Mylotarg™ (gemtuzumab ozogamicin) – Expanded indication

- On June 16, 2020, the FDA approved Pfizer's Mylotarg (gemtuzumab ozogamicin), for the treatment of newly-diagnosed CD33-positive acute myeloid leukemia (AML) in adults and pediatric patients 1 month and older.
    - Mylotarg was previously approved for this indication in adults only.
- Mylotarg is also approved for the treatment of relapsed or refractory CD33-positive AML in adults and in pediatric patients 2 years and older.
- The approval of Mylotarg for the expanded indication was based on study AAML0531, a randomized study of 1,063 patients with newly-diagnosed AML ages 0 to 29 years. Patients were randomized to 5-cycle chemotherapy alone or with Mylotarg. Overall, 94% of patients were < 18 years of age, and 6% were adults. Supportive evidence of efficacy was provided by event-free survival (EFS), measured from the date of study entry until induction failure, relapse, or death by any cause.
    - The EFS hazard ratio was 0.84 (95% CI: 0.71 to 0.99). The estimated percentage of patients free of induction failure, relapse, or death at five years was 48% (95% CI: 43, 52) in the Mylotarg + chemotherapy arm vs. 40% (95% CI: 36, 45) in the chemotherapy alone arm.
    - There was no difference between treatment arms in overall survival.
- Mylotarg carries a boxed warning for hepatotoxicity.
- The recommended intravenous dose of Mylotarg for the treatment of newly diagnosed CD33-positive AML in pediatric patients is 3 mg/m^2 for patients with body surface area (BSA) greater than or equal to 0.6 m^2 and 0.1 mg/kg for patients with BSA less than 0.6 m^2.
    - For Induction 1, Mylotarg is given once in combination with standard chemotherapy. No Mylotarg is given in the second induction cycle.
    - No Mylotarg is given in the first or third intensification cycles. For intensification 2, Mylotarg is given once in combination with standard chemotherapy. Consider the risks and potential benefits before giving Mylotarg during intensification 2.
    - Refer to the Mylotarg drug label for dosing for all its other indications.