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/m^2) 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.

This announcement was originally published on the Food and Drug Administration website on June 16, 2020.

© 2026 Smart + Strong All Rights Reserved.

http://beta.docker.cancerhealth.com/blog/fda-approves-mylotarg-children-acute-myeloid-leukemia