Mass General Brigham
Health Plan

Mylotarg (gemtuzumab ozogamicin)
Effective 07/01/2025

Plan	☒ MassHealth ☐Commercial/Exchange	**Program Type**	☒ Prior Authorization ☐ Quantity Limit ☐ Step Therapy
Benefit	☐ Pharmacy Benefit ☒ Medical Benefit		
Specialty Limitations	N/A		
Contact Information	**Medical Benefit** **Pharmacy Benefit**	Phone: 833-895-2611 Phone: 800-711-4555	Fax: 888-656-6671 Fax: 844-403-1029
Exceptions	N/A		

Overview
Mylotarg (gemtuzumab ozogamicin) is a CD33-directed antibody-drug conjugate (ADC) indicated for the treatment of newly-diagnosed CD33-positive acute myeloid leukemia (AML) in adults and pediatric patients one month and older (as combination therapy and as monotherapy) and the treatment of relapsed or refractory CD33-positive AML in adults and in pediatric patients two years and older (as monotherapy).

Coverage Guidelines
Authorization may be reviewed on a case by case basis for members who are new to the plan currently receiving treatment with requested medication excluding when the product is obtained as samples or via manufacturer's patient assistance programs.
OR
Authorization may be granted for members when all the following criteria are met:

Newly-diagnosed CD33-positive AML in adults and pediatric patients 1 month and older

1. Indication of newly diagnosed CD33-positive AML
2. Prescriber is an oncologist or hematologist or consult notes from an oncologist or hematologist are provided
3. Member ≥1 month of age
4. Appropriate dosing
5. **ONE** of the following:
    a. Requested agent will be used in combination with cytarabine and daunorubicin or fludarabine
    b. Clinical rationale why combination therapy with cytarabine and daunorubicin or fludarabine is not appropriate
    c. Member is ≥ 60 years of age

Relapsed or refractory CD33-positive AML in adults and in pediatric patients 2 years and older

1. Diagnosis of relapsed or refractory CD33-positive AML
2. Prescriber is an oncologist or hematologist or consult notes from an oncologist or hematologist are provided
3. Appropriate dosing

4. Member ≥2 years of age
5. **ONE** of the following:
    a. Relapsed or refractory AML
    b. Prior therapy for the treatment of AML with one systemic therapy *(refer to Appendix for common AML treatment regimens)*

Limitations

1. Initial approvals:
    a. Newly-diagnosed CD33-positive AML in adults and pediatric patients 1 month and older: **Three cycles**
    b. Relapsed or refractory CD33-positive AML in adults and in pediatric patients two years and older: **One treatment cycle**
2. Reauthorizations for newly-diagnosed CD33-positive AML in adults and pediatric patients 1 month and older for monotherapy: maximum of one cycle of induction and eight cycles of continuation.

Appendix

Common AML Treatment Regimens

Examples of Treatment Induction Regimens

- Patients <60 Years of Age
    - Cytarabine 1.5 to 3 g/m^2 every 12 hours X6 days
    - Standard-dose cytarabine with idarubicin or daunorubicin
    - Standard-dose cytarabine with daunorubicin or oral midostaurin (FLT3 mutated)
    - Dual drug liposomal encapsulation of cytarabine and daunorubicin
- Patients ≥60 Years of Age
    - Standard-dose cytarabine (100 to 200 mg/m^2 x seven days) with idarubicin 12 mg/m^2 or daunorubicin 60 to 90 mg/m^2 x three days or mitoxantrone 12 mg/m^2 x three days
    - Low-intensity therapies: azacytidine, decitabine
    - Dual-drug liposomal encapsulation of daunorubicin 44 mg/m^2 and cytarabine 100 mg/m2 on days one, three and five for one cycle (category 1)
    - Standard dose cytarabine 200 mg/m^2 x seven days with daunorubicin 60 mg/m^2 x three days and oral midostaurin 50 mg every 12 hours, days 8 to 21 (FLT3-mutated AML)
    - Venetoclax once daily by mouth and decitabine 20 mg/m^2 (days one to five of each 28 day cycle)
    - Venetoclax once daily by mouth and azacytidine 75 mg/m^2 (days one to seven of each 28-day cycle)
    - Venetoclax once daily by mouth and low-dose cytarabine 20 mg/m^2/day (days 1 to 10 of each 28-day cycle)
    - Standard-dose cytarabine 200 mg/m^2 x seven days with daunorubicin 60 mg/m^2 x three days and a single dose of gemtuzumab ozogamicin 3 mg/m^2 given on day one, or day two, or day three, or day four; alternatively, three total doses may be given on days one, four and seven (CD33-positive)

References

1. Mylotarg [prescribing information]. Phildelphia (PA): Wyeth Pharmaceticals, Inc (Pfizer); 2021 Sep.
2. FDA approves Mylotarg for treatment of acute myeloid leukemia [press release on the internet]. FDA; 2017 Sept 1 [cited 2021 Aug 31].

3. FDA: Pfizer Voluntarily Withdraws Cancer Treatment Mylotarg from U.S. Market [press release on the internet]. Fierce Pharma; 2010 Jun 21 [cited 2021 Aug 31]. Available from: http://www.fiercepharma.com/pharma/fda-pfizer-voluntarily-withdraws-cancer-treatment-mylotarg-from-u-s-market.
4. SEER Stat Fact Sheets: Acute Myeloid Leukemia (AML). http://seer.cancer.gov/statfacts/html/amyl.html. Accessed September 1, 2021.
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6. National Comprehensive Cancer Network. NCCN Clinical Practice Guidelines in Oncology Acute Myeloid Leukemia. Version 3.2021. 2021 Mar 2 [cited 2021 Sep 1]. Available from: https://www.nccn.org/professionals/physician_gls/pdf/aml.pdf.
7. Castaigne S, Pautas C, Terré C, Raffoux E, Bordessoule D, Bastie JN, et al. Effect of gemtuzumab ozogamicin on survival of adult patients with de-novo acute myeloid leukaemia (ALFA-0701): a randomised, open-label, phase 3 study. Lancet. 2012 Apr 21;379(9825):1508-16.
8. Taksin AL, Legrand O, Raffoux E