

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

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Expert panel



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University of Antwerp
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Haemato-Oncologist
MD Anderson Cancer Center
University of Texas, TX, USA



Dr Sigurd Broesby-Olsen

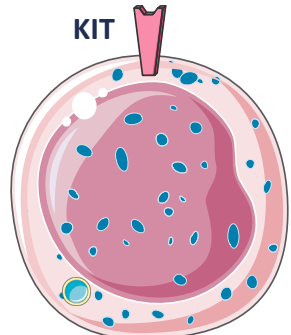
Dermatologist/Allergist
Odense University Hospital
Odense, Denmark



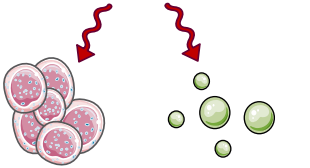
How is systemic mastocytosis diagnosed?

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 organs¹

Clinical presentation ranges from indolent to aggressive disease¹

Genetic and molecular abnormalities associated with SM subtypes^{1,2}

- Activating *KIT* mutations found in a majority of SM cases in adults, regardless of subtype¹

ICC/WHO criteria used to distinguish SM from other MC disorders¹

Main symptoms:^{2,3}

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

UP skin lesions⁵



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 (accessed 28 June 2024).



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



Treatment goals in systemic mastocytosis

HCP-selected
treatment goals:



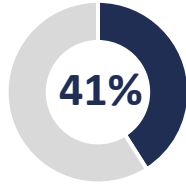
**51-item
HCP survey**

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



#1 Most important

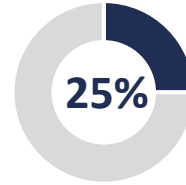
ISM
(n=111)



Better QoL

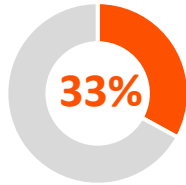


#2 Second most important

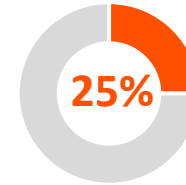


Improvement of symptoms

AdvSM
(n=96)



Improved PFS/OS



Delay disease progression,
reduce risk of organ damage

Other goals

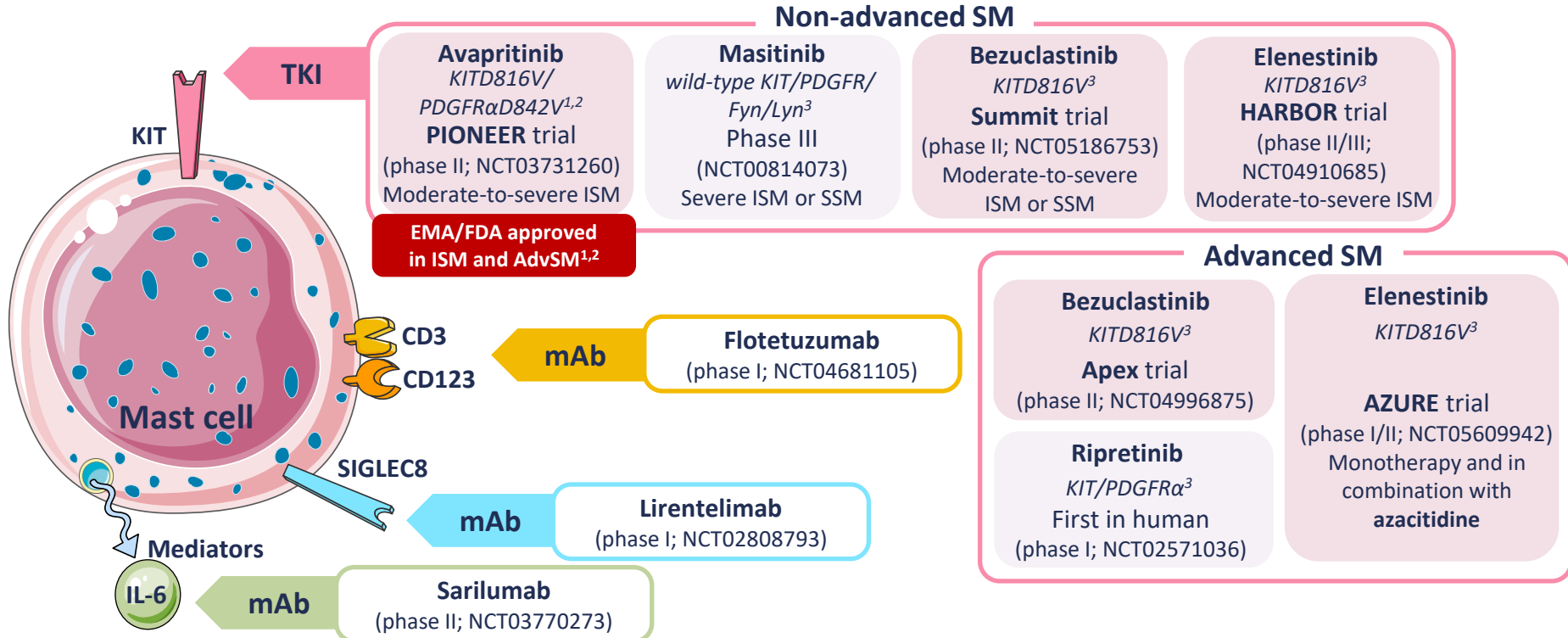
- Maintain ability to do usual activities
- Reduction in anaphylactic episodes
- Normal blood counts
- Normalization of bone marrow
- Reduction in steroid use
- Reduction in tryptase levels



