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

**Massimo Triggiani**,<sup>1</sup> Deepti H Radia,<sup>2</sup> Cristina Bulai Livideanu,<sup>3</sup> Amélie Beaux,<sup>4</sup> Nicole Hegmann,<sup>5</sup> Franziska Ruëff,<sup>6</sup> Waltraud Schinhofen,<sup>7</sup> Iván Alvarez-Twose,<sup>8</sup> Eugenia Ribada,<sup>9</sup> Celeste C Finnerty,<sup>10</sup> Coralie Gressier-Sayag,<sup>11</sup> Dakota Powell,<sup>11</sup> Teresa Green,<sup>11</sup> Ruben Mesa,<sup>12</sup> Jessica Hobart<sup>13</sup>

<sup>1</sup>Division of Allergy and Clinical Immunology, University of Salerno, Salerno, Italy; <sup>2</sup> Guy's and St. Thomas' NHS Foundation Trust, London, UK; <sup>3</sup>French Reference Center for Mastocytosis (CEREMAST), Department of Dermatology, Toulouse University Hospital, Toulouse, France; <sup>4</sup>French Association of Patients with Mast Cell Diseases (ASSOMAST), Paris, France; <sup>5</sup>Mastocytosis Self-Support Network, eV, Odenthal, Germany; <sup>6</sup>Department of Dermatology and Allergy, University Hospital Ludwig Maximilian University of Munich, Munich, Germany; <sup>7</sup>Self-Support Association Mastocytosis eV, Toenisvorst, Germany; <sup>8</sup>Institute of Mastocytosis Studies of Castilla-La Mancha (CLMast), Toledo, Spain; <sup>9</sup>Spanish Association of Mastocytosis and Related Diseases (AEDM), Madrid, Spain; <sup>10</sup>The Mast Cell Disease Society, Sterling, MA, USA; <sup>11</sup>Blueprint Medicines Corporation, Cambridge, MA, USA; <sup>12</sup>Atrium Health Wake Forest Baptist Comprehensive Cancer Center, Winston-Salem, NC, USA; <sup>13</sup>The UK Mastocytosis Support Group, London, UK.

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# PRISM: Introduction

- Systemic mastocytosis (SM) is a clonal mast cell disease primarily driven by the *KIT* D816V mutation<sup>1</sup>
- The majority of patients with SM have indolent systemic mastocytosis (ISM), a subtype of non-advanced disease, characterized by unpredictable, severe, and debilitating skin, gastrointestinal, and systemic symptoms<sup>1,2</sup>
- A substantial number of patients with ISM experience a significant reduction in their quality of life (QoL) due to chronic, debilitating symptoms such as fatigue, abdominal pain, and anaphylactic reactions. These symptoms often lead to frequent healthcare visits and interventions, significantly affecting patients' daily activities and overall well-being<sup>2,3</sup>
- Previously, the US-based TouchStone survey reported that patients with advanced SM and ISM experienced high symptom burden, poor QoL, and significant impacts on patients' ability to work<sup>4</sup>
- The Perceptions, Realities & Insights on SM (PRISM) Patient Survey was administered in seven European countries to understand the experiences of patients with SM
- 540 patients with SM participated in PRISM; 237 patients reported having ISM
- This analysis reports PRISM survey results for patients with ISM



# PRISM: Largest European survey to assess symptomology, use of medications, and impact of SM on QoL and overall functioning

## Patient Survey

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

## Methods

- Designed by an international steering committee composed of SM clinical experts and patient advocates
- Ethics committees for participating countries (Italy, the UK, Spain, Germany, France, Switzerland, and Austria) approved the study materials
- Eligible participants  $\geq 18$  years old with a self-reported SM diagnosis who provided informed consent were recruited to participate in this survey
- Descriptive statistics on survey responses from the ISM patient cohort are reported



The majority of these patients with ISM were female with a mean age of approximately 50 years and were diagnosed on average more than 9 years ago

