The Socio-Emotional Impact of Indolent Systemic Mastocytosis: Insights From the PRISM Survey

Poster Number 000488

Jessica Hobart,¹ Ruben Mesa,² Iván Alvarez-Twose,³ Amélie Beaux,⁴ Gabriela Coletti,⁵ Nicole Hegmann,⁶ Cristina Bulai Livideanu,ˀ Patrizia Marcis,⁶ Deepti H. Radia,⁶ Eugenia Ribada,¹⁰ Franziska Ruëff,¹¹ l Waltraud Schinhofen,¹² Dakota Powell,¹³ Teresa Green,¹³ Cristian Perez,¹³ Massimo Triggiani¹⁴

¹The UK Mastocytosis Support Group, London, UK; ²Atrium Health Wake Forest Baptist Comprehensive Cancer Center, Winston-Salem, NC, USA; ³Institute of Mastocytosis Studies of Castilla-La Mancha (CLMast), Toledo, Spain; ⁴French Association of Patients with Mast Cell Diseases (ASSOMAST), Paris, France; ⁵SELMAS mastozytose.ch, Zurich, Switzerland; ⁶Mastocytosis Self-Support Network, eV, Odenthal, Germany; ⁷French Reference Center for Mastocytosis (CEREMAST), Department of Dermatology, Toulouse University Hospital, Toulouse, France; ⁸Associazione Italiana Mastocitosi (ASIMAS) ODV, Milan, Italy; ⁹Guy's and St. Thomas' NHS Foundation Trust, London, UK; 10 Spanish Association of Maximilian University of Munich, Munich, Germany; 12 Mastozytose eV, Deutschland Self-Support Association Mastocytosis eV, Grefrath, Germany; 13 Blueprint Medicines Corporation, Cambridge, MA, USA; 14 Division of Allergy and Clinical Immunology, University of Salerno, Salerno, Italy

Introduction

- Systemic mastocytosis (SM) is a clonal mast cell disease driven by KIT D816V in ~95%
- Approximately 85% of patients diagnosed with SM have indolent systemic mastocytosis (ISM),4-6 characterized by unpredictable, severe, and debilitating skin, gastrointestinal, and systemic symptoms^{6,7}
- A substantial number of patients with ISM experience a significant reduction in their quality of life due to chronic, debilitating symptoms such as fatigue, abdominal pain, and anaphylactic reactions. These symptoms often lead to frequent healthcare visits and interventions, and significantly affect patients' daily activities and overall well-being8,9
- The Perceptions Realities and Insights on Systemic Mastocytosis (PRISM) survey sought to understand experiences of patients with SM from patient and healthcare provider perspectives¹⁰
- Previous results from the PRISM survey showed patients with ISM reported moderate to severe disease-related symptoms, frequent anaphylaxis (despite being treated with multiple symptom-targeted medications), impaired physical and mental functioning, and diseaserelated impact on their ability to work¹¹
- Here, we conducted a secondary analysis of PRISM data to further explore the socio-emotional impact of ISM on patients and its relationship to symptom severity

Methods

- The methods for conducting the PRISM survey have been reported previously¹⁰
- PRISM was administered in seven European countries (Austria, France, Germany, Italy, Spain, Switzerland, and the UK) from November 2022–August 2023
- Eligible patients were aged ≥18 years with an SM diagnosis

