Novartis Oncology

Systemic Mastocytosis Fact Sheet

What is Systemic Mastocytosis?

- Systemic mastocytosis (SM) is a group of rare diseases in which uncontrolled growth and accumulation of mast cells (a type of white blood cell) occurs in one or more organs[1]
- In patients with SM, mast cells can accumulate in multiple organ systems, including the skin, GI tract, spleen, lymph nodes and bone marrow[2]
  - » SM can be characterized into indolent (ISM) and advanced forms. ISM is the benign form of SM and is generally associated with a good prognosis, while advanced SM can be fatal within a few years[1]
  - » The mast cells release substances such as histamine, which can lead to symptoms including itching, fever, abdominal pain, nausea and vomiting[2]

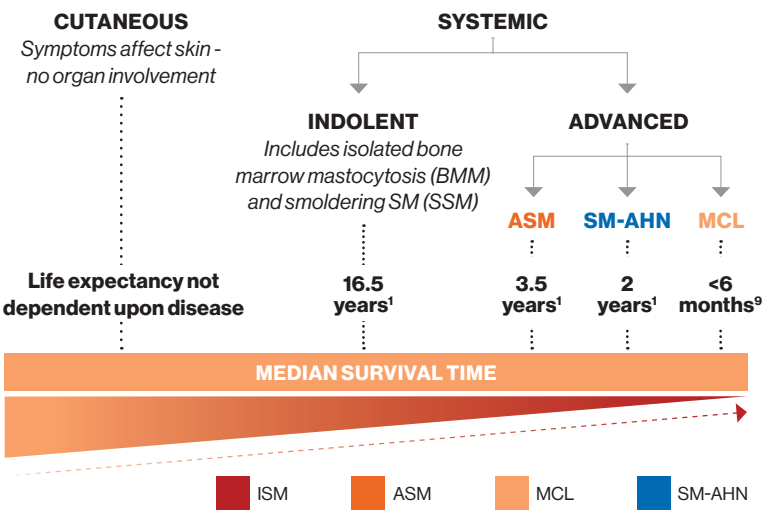
SM affects between
1 in 20,000
and
1 in 40,000
people **worldwide**[5]

A Closer Look at Advanced SM

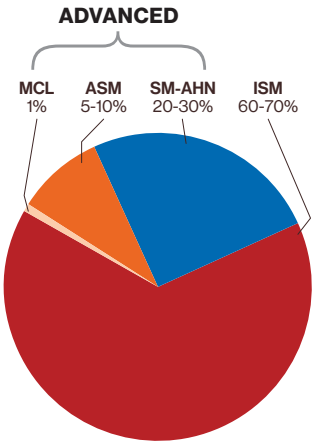
- In advanced SM, mast cells accumulate in such high quantities that they lead to organ damage and dysfunction, bone fractures and anemia.[3] Subtypes of advanced SM include aggressive systemic mastocytosis (ASM), mast cell leukemia (MCL) and SM with an associated hematologic neoplasm (SM-AHN)[2]
- Treatment is tailored towards each individual patient and their disease[4]
- The major goal of treatment is to control mast cell growth and expansion[4]

The uncontrolled proliferation of mast cells is caused in many people by a KIT mutation.[6] **D816V is the most common** mutation in SM, occurring in
~90%
of SM patients[6]

Types of Mastocytosis[7]



Percentage of SM Cases by Subtype[8]



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1. Lim KH, Tefferi A, Lasho T, et al. *Blood.* 2009;113:5727-5736. **2.** Arock M, Akin C, Hermine O, et al. *Eur J Haematology.* 2015;94(6):474-494. **3.** Verstovsek S. *Eur J Haematology.* 2013;90(2):89-98. **4.** Valent P. *Am J of Cancer Res.* 2013;3(2):159. **5.** Orpha.net. http://www.orpha.net/consor/cgi-bin/OC_Exp.php?Expert=2467. Accessed February 1, 2017. **6.** Garcia-Montero AC, Jara-Acevedo M, Teodosi C, et al. *Blood.* 2006;108(7):2366-2372. **7.** Pardanani A. *Am J Hematol.* 2015;90:251-262. **8.** Gotlib J, Kluin-Nelemans HC, George TI, et al. Supplement to: Efficacy and safety of midostaurin in advanced systemic mastocytosis. *N Engl J Med.* 2016;374:2530-2541. **9.** Georgin-Lavialle S, Lhermitte L, Dubreuil P, et al. *Blood.* 2013;121:1285-1295.

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