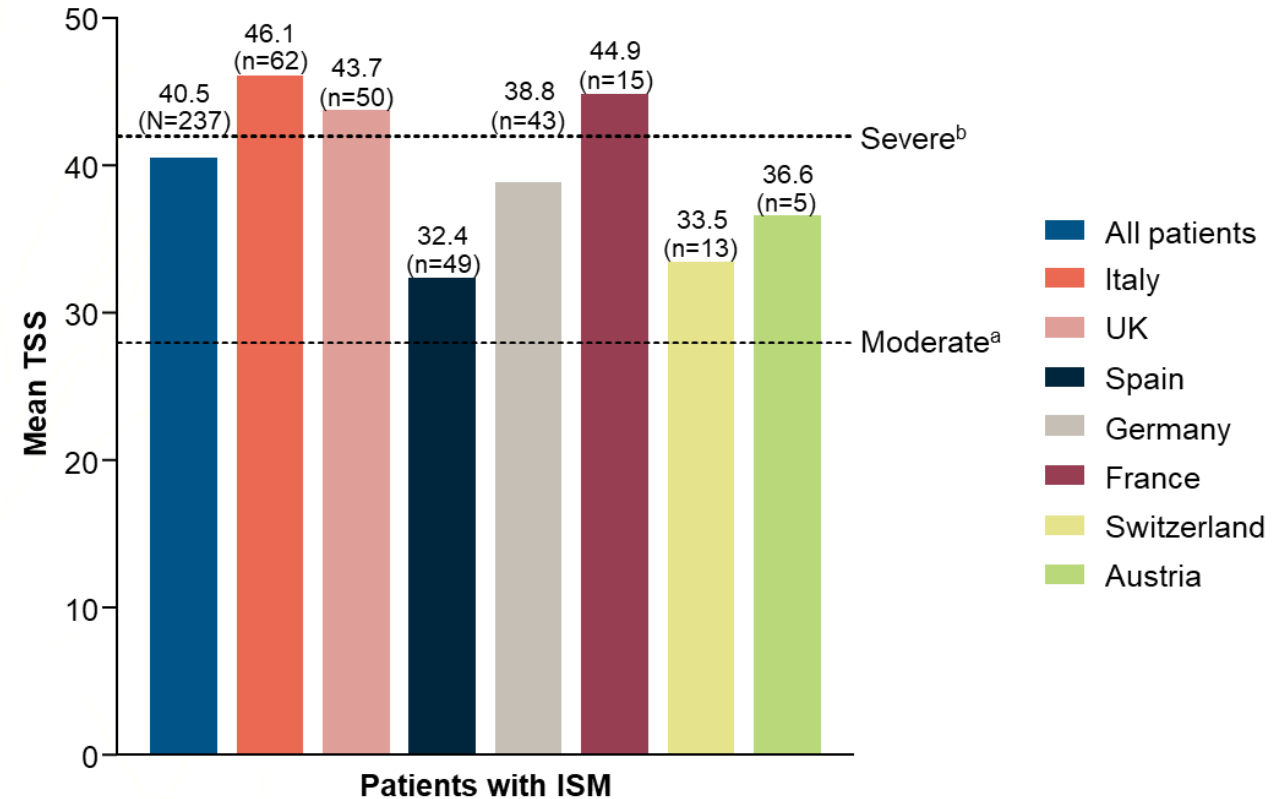
	All patients with ISM (N=237)	Italy (n=62)	UK (n=50)	Spain (n=49)	Germany (n=43)	France (n=15)	Switzerland (n=13)	Austria (n=5)
<b>Age, years, mean (SD)</b>	49.1 (12.7)	45.6 (12.7)	51.0 (14.3)	50.5 (10.0)	56.6 (10.8)	37.1 (8.2)	46.6 (9.1)	36.8 (9.7)
<b>Sex, female, n (%)</b>	168 (71)	42 (68)	43 (86)	38 (78)	30 (70)	3 (20)	10 (77)	2 (40)
<b>Time since ISM diagnosis, months, mean</b>	114.7	80.0	95.8	152.0	170.5	68.9	111.1	37.6

ISM, indolent systemic mastocytosis; SD, standard deviation.