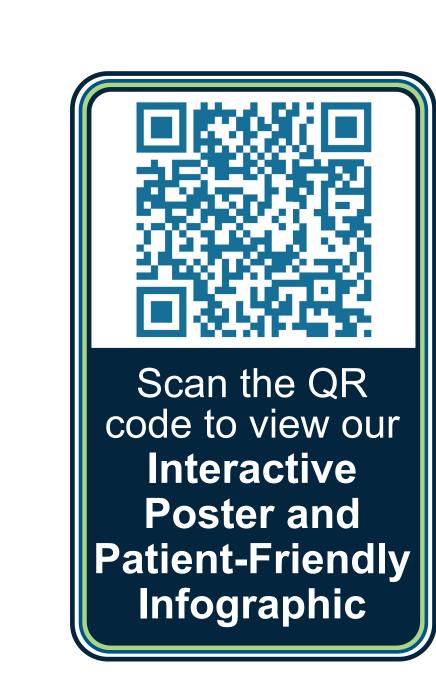
Patient survey

- 119 item questionnaire
- ISM-SAF^{a,12}
- Emotional, cognitive and mental health symptoms attributed to SM
- Impact of SM on feelings and daily life survey
- EQ-5D-5L survey, a general health measure assessing problems with daily living¹³
- SF-12 quality of life assessment¹⁴

where 0 = no [symptom] and 10 = worst imaginable [symptom]. Individual symptom severity scores are summed to generate a TSS (0–110). Patients score statements about their disease on a 1–5 scale: 1 = No impact at all, 2 = A little bit, 3 = Somewhat, 4 = Quite a bit, 5 = A great deal of impact. EQ-5D-5L. Euro-QoL five-dimension-five level: ISM-SAF. Indolent Systemic Mastocytosis Symptom Assessment Form; SF-12, 12-item Short Form survey; SM, systemic mastocytosis; TSS, total symptom score.

Acknowledgements

Medical writing support was provided by Rachel O'Meara, PhD, and Travis Taylor, BA, of Paragon. Funded by Blueprint Medicines Corporation. The sponsor reviewed and provided feedback on the poster. However, the authors had full editorial control and provided final approval of all content.



Conflicts of interest/disclosures

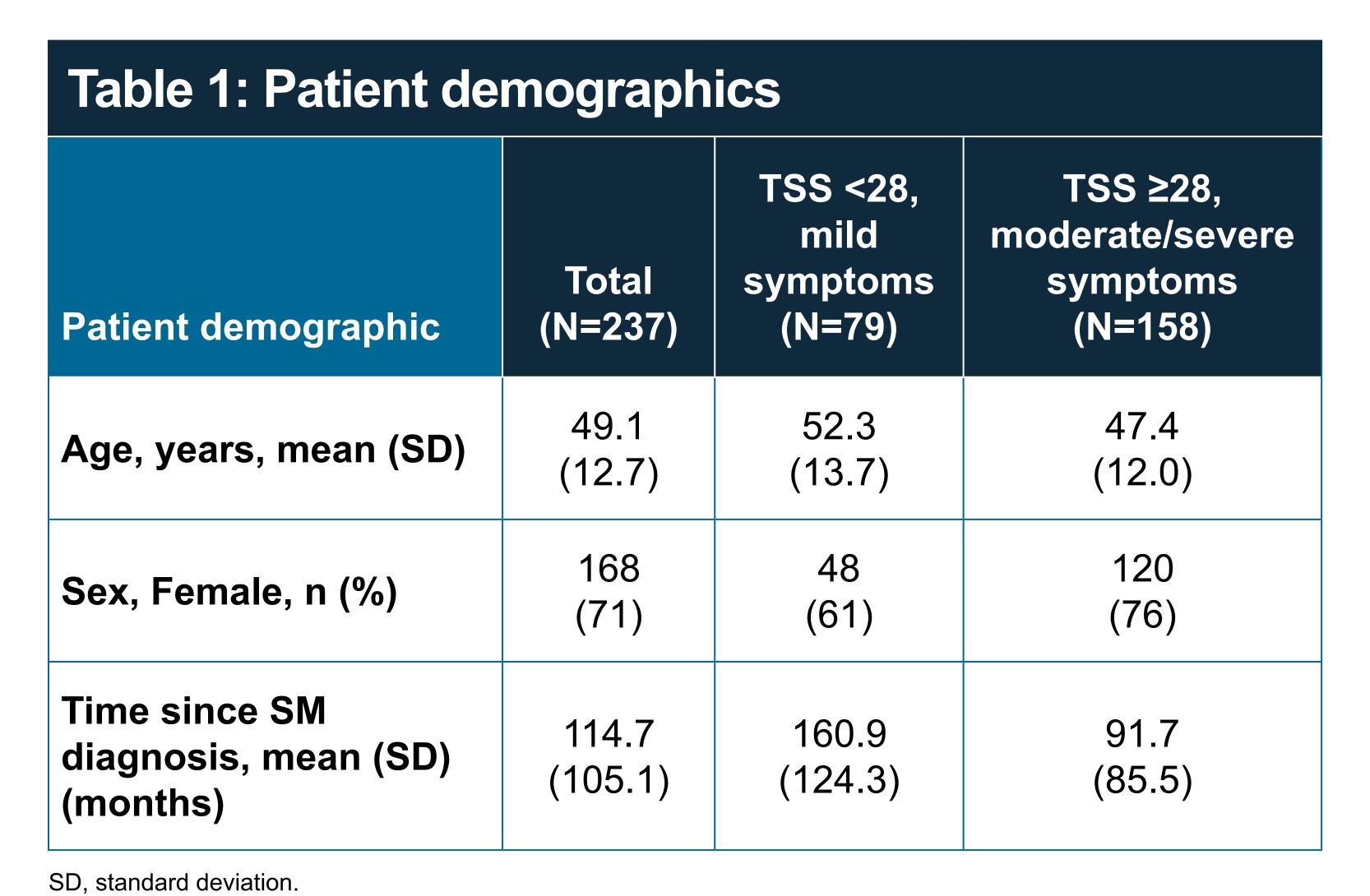
Jessica Hobart's organization received honoraria from Blueprint Medicines Corporation for participation in development of the PRISM study. Full disclosures for all authors are available upon request at medinfo@blueprintmedicines.com.

