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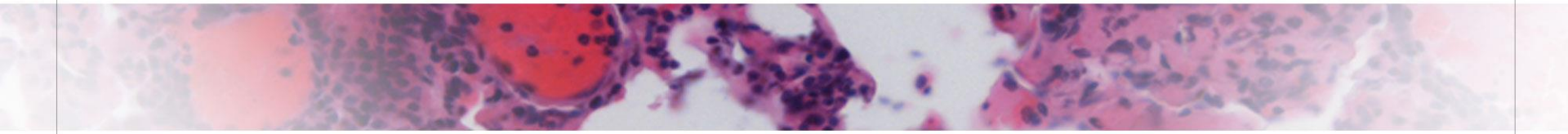

Perceptions of patient disease burden and management approaches of systemic mastocytosis (SM) by healthcare providers (HCPs): results from the Touchstone SM survey

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SM is a rare, clonal, mast cell (MC) neoplasm

Characterized by MC hyperactivation, proliferation, degranulation, and mediator release[1,2]

- Symptoms are unpredictable, and may lead to significant morbidity and shortened overall survival[1,2]
  - Can include severely debilitating gastrointestinal, skin, neurocognitive, and systemic symptoms (including life-threatening anaphylaxis)
- In advanced SM, MC organ infiltration and damage occur[1,2]

Diagnosis of SM is challenging and often delayed, requiring multi-disciplinary teams[2,3]

- Driven by *KIT* D816V mutation in ~95% of cases[4–6]
- Patient treatment and management are challenging[2,3]
- Allergists/immunologists and hematologists/oncologists play key roles[2,3]
- Further information is needed across specialties to enable optimal care of patients with SM,[2] as there are few multi-disciplinary SM practices in the USA

Here we report findings from the TouchStone SM survey of HCPs in the USA to inform on perceptions of disease and disease management strategies

HCP, healthcare provider; MC, mast cell; SM, systemic mastocytosis.

1. Rossignol J et al. *F1000Research*. 2019;8(https://doi.org/10.12688/f1000research.19463.1); 2. Valent P et al. *Int J Mol Sci*. 2019;20:2976; 3. Jennings SV et al. *Immunol Allergy Clin North Am*. 2018;38:505–525; 4. Garcia-Montero AC et al. *Blood*. 2006;108:2366–2372; 5. Kristensen T et al. *J Mol Diagn*. 2011;13:180–188; 6. Pardanani A. *Am J Hematol*. 2019;94:363–377.

TouchStone HCP survey: methods

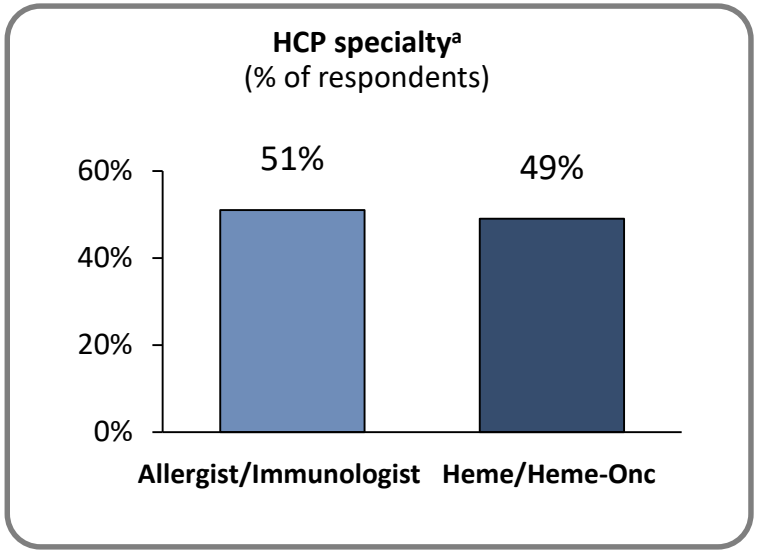
- Practicing allergists/immunologists and hematologists/oncologists recruited through double-blinded market research survey panels, with individual responses kept confidential
- HCPs were considered eligible if they cared for ≥4 patients with SM and had been in practice ≥3 years post-fellowship