# Patients reported having moderate to severe ISM symptoms

## Mean Total Symptom Score (TSS)

ISM Symptom Assessment Form (ISM-SAF)	
ISM Symptom	Scoring
Abdominal pain	<p>The ISM-SAF questionnaire calculates a total symptom score (TSS) based on severity of 11 ISM symptoms</p> <p>Each symptom is scored 0–10; where: 0 = no [symptom] 10 = <b>worst imaginable</b> [symptom]</p> <p>Individual symptom severity scores are summed to generate a TSS (0–110)</p>
Diarrhea	
Nausea	
Spots	
Itching	
Flushing	
Brain fog	
Headache	
Dizziness	
Bone pain	
Fatigue	



<sup>a</sup>TSS ≥ 28 indicates moderate symptom profile.<sup>1</sup>

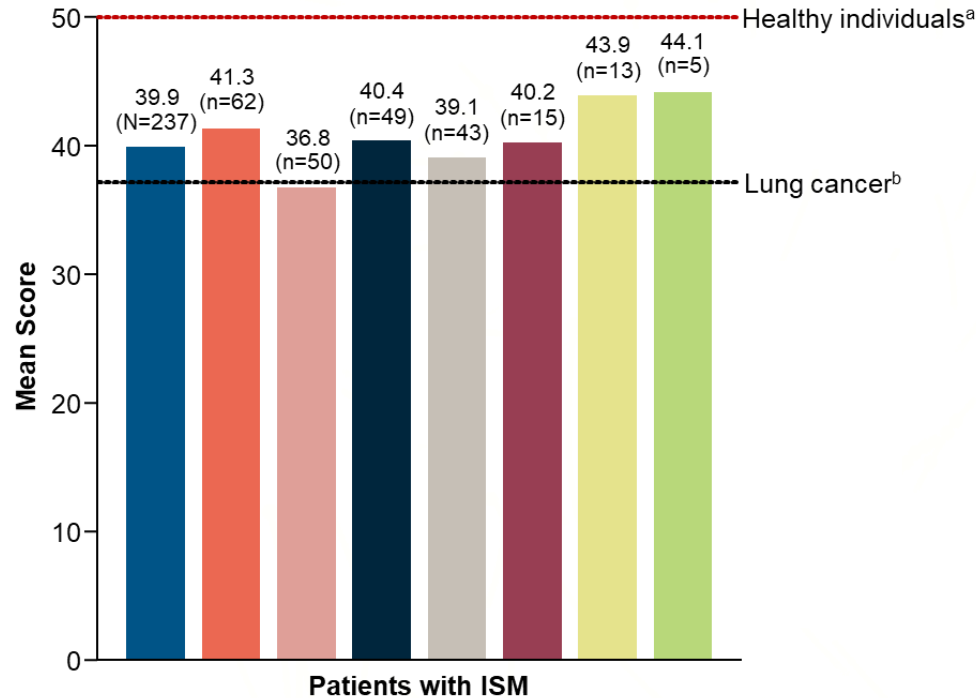
<sup>b</sup>TSS > 42 indicates severe symptom profile.<sup>2</sup>

ISM, indolent systemic mastocytosis; ISM-SAF, Indolent Systemic Mastocytosis Symptom Assessment Form.