References

1. Rossignol J et al. *F1000Research*. 2019;8:1961; 2. Pardanani A. *Am J Hematol*. 2023;98:1097–1116; 3. Kristensen T et al. *Am J Hematol*. 2014;89:493–498; 4. Ungerstedt J et al. *Cancers*. 2022;14:3942; 5. Sperr WR et al. Lancet Haematol. 2019;6:e638–e649; 6. Cohen SS et al. British J Haematol. 2014;166:521–528; 7. Pardanani A. *Am J Hematol*. 2019;94:363–377; 8. Mesa RA et al. *Cancer*. 2022;128:3700–37708; 9. van Anrooji et al. *Allergy*. 2016;71:1585–593; 10. Radia DH et al. Presented at EHA 2024, P2292; 11. Triggiani M et al. Presented at EAACI 2024, 000405; 12. Taylor F et al. Orphanet J Rare Dis. 2021;16:414; 13. EuroQol group. Health Policy. 1990;16:199–208; 14. Ware J Jr et al. Med Care. 1996;34:220–223.

Results

 From a total of 540 patients who participated in the PRISM survey, results from 237 patients with ISM are reported (Table 1). Among patients with ISM, 67% reported a total symptom score (TSS) of ≥28 indicating moderate or severe symptoms, and 33% reported a TSS of <28, indicating mild symptoms



