Pemazyre® (pemigatinib) – New indication

- On August 26, 2022, Incyte announced the FDA approval of Pemazyre (pemigatinib), for the treatment of adults with relapsed or refractory myeloid/lymphoid neoplasms (MLNs) with fibroblast growth factor receptor 1 (FGFR1) rearrangement.
- Pemazyre is also approved for the treatment of adults with previously treated, unresectable locally advanced or metastatic cholangiocarcinoma with a fibroblast growth factor receptor 2 (FGFR2) fusion or other rearrangement as detected by an FDA-approved test.
- MLNs with FGFR1 rearrangement are extremely rare and aggressive blood cancers that may impact less than 1 in 100,000 people in the U.S.
- The approval of Pemazyre for the new indication was based on FIGHT-203, an open-label, single-arm study in 28 patients with MLNs with FGFR1 rearrangement. Efficacy was established based on complete response (CR).
    - In patients with chronic phase in the marrow with or without extramedullary disease (EMD) (N = 18), the CR rate was 78% (95% CI: 52, 94). The median duration of CR was not reached (range, 1+ to 988+ days).
    - In patients with blast phase in the marrow with or without EMD (N = 4), two patients achieved a CR (duration: 1+ and 94 days).
    - In patients with EMD only (N = 3), 1 patient achieved a CR (duration: 64+ days).
    - For all patients (N = 28 including 3 patients without evidence of morphologic disease), the complete cytogenetic response rate was 79% (95% CI 59, 92).
- The most common adverse reactions (≥ 20%) with Pemazyre use were hyperphosphatemia, nail toxicity, alopecia, stomatitis, diarrhea, dry eye, fatigue, rash, abdominal pain, anemia, constipation, dry mouth, epistaxis, serous retinal detachment, extremity pain, decreased appetite, dry skin, dyspepsia, back pain, nausea, blurred vision, peripheral edema, and dizziness.
- The recommended dosage of Pemazyre for MLNs with FGFR1 rearrangement is 13.5 mg orally once daily on a continuous basis. Treatment should be continued until disease progression or unacceptable toxicity occurs.
    - Refer to the Pemazyre drug label for dosing for cholangiocarcinoma.