Besponsa™ (inotuzumab ozogamicin) – Updated indication

- On March 6, 2024, the FDA approved Pfizer's Besponsa (inotuzumab ozogamicin), for the treatment of relapsed or refractory CD22-positive B-cell precursor acute lymphoblastic leukemia (ALL) in adult and pediatric patients 1 year and older.
    - Besponsa was previously approved for the treatment of adults with relapsed or refractory B-cell precursor ALL.
- The approval of Besponsa for the updated indication was based on a single-arm, open-label study in 53 pediatric patients ≥ 1 and < 18 years of age with relapsed or refractory CD22-positive B-cell precursor ALL. Efficacy was established on the basis of the complete remission (CR) rate, duration of CR, and proportion of patients with minimal residual disease (MRD) negative CR.
    - In all patients, 22/53 (42%, 95% CI: 28.1, 55.9) patients achieved CR, and the median duration of CR was 8.2 months (95% CI: 2.6, not estimable).
    - The MRD negativity rate in patients with CR was 21/22 (95.5%, 95% CI: 77.2, 99.9) based on flow cytometry, and 19/22 (86.4%, 95% CI: 65.1, 97.1) based on RQ-PCR.
- Besponsa carries a boxed warning for hepatotoxicity, including hepatic veno-occlusive disease; and increased risk of post-hematopoietic stem cell transplant (HSCT) non-relapse mortality.
- For the first cycle, the recommended total dose of Besponsa for all patients is 1.8 mg/m^2 per cycle, administered as 3 divided doses on day 1 (0.8 mg/m^2), Day 8 (0.5 mg/m^2), and day 15 (0.5 mg/m2). Cycle 1 is 3 weeks in duration but may be extended to 4 weeks if the patient achieves a CR or CR with incomplete hematologic recovery (CRi), and/or to allow recovery from toxicity. For subsequent cycles:
    - In patients who achieve a CR or CRi, the recommended total dose is 1.5 mg/m^2 per cycle, administered as 3 divided doses on day 1 (0.5 mg/m^2), day 8 (0.5 mg/m^2), and day 15 (0.5 mg/m^2). Subsequent cycles are 4 weeks in duration; or
    - In patients who do not achieve a CR or CRi, the recommended total dose is 1.8 mg/m^2 per cycle given as 3 divided doses on day 1 (0.8 mg/m^2), day 8 (0.5 mg/m^2), and day 15 (0.5 mg/m^2). Subsequent cycles are 4 weeks in duration. Patients who do not achieve a CR or CRi within 3 cycles should discontinue treatment.
- For patients proceeding to HSCT, the recommended duration of treatment with Besponsa is 2 cycles. A third cycle may be considered for those patients who do not achieve CR or CRi and minimal residual disease negativity after 2 cycles.
- For patients not proceeding to HSCT, additional cycles of treatment, up to a maximum of 6 cycles, may be administered.