psychological, and fatigue problems due to ISM

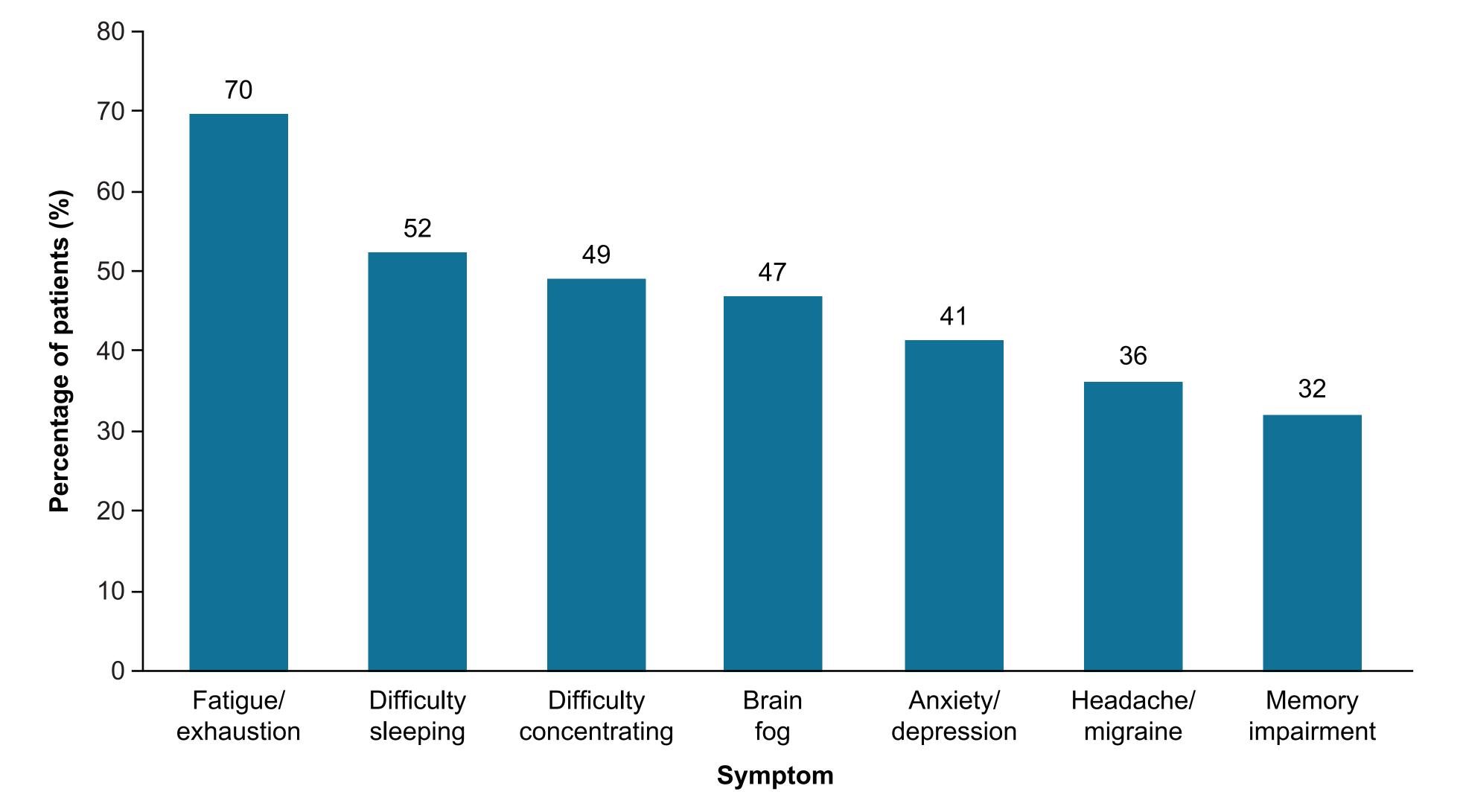
