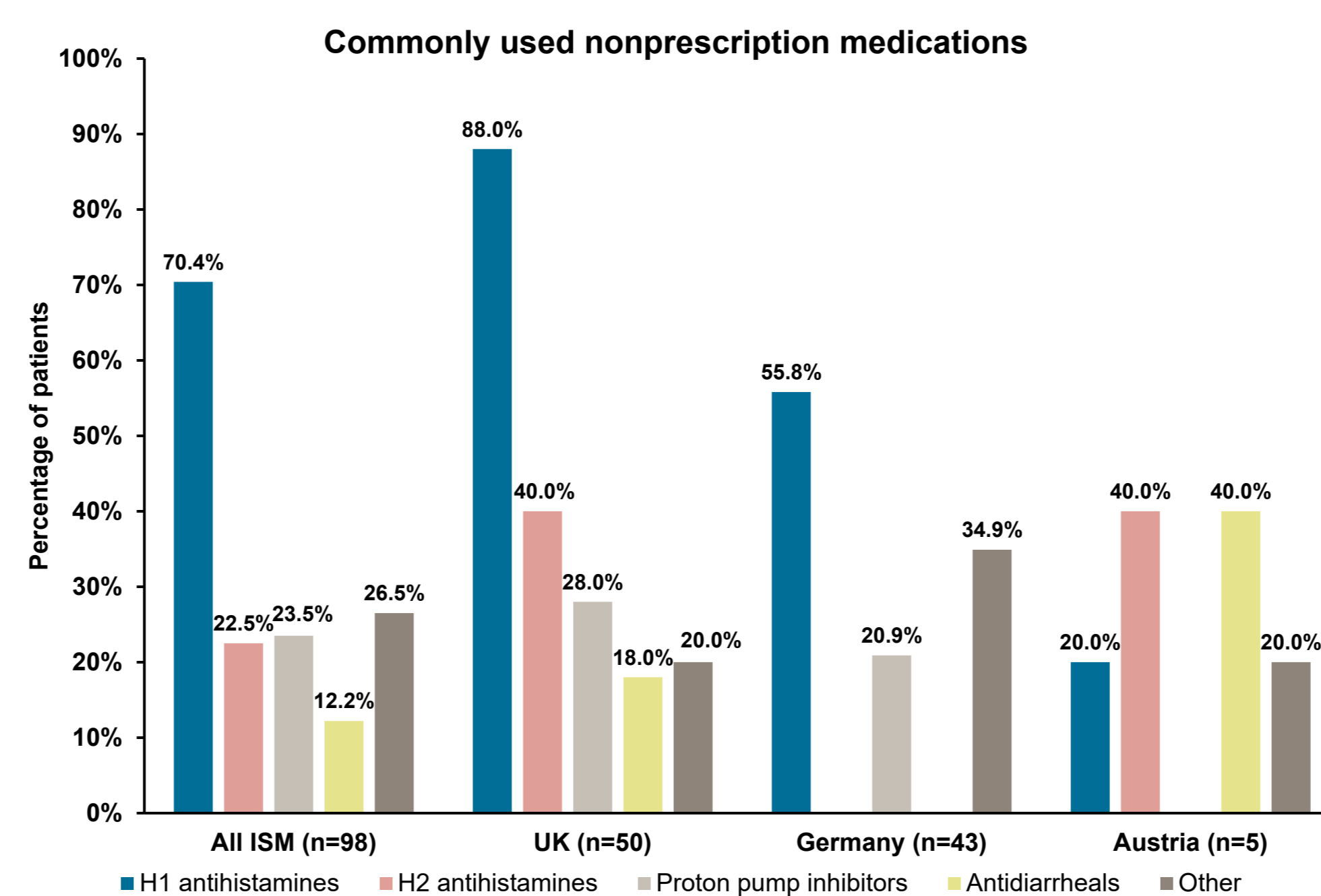
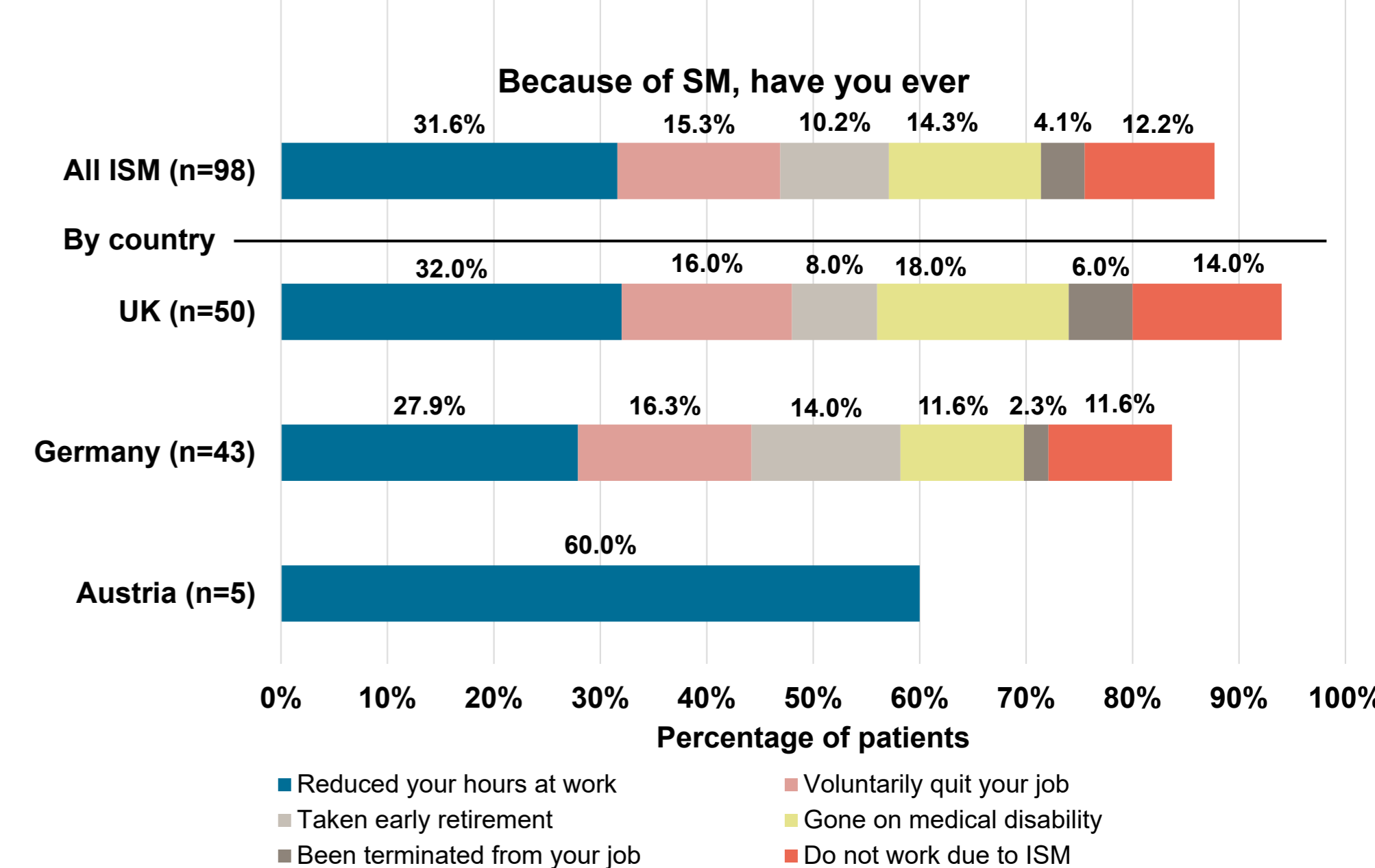


