

**Multidisciplinary insights:  
Navigating the challenges of systemic  
mastocytosis diagnosis and management**

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# Expert panel



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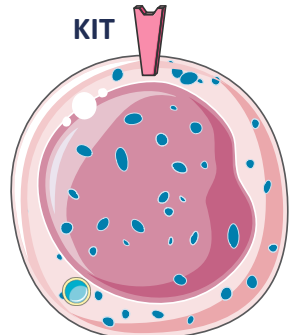
**Dermatologist/Allergist**  
Odense University Hospital  
Odense, Denmark



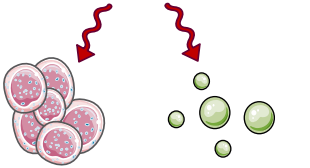
# **How is systemic mastocytosis diagnosed?**

## **Exploring the current patient journey**

# Systemic mastocytosis is a heterogeneous disease



Mast cell



Clonal expansion

Mediator release



Neoplastic clonal proliferation of abnormal MCs in extracutaneous organs<sup>1</sup>

Clinical presentation ranges from indolent to aggressive disease<sup>1</sup>

Genetic and molecular abnormalities associated with SM subtypes<sup>1,2</sup>

- Activating *KIT* mutations found in a majority of SM cases in adults, regardless of subtype<sup>1</sup>

ICC/WHO criteria used to distinguish SM from other MC disorders<sup>1</sup>

## Main symptoms:<sup>2,3</sup>

- Anaphylaxis
- GI symptoms
- Fatigue
- Flushing, pruritis
- Bone and muscle pain (osteopenia/osteoporosis)



UP skin lesions<sup>5</sup>



Monomorphic



Polymorphic

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GI, gastrointestinal; ICC, International Consensus Classification; MC, mast cell; SM, systemic mastocytosis; UP, urticaria pigmentosa; WHO, World Health Organization.

1. Pardanani A. *Am J Hematol*. 2023;98:1097–116; 2. Lee HJ, et al. *Blood Res*. 2023;58:S96–108; 3. van der Weide HY, et al. *Crit Care*. 2015;19:238;

4. Hartmann K, et al. *J Allergy Clin Immunol*. 2016;137:35–45; 5. Broesby-Olsen S. Available at [https://www.eaaci.org/images/Atlas/Global\\_Atlas\\_IV\\_v1.pdf](https://www.eaaci.org/images/Atlas/Global_Atlas_IV_v1.pdf) (accessed 28 June 2024).



**What are the current treatment options  
for systemic mastocytosis?**



# Treatment goals in systemic mastocytosis

HCP-selected  
treatment goals:



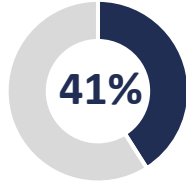
## 51-item HCP survey

51% A/I specialists and  
49% H/O specialists



**#1** Most important

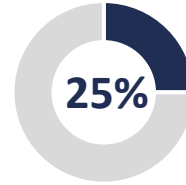
ISM  
(n=111)



Better QoL

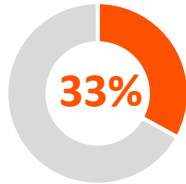


**#2** Second most important

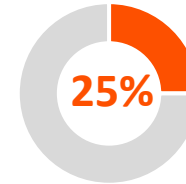


Improvement of symptoms

AdvSM  
(n=96)



Improved PFS/OS



Delay disease progression,  
reduce risk of organ damage

## Other goals

- Maintain ability to do usual activities
- Reduction in anaphylactic episodes
- Normal blood counts
- Normalization of bone marrow
- Reduction in steroid use
- Reduction in tryptase levels