**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.

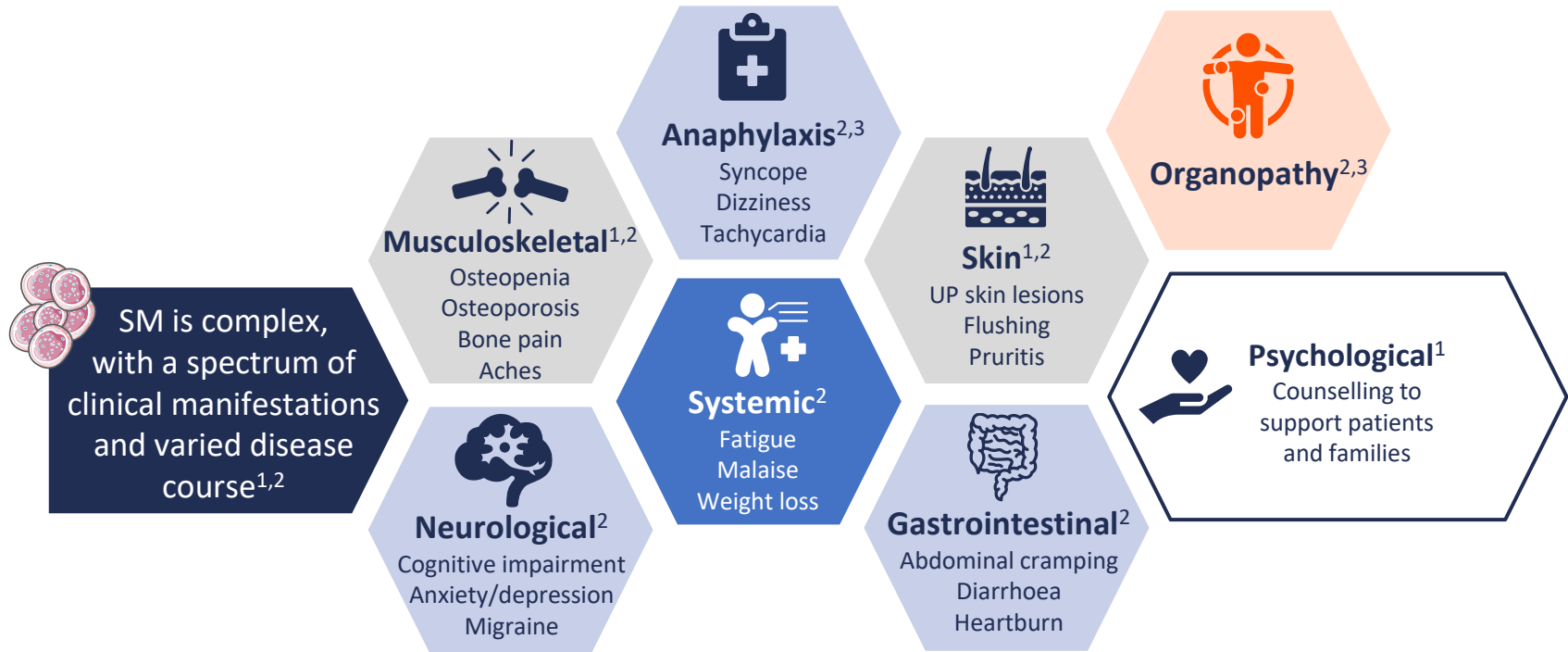
1. EMA. Avapritinib SmPC. Available at: <https://bit.ly/3KoCCJb> (accessed 24 May 2024); 2. FDA. Avapritinib PI. Available at:

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