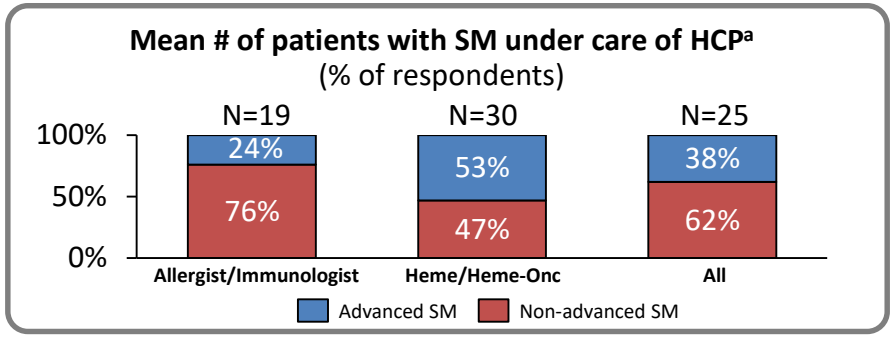
- A 47-item survey was created and administered online to HCPs, with questions on the following:
  - Background (specialty, number of patients, type and frequency of visits)
  - SM diagnosis (how made, how long it takes, patient communication)
  - SM symptoms and perceived impact on patient's daily life (how assessed, what symptoms patients experience, how HCPs discuss symptoms)
  - Disease management (types of treatment, goals of treatment, satisfaction, unmet needs)

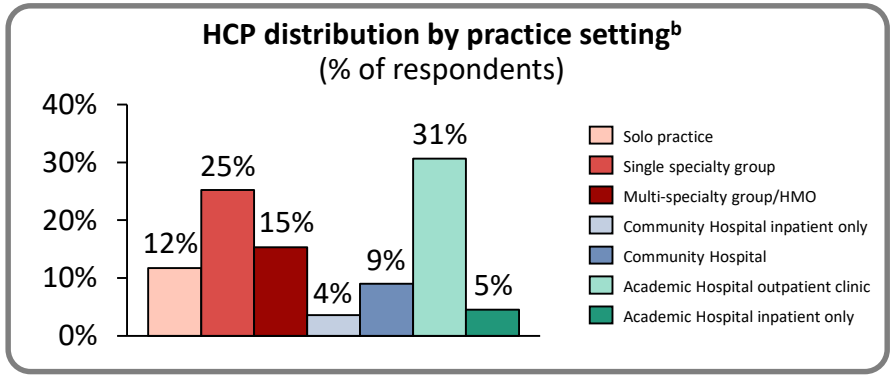
- Descriptive analyses were summarized by physician specialty and SM sub-type

TouchStone HCP survey participants: practice profile

Survey respondent demographics (All HCPs n=119)