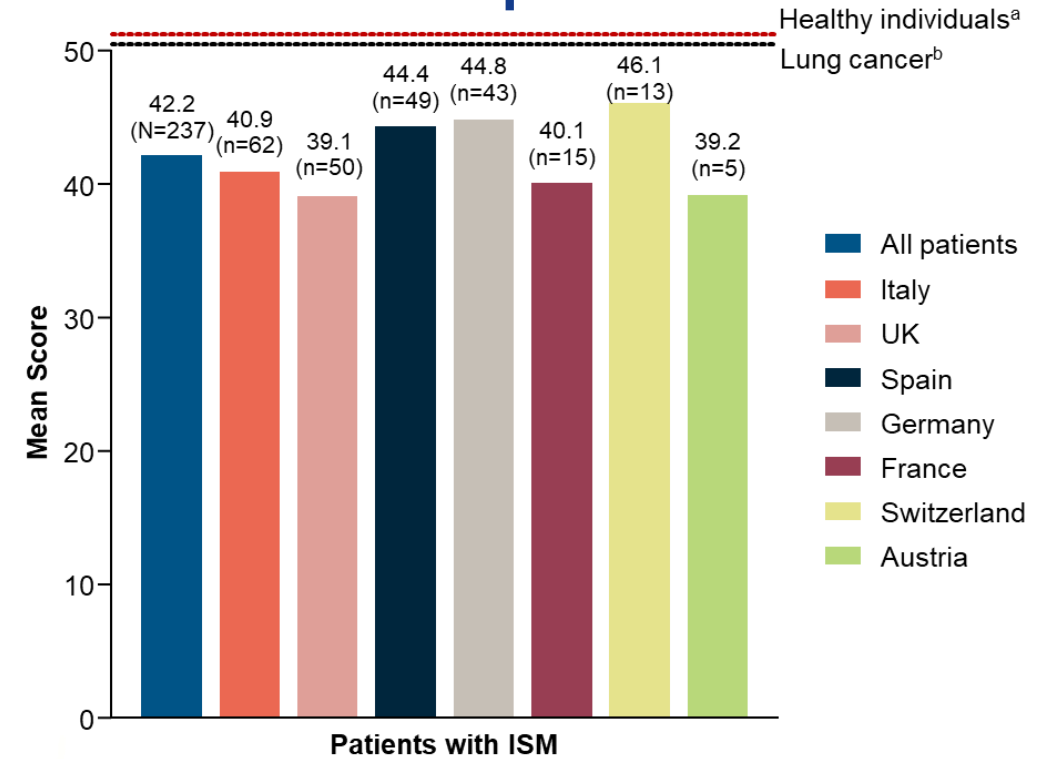
1. Shields AL, et al. *Orphanet J Rare Dis.* 2023;18(1):69; 2. Gotlib J, et al. *NEJM Evid.* 2023;2(6):EVIDoa2200339.

# Patients with ISM reported substantially impaired physical and mental functioning

## SF-12 physical component score



## SF-12 mental component score



<sup>a</sup>Mean score for healthy individuals is approximately 50.<sup>1</sup>

<sup>b</sup>Mean physical component score for patients with lung cancer is 37.2 and the mean mental component score is 50.1.<sup>2</sup>

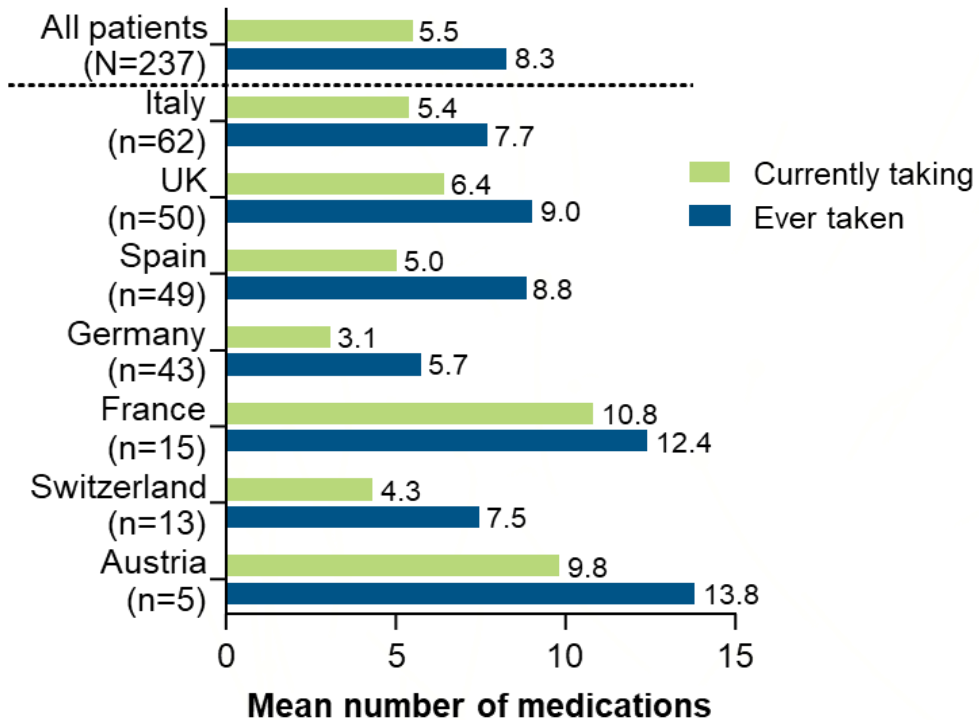
ISM, indolent systemic mastocytosis; SF-12, 12-item short form survey.

1. Ware JE, et al. *SF-12: How to Score the SF-12 Physical and Mental Health Summary Scales*. 4th ed. QualityMetric Inc.; Health Assessment Lab; 2002; 2. Kenzik KM et al. *Cancer*. 2015 Aug 15;121(16):2831-839.



# Patients reported high lifetime use of symptom-directed medications and rates of anaphylaxis

## Mean number of ISM medications

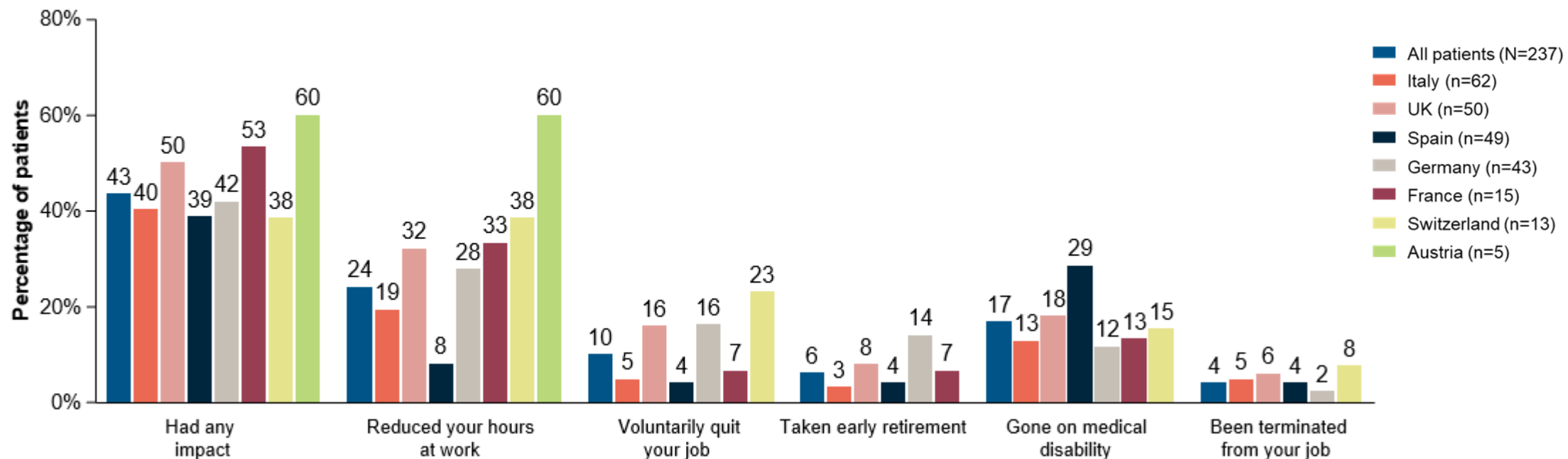


- Patients reported currently taking a mean number of 5.5 medications
- 38% of patients reported experiencing anaphylactic episodes during the prior year
  - 21% of patients experiencing anaphylaxis had 5 or more episodes
- Food (37%), stress (31%), medication (24%), and temperature changes (27%) were the most common anaphylaxis triggers for patients

ISM, indolent systemic mastocytosis.

# Patients with ISM reported a significant workforce impact

## Impact of ISM on work status



**43% of patients reported that ISM had meaningful impact on their ability to work, with 24% reducing hours and 17% going on medical disability**

ISM, indolent systemic mastocytosis.

# **Conclusions:** PRISM survey findings in ISM show high disease burden and unmet need for patients despite use of polypharmacy

- To date, PRISM is the largest survey to evaluate the burden of ISM from a patient perspective
- These data show that patients with ISM report moderate to severe disease-related symptoms, impaired physical and mental functioning, and frequent anaphylaxis despite being treated with multiple symptom-targeted medications (current mean: 5.5)
- Additionally, ISM impacts patients' ability to work in approximately 43% of patients



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ISM, indolent systemic mastocytosis; PRISM, Perceptions Realities and Insights on Systemic Mastocytosis.

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