

FDA Approves Mylotarg for Children With Acute Myeloid Leukemia

Mylotarg consists of an antibody that binds to the CD33 receptor protein on malignant B cells combined with a chemotherapy drug.

June 19, 2020 By [Food and Drug Administration \(FDA\)](#)

FDA approves gemtuzumab ozogamicin for CD33-positive AML in pediatric patients

On June 16, 2020, the Food and Drug Administration extended the indication of gemtuzumab ozogamicin (MYLOTARG, Wyeth Pharmaceuticals LLC) for newly-diagnosed CD33-positive acute myeloid leukemia (AML) to include pediatric patients 1 month and older.

Efficacy and safety in the pediatric population were supported by data from AAML0531 (NCT00372593), a multicenter 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 gemtuzumab ozogamicin (3 mg/m²) administered once on day 6 in Induction 1 and once on day 7 in Intensification 2.

The main efficacy outcome measure was event-free survival (EFS) measured from the date of trial entry until induction failure, relapse, or death by any cause. The EFS hazard ratio was 0.84 (95% CI: 0.71-0.99). The estimated percentage of patients free of induction failure, relapse, or death at five years was 48% (95% CI: 43%-52%) in the gemtuzumab ozogamicin + chemotherapy arm versus 40% (95% CI: 36%-45%) in the chemotherapy alone arm. No difference between treatment arms in overall survival was demonstrated.

The most common grade 3 and higher adverse reactions that occurred during Induction 1 and Intensification 2 in ≥ 5% of patients who received gemtuzumab ozogamicin were infection, febrile neutropenia, decreased appetite, hyperglycemia, mucositis, hypoxia, hemorrhage, increased transaminase, diarrhea, nausea, and hypotension.

[View full prescribing information for MYLOTARG.](#)

This application was granted priority review. A description of FDA expedited programs is in the [Guidance for Industry: Expedited Programs for Serious Conditions-Drugs and Biologics](#).

Healthcare professionals should report all serious adverse events suspected to be associated with

the use of any medicine and device to FDA's [MedWatch Reporting System](#) or by calling 1-800-FDA-1088.

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