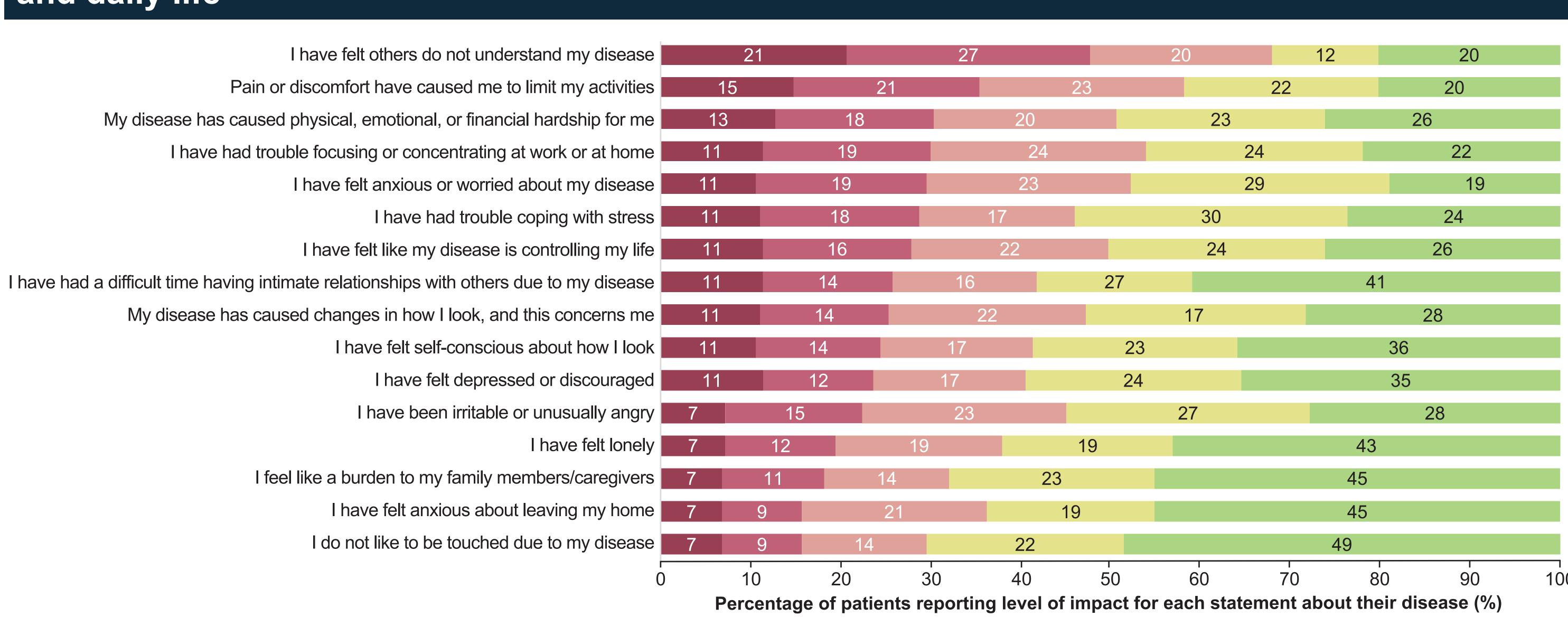
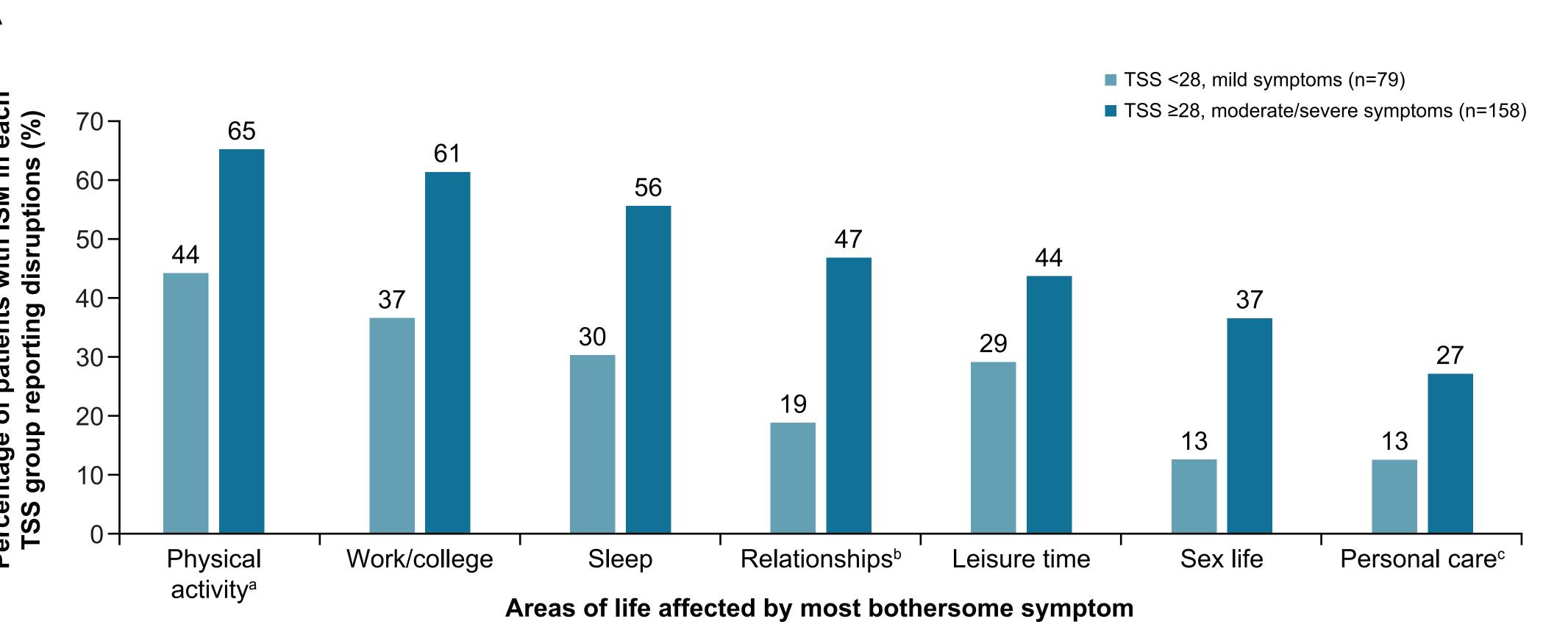