Figure 7. Patients Reported ISM Impacted Their Ability to Work



Conclusions

- Interim data from the PRISM survey demonstrate that patients with ISM experience high burden of disease, including moderate-to-severe, disease-related symptoms and poor QoL despite taking multiple over-the-counter and prescription medications
- The burden of ISM has a significant impact on patients' ability to work, causing one third of patients to reduce hours at work, 15% of patients to quit their jobs, and 12% of patients do not work because of ISM
- These patient-reported data highlight that there is a substantial burden of disease experienced by patients with ISM and demonstrate an ongoing need to advance treatment options beyond supportive care

References

- Pardanani A. *Am J Hematol* 2021;96:508-525; 2. Mesa RA, et al. *Cancer* 2022;128:3700-3708; 3. Janssen B, Szende A. *Self-Reported Population Health: An International Perspective Based on EQ-5D*: Springer 2014; 4. National Longitudinal Surveys, U.S. Residents aged 45-54, 2018.

Acknowledgments

The authors thank Alkermi LLC for their assistance in drafting this poster and the PRISM Steering Committee for their involvement. Medical writing and editorial support was provided by Heyuan Sun, PhD, of ProEd Communications, supported by Blueprint Medicines Corporation, Cambridge, MA, USA.

Disclosures

Study sponsored by Blueprint Medicines. TG, DP, and CGS are employees of Blueprint Medicines and own stock in Blueprint Medicines.