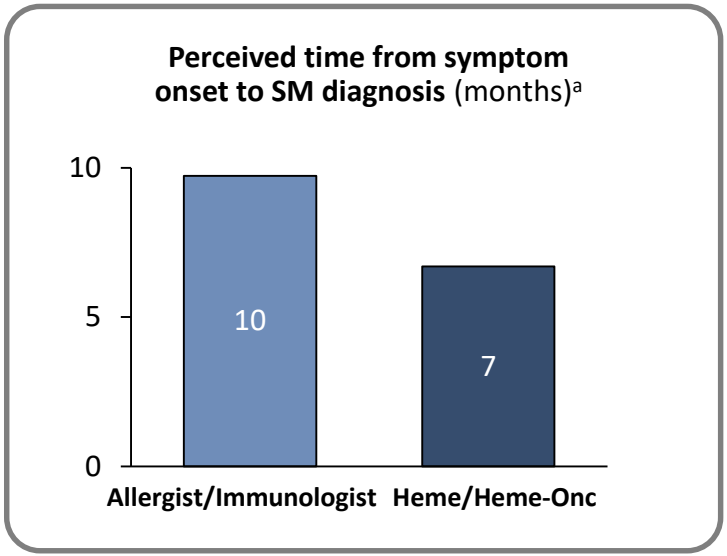


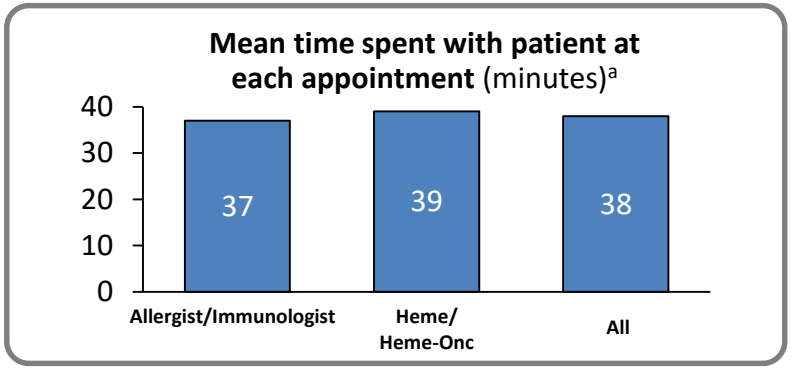


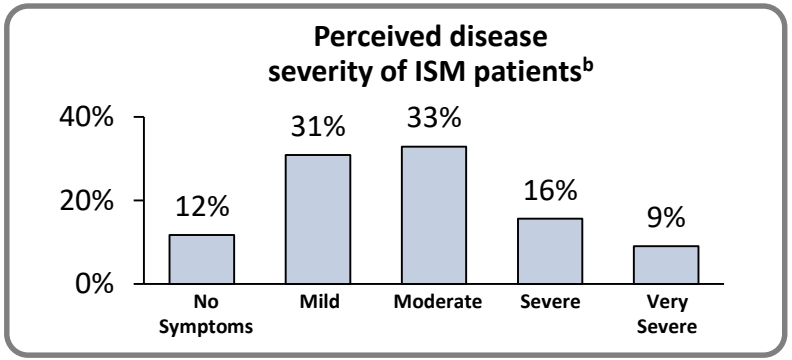
[a]Total respondent sample was N=119, after removing 1 respondent due to poor data quality, and included 60 allergists/immunologists and 59 hematologists/oncologists. [b]Data from 111 HCPs respondents.
HMO, health maintenance organization.

TouchStone HCP survey participants: practice profile

Survey respondent demographics (All HCPs n=119)



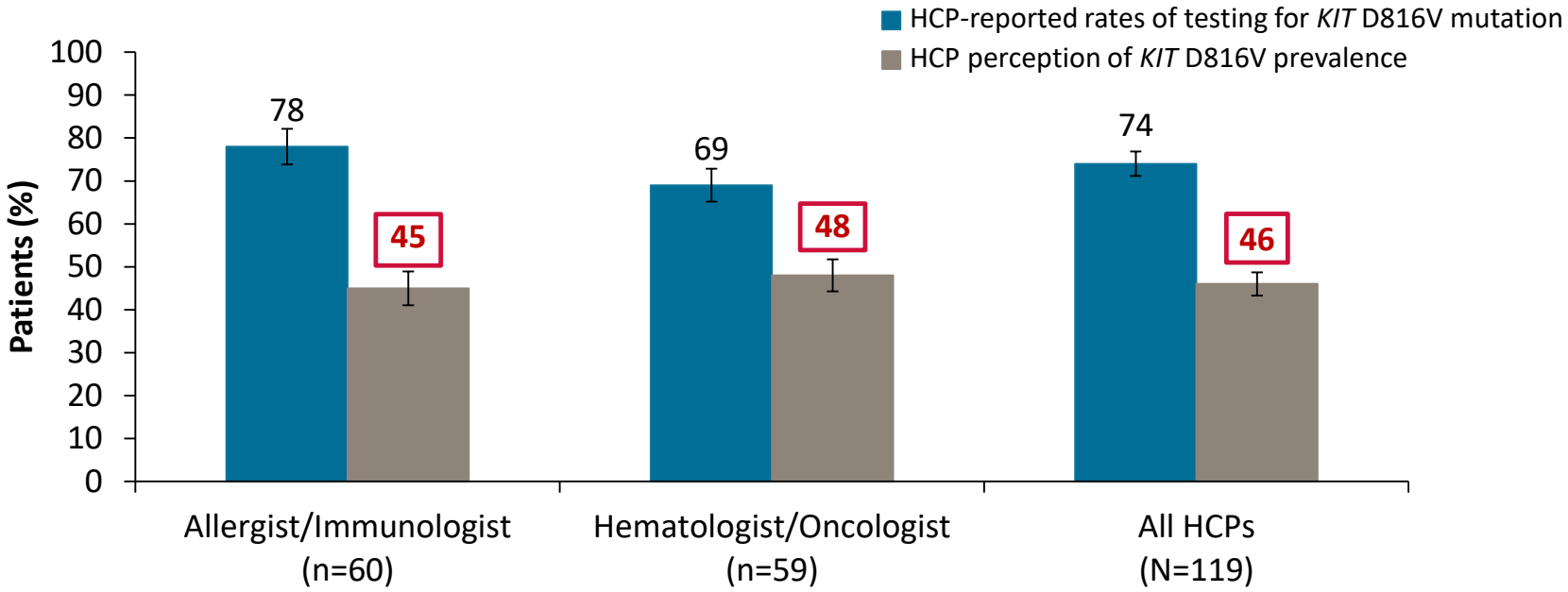




[a]Total respondent sample was N=119, after removing 1 respondent due to poor data quality, and included 60 allergists/immunologists and 59 hematologists/oncologists. [b]Data from 111 HCPs respondents.
ISM, indolent systemic mastocytosis (non-advanced SM form).

Participating HCPs reported they test a majority (74%) of their patients with SM for *KIT* D816V mutation

- HCP respondents reported lower *KIT* D816V prevalence compared with published estimates of 95%[1–3]



1. Garcia-Montero AC et al. *Blood*. 2006;108:2366–2372; 2. Kristensen T et al. *J Mol Diagn*. 2011;13:180–188; 3. Pardanani A. *Am J Hematol*. 2019;94:363–377.