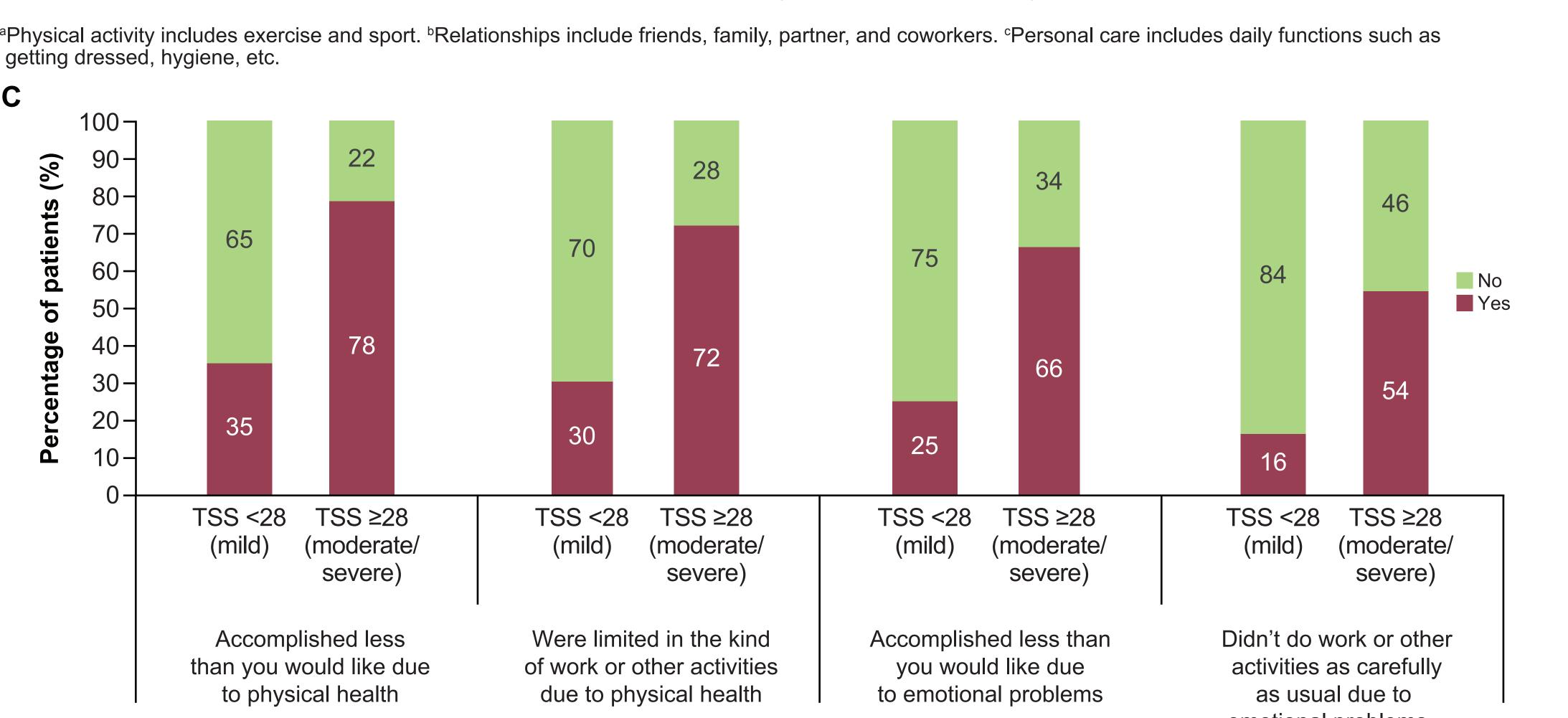
Figure 1. Patients, regardless of TSS score, reported cognitive, Figure 2. Patients, regardless of TSS score, reported that ISM impacted their emotional health and daily life



