FDA approves avapritinib for advanced systemic mastocytosis

September 2021—Blueprint Medicines announced that the FDA approved Ayvakit (avapritinib) for the treatment of adult patients with advanced systemic mastocytosis, including aggressive systemic mastocytosis, systemic mastocytosis with an associated hematological neoplasm, and mast cell leukemia. Advanced systemic mastocytosis patients can now receive a targeted therapy designed to potently and selectively inhibit D816V mutant KIT.

Efficacy was evaluated in EXPLORER and PATHFINDER, two multicenter, single-arm, open-label clinical trials enrolling patients with advanced systemic mastocytosis. The main efficacy outcome measure was overall response rate per modified International Working Group, Myeloproliferative Neoplasms Research and Treatment, and European Competence Network on Mastocytosis criteria. Additional efficacy measures were duration of response, time to response, and changes in individual measures of mast cell burden.

In 53 evaluable patients who had a median follow-up of 11.6 months, the overall response rate was 57 percent (95 percent confidence interval [CI], 42 percent, 70 percent), with 28 percent complete remissions and 28 percent partial remissions. The median duration of response was 38.3 months (95 percent CI, 19 months, not estimable) and the median time to response was 2.1 months.

Avapritinib is not recommended for the treatment of patients with advanced systemic mastocytosis with platelet counts of less than 50×10^9/L.

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