Ayvakit™ (avapritinib) – New indication

- On June 16, 2021, Blueprint Medicines announced the FDA approval of Ayvakit (avapritinib), for the treatment of adult patients with advanced systemic mastocytosis (AdvSM). AdvSM includes patients with aggressive systemic mastocytosis (ASM), systemic mastocytosis with an associated hematological neoplasm (SM-AHN), and mast cell leukemia (MCL).
    - Ayvakit is not recommended for the treatment of patients with AdvSM with platelet counts of less than 50 x 10^9/L.
- Ayvakit is also approved for the treatment of adults with unresectable or metastatic gastrointestinal stromal tumors (GIST) harboring a platelet-derived growth factor receptor alpha (PDGFRA) exon 18 mutation, including PDGFRA D842V mutations.
- SM is a rare disease driven by the KIT D816V mutation. Uncontrolled proliferation and activation of mast cells result in chronic, severe and often unpredictable symptoms for patients across the spectrum of SM.
- The approval of Ayvakit for the new indication was based on EXPLORER and PATHFINDER, two single-arm, open-label studies in patients with AdvSM. The efficacy of Ayvakit was based on overall response rate (ORR) in 53 patients with AdvSM dosed at up to 200 mg daily. Additional efficacy outcome measures were duration of response (DOR) and time to response.
    - The ORR was 57% (95% CI: 42, 70).
    - The median DOR was 38.3 months (95% CI: 19, not estimable) and the median time to response was 2.1 months.
- The most common adverse events (≥ 20%) with Ayvakit use in AdvSM were edema, diarrhea, nausea, and fatigue/asthenia.
- The recommended dosage of Ayvakit in patients with AdvSM is 200 mg orally once daily. Treatment should be continued until disease progression or unacceptable toxicity.
    - Refer to the Ayvakit drug label for dosing for GIST.