Patients scored each statement on a 1–5 scale: ■ 1 = No impact at all, ■ 2 = A little bit, ■ 3 = Somewhat, ■ 4 = Quite a bit, ■ 5 = A great deal of impact

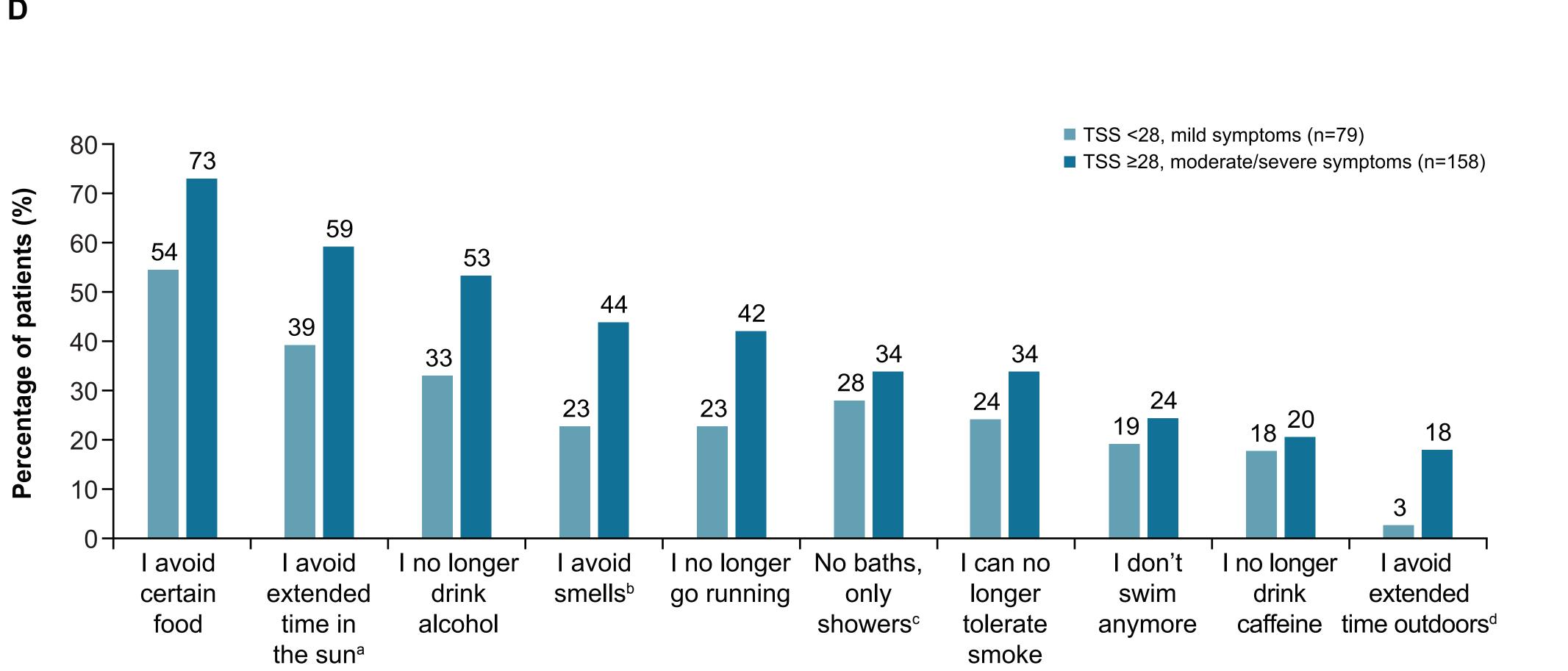
Figure 3. Patients across the spectrum of ISM reported (A) disruptions in daily life, (B) problems with daily living (EQ-5D-5L), (C) limitations to activities in the past 4 weeks (SF-12), and (D) making adjustments to their daily lives, with more symptomatic patients reporting more disruptions, limitations, and adjustments





