Ayvakit® (avapritinib) – New orphan indication

- On May 22, 2023, Blueprint Medicines announced the FDA approval of Ayvakit (avapritinib), for the treatment of adult patients with indolent systemic mastocytosis (ISM).
    - Ayvakit is not recommended for the treatment of patients with ISM 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 tumor (GIST) harboring a platelet-derived growth factor receptor alpha (PDGFRA) exon 18 mutation, including PDGFRA D842V mutations.
    - Advanced systemic mastocytosis (AdvSM). AdvSM includes patients with aggressive systemic mastocytosis, systemic mastocytosis with an associated hematological neoplasm, and mast cell leukemia.
- The approval of Ayvakit for the new indication was based on PIONEER, a randomized, double-blind, placebo-controlled study in 212 adult patients with ISM. Patients were randomized to receive Ayvakit with best supportive care vs. placebo with best supportive care. Efficacy was based on the absolute mean change from baseline to week 24 in the ISM-Symptom Assessment Form (ISM-SAF) total symptom score (TSS). The ISM-SAF is a patient-reported outcome measure assessing ISM signs and symptoms. The item scores were summed to calculate a daily ISM-SAF TSS (range 0 to 110), with higher scores indicating greater symptom severity.
    - The absolute mean change in the ISM-SAF TSS was -15.33 for Ayvakit vs. -9.64 with placebo (difference -5.69, 95% CI: -10.16, -1.23; p = 0.012).
- The most common adverse reactions (≥ 10%) with Ayvakit use in ISM were eye edema, dizziness, peripheral edema and flushing.
- The recommended dosage of Ayvakit is 25 mg orally once daily in patients with ISM.
    - Refer to the Ayvakit drug label for dosing for its other indications.