# FDA Ayvakit for Advanced Systemic Mastocytosis

June 21, 2021 By Food and Drug Administration (FDA)

---

On June 16, 2021, the Food and Drug Administration approved avapritinib (Ayvakit, Blueprint Medicines Corp.) for adult patients with advanced systemic mastocytosis (AdvSM), including patients with aggressive systemic mastocytosis (ASM), systemic mastocytosis with an associated hematological neoplasm (SM-AHN), and mast cell leukemia (MCL).

Efficacy was evaluated in EXPLORER (NCT02561988) and PATHFINDER (NCT03580655), two multi-center, single-arm, open-label clinical trials enrolling patients with AdvSM. The main efficacy outcome measure was overall response rate (ORR) per modified IWG-MRT-ECNM criteria, as adjudicated by a central committee. Additional efficacy measures were duration of response (DOR), time to response, and changes in individual measures of mast cell burden. Fifty-three patients received daily doses of avapritinib, up to 200 mg.

The ORR in all evaluable patients in both trials combined was 57% (95% CI: 42, 70) (n=53), with 28% complete remissions and 28% partial remissions. The median duration of response was 38.3 months (95% confidence interval: 19, not estimable) and the median time to response was 2.1 months.

The most common adverse reactions (incidence ≥ 20%) in patients with AdvSM were edema, diarrhea, nausea, and fatigue/asthenia.

Avapritinib is not recommended for the treatment of patients with AdvSM with platelet counts of less than 50 X $10^9$/L.

The recommended avapritinib dose is 200 mg orally once daily for patients with AdvSM.

View full prescribing information for Ayvakit.

This application was granted priority review, breakthrough designation and orphan drug designation. A description of FDA expedited programs is in the Guidance for Industry: Expedited Programs for Serious Conditions-Drugs and Biologics.

Healthcare professionals should report all serious adverse events suspected to be associated with the use of any medicine and device to FDA's MedWatch Reporting System or by calling 1-800-

FDA-1088.

This announcement was originally published on the Food and Drug Administration website on June 16, 2021.

---

© 2026 Smart + Strong All Rights Reserved.
http://beta.docker.cancerhealth.com/blog/fda-ayvakit-advanced-systemic-mastocytosis