^aSF-12 does not define physical health. ^bSF-12 presents 'feeling depressed or anxious' as examples of emotional problems.

Severe problems/symptoms



°Full survey text was 'I don't take baths and only take showers.' defull survey text was 'I can no longer stay outdoors for an extended period of time.'

- Overall, 67% of patients with ISM participating in the PRISM survey reported experiencing moderate or severe symptoms (TSS ≥28)
- All patients reported emotional and cognitive effects associated with their condition, as well as limitations on social, leisure, and daily activities
- A considerable proportion of patients responded 'quite a bit' or 'a great deal' when asked whether they felt that others did not understand their disease (48%), whether they felt anxious or worried about their disease (30%), or whether their disease was controlling their life (27%)
- Patients reported disruptions in many areas of their lives, including physical activity (TSS <28: 44%; TSS ≥28: 65%), work/college (TSS <28: 37%; TSS ≥28: 61%), relationships (TSS <28: 19%; TSS ≥28: 47%), leisure time (TSS <28: 29%; TSS ≥28: 44%), and personal care (TSS <28: 13%; TSS ≥28: 27%); with higher TSS scores corresponding to more disruptions
- Most patients also reported problems with pain/discomfort (TSS <28: 34%; TSS ≥28: 93%), anxiety/depression (TSS <28: 42%; TSS ≥28: 75%) and usual activities (TSS <28: 62%; TSS ≥28: 75%) as assessed by the Euro-QoL five-dimension-five level (EQ-5D-5L) survey
- Patients reported limitations to their activities due to ISM, including accomplishing less (TSS <28: 35%; TSS ≥28: 78%) and being limited in the kinds of work/activities (TSS <28: 30%; TSS ≥28: 72%) due to physical health, and accomplishing less (TSS <28: 25%; TSS ≥28: 66%) and being less careful with work/activities (TSS <28: 16%; TSS ≥28: 54%) due to emotional problems, with a greater proportion of patients with higher TSS reporting such limitations
- Adjustments made to daily life due to ISM included avoiding certain foods (TSS <28: 54%; TSS ≥28: 73%), avoiding extended sun exposure (TSS <28: 39%; TSS ≥28: 59%), no longer drinking alcohol (TSS <28: 33%; TSS ≥28: 53%), and avoiding smells (TSS <28: 23%; TSS ≥28: 44%); patients with higher TSS scores reported making more adjustments to their daily life

Conclusions

- Patients across the ISM spectrum reported disruptions, problems, limitations, and adjustments to their daily lives across all survey instruments. These were generally more severe in patients with greater symptomatic burden (TSS ≥28)
- These findings underscore the profound socio-emotional impact of ISM on patients, highlighting the need for a multidisciplinary approach that enhances overall quality of life and integrates mental health support into the overall disease management approach