BlueCross BlueShield Federal Employee Program

MYLOTARG
(gemtuzumab ozogamicin)

RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Mylotarg (gemtuzumab ozogamicin) is a CD33-directed antibody-drug conjugate. The conjugate binds to CD33-expressing tumor cells and induces cell cycle arrest and apoptopic cell death. Mylotarg is indicated for the treatment of CD33-positive acute myeloid leukemia (AML) which is a rapidly progressing cancer that forms in the bone marrow and results in an increased number of abnormal white blood cells in the bloodstream and bone marrow (1).

Regulatory Status

FDA-approved indications: Mylotarg is a CD33-directed antibody-drug conjugate indicated for: (1)

1. Treatment of newly-diagnosed CD33-positive acute myeloid leukemia (AML) in adults and pediatric patients 1 month and older
2. Treatment of relapsed or refractory CD33-positive AML in adults and in pediatric patients 2 years and older

Mylotarg has a boxed warning for hepatotoxicity, including life-threatening and sometimes fatal hepatic VOD events, which have been reported in patients receiving Mylotarg as a single agent or as part of a combination chemotherapy regimen. It is recommended to assess ALT, AST, total bilirubin, and alkaline phosphatase prior to each dose of Mylotarg. Also, physicians should monitor for signs and symptoms of VOD; these may include elevations in ALT, AST, total bilirubin, hepatomegaly, rapid weight gain, and ascites (1).

The safety and effectiveness of Mylotarg in pediatric patients less than 1 month of age with newly-diagnosed de novo AML have not been established. The safety and effectiveness of Mylotarg as a single agent in pediatric patients less than 2 years of age with relapsed or refractory AML have not been established (1).

Summary

Mylotarg (gemtuzumab ozogamicin) is a CD33-directed antibody-drug conjugate indicated for the treatment of acute myeloid leukemia (AML). Mylotarg has a boxed warning for hepatotoxicity. The safety and effectiveness of Mylotarg in pediatric patients less than 1 month of age with newly-diagnosed de novo AML have not been established. The safety and effectiveness of Mylotarg as a

single agent in pediatric patients less than 2 years of age with relapsed or refractory AML have not been established (1).

Prior approval is required to ensure the safe, clinically appropriate, and cost-effective use of Mylotarg while maintaining optimal therapeutic outcomes.

References

1. Mylotarg [package insert]. Philadelphia, PA: Wyeth Pharmaceuticals Inc.; August 2021.
2. NCCN Drugs & Biologics Compendium® Gemtuzumab ozogamicin 2025. National Comprehensive Cancer Network, Inc. Accessed on January 9, 2025.