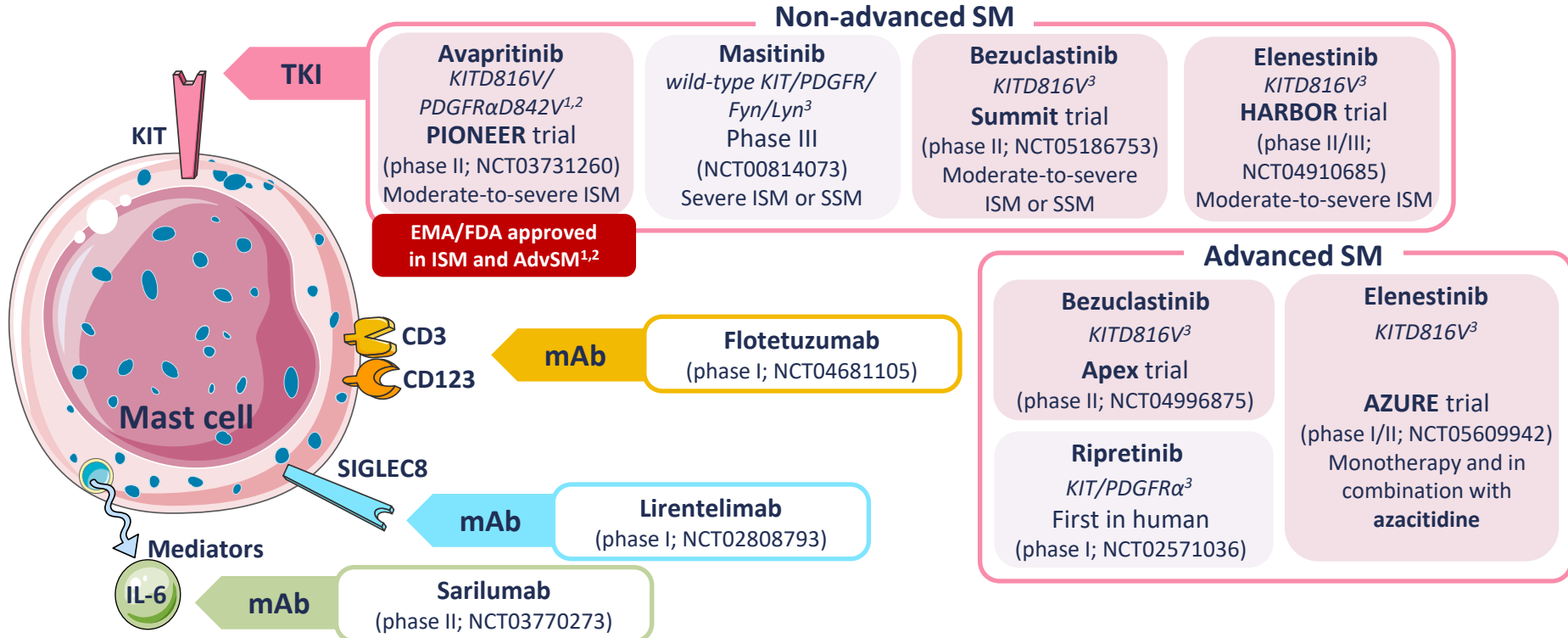


**What does the future hold?  
Novel and emerging treatment strategies  
for systemic mastocytosis**





# Emerging therapies and indications



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AdvSM, advanced SM; ISM, indolent SM; mAb, monoclonal antibody; SM, systemic mastocytosis; SSM, smoldering SM; TKI, tyrosine kinase inhibitor.

1. EMA. Avapritinib SmPC. Available at: <https://bit.ly/3KoCCJb> (accessed 24 May 2024); 2. FDA. Avapritinib PI. Available at:

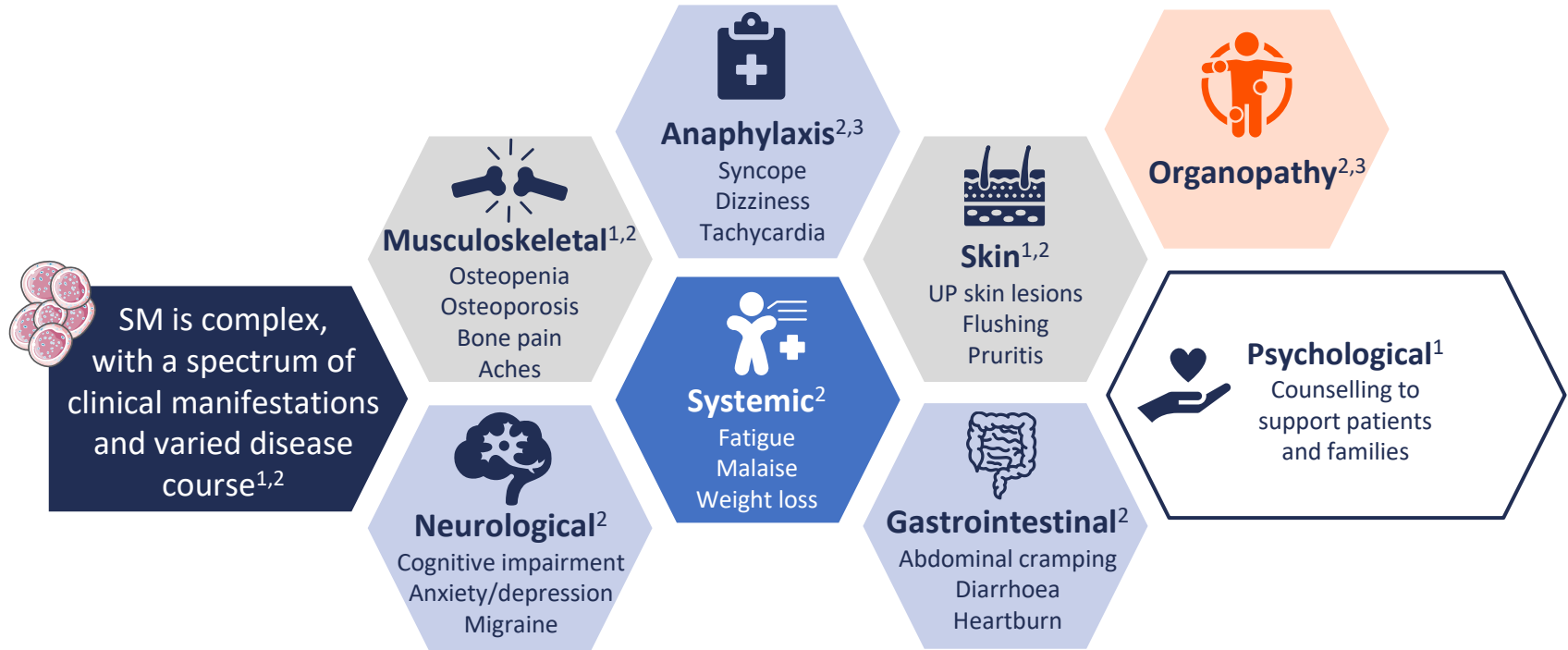
[www.accessdata.fda.gov/drugsatfda\\_docs/label/2023/212608s013lbl.pdf](http://www.accessdata.fda.gov/drugsatfda_docs/label/2023/212608s013lbl.pdf) (accessed 24 May 2024); 3. Sciumè M, et al. *Pharmaceuticals (Basel)*. 2022;15:738. Trial information searchable by NCT number at [www.clinicaltrials.gov](http://www.clinicaltrials.gov) (accessed 13 May 2024).



**Can we do more for our patients?  
Multidisciplinary management approaches**



# MDT management needs in systemic mastocytosis



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MDT, multidisciplinary team; SM, systemic mastocytosis; UP, urticaria pigmentosa.

1. Zanotti R, et al. *Mediterr J Hematol Infect Dis*. 2021;13:e2021068; 2. Veitch S, Radia DH. *Hematology Am Soc Hematol Educ Program*. 2023;2023:396–406;

3. Lee HJ, et al. *Blood Res*. 2023;58:S96–108.