Vidaza® (azacitidine) – New indication

- On May 20, 2022, the FDA approved Celgene's Vidaza (azacitidine) injection for the treatment of pediatric patients aged 1 month and older with newly diagnosed juvenile myelomonocytic leukemia (JMML).

- Vidaza is also approved for the treatment of adult patients with the following French-American-British myelodysplastic syndrome subtypes: refractory anemia (RA) or RA with ringed sideroblasts (if accompanied by neutropenia or thrombocytopenia or requiring transfusions), RA with excess blasts, RA with excess blasts in transformation, and chronic myelomonocytic leukemia.

- Azacitidine is also available as an oral tablet (Onureg®) for continued treatment of adult patients with acute myeloid leukemia.

- The approval of Vidaza for the new indication was based on an open-label study to evaluate Vidaza prior to hematopoietic stem cell transplantation in 18 pediatric patients with JMML. The efficacy of Vidaza was established based on clinical complete remission (cCR) or clinical partial remission (cPR) at 3 months.

    — There was a total of 9 patients (95% CI: 26, 74) with a confirmed clinical response. Of these 9 patients, there were 3 cCR and 6 cPR.

- The most common adverse reactions (> 30%) with Vidaza use in pediatric patients with JMML were pyrexia, rash, upper respiratory tract infection, and anemia.

- The recommended dose of Vidaza for the treatment of JMML is administered as an intravenous infusion daily for 7 days in a 28-day cycle. Patients should be treated for a minimum of 3 cycles and maximum of 6 cycles. For pediatric patients aged 1 month to less than 1 year or weighing less than 10 kg, the recommended dose is 2.5 mg/kg. For patients aged 1 year and older and weighing 10 kg or greater, the dose is 75 mg/m^2.

    — Vidaza injection should not be substituted for oral azacitidine. The indications and dosing regimen for Vidaza differ from that of oral azacitidine
    — Refer to the Vidaza drug label for dosing for all its other indications.