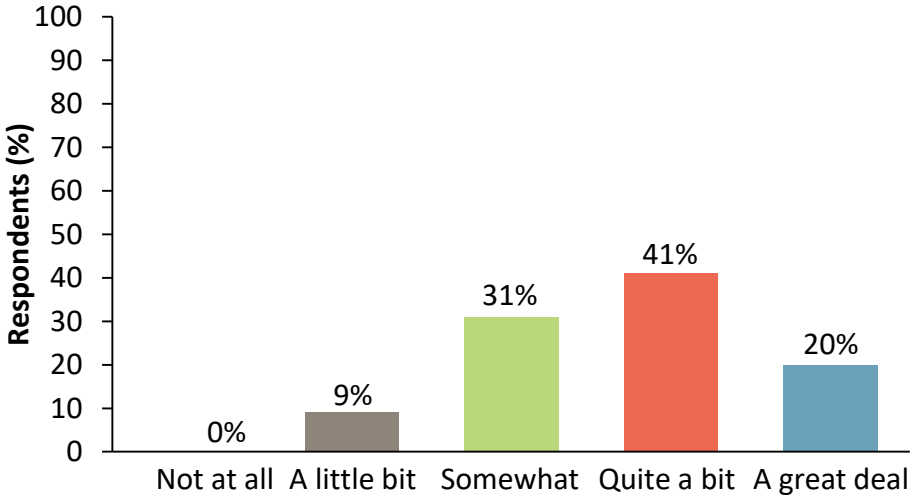
Improved overall and progression-free survival, and quality of life were most important SM treatment goals reported by HCPs

Treatment goal	Treatment goals for patients with advanced SM[a]		Treatment goals for patients with non-advanced SM[b]	
	Most important (%)	2nd most important (%)	Most important (%)	2nd most important (%)
Improved progression-free survival/overall survival	34	13	18	12
Better quality of life	25	25	40	18
Delay disease progression, reduce risk of organ damage	21	24	13	24
Improvement of symptoms	8	24	15	24
Maintain ability to do usual activities	4	8	6	13
Reduction in number of anaphylactic episodes	3	1	3	3
Other (e.g., normal blood counts, normalization of bone marrow, reduction in steroid use, and reduction in tryptase levels)	6	6	4	7

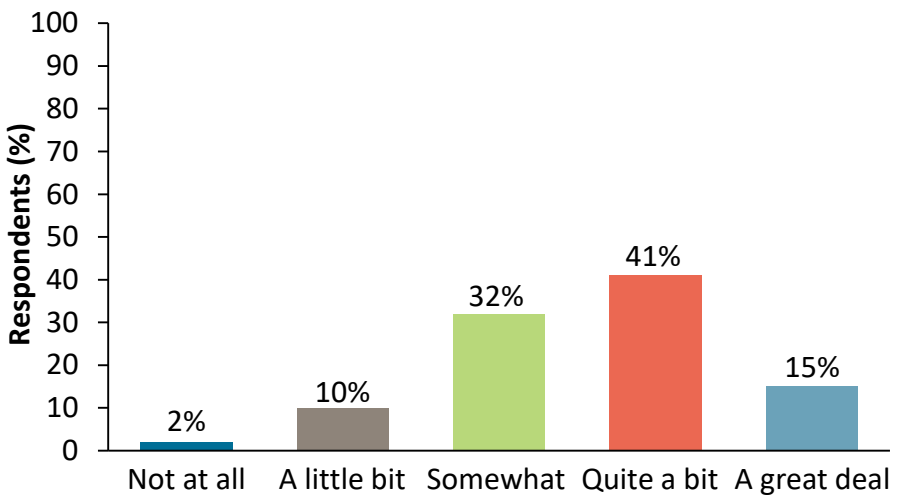
[a]Data from 104 respondents. [b]Data from 119 respondents.

Majority of HCPs responded that patients with ISM feel depressed or discouraged and report limited activities due to pain or discomfort

Patients with ISM feel **depressed or discouraged**



Patients with ISM feel their **pain or discomfort** causes them to limit their activities



Data from 111 respondents.

Conclusions

- HCPs participating in the TouchStone SM survey reported a perceived prevalence of *KIT* D816V mutations among SM patients lower than published estimates.[1–3]
- The most important treatment goals for patients with advanced and non-advanced SM were improved progression-free survival/overall survival and better quality of life.
- A majority of HCPs indicated that patients with ISM under their care report feeling depressed or discouraged and limit their activities due to pain or discomfort.
- Allergists/immunologists and hematologists/oncologists both play a role in the treatment of patients with advanced and non-advanced SM
- Future studies should link the HCP perceptions of the burden/management of SM to those of their patients.

1. Garcia-Montero AC et al. *Blood*. 2006;108:2366–2372; 2. Kristensen T et al. *J Mol Diagn*. 2011;13:180–188; 3. Pardanani A. *Am J Hematol*. 2019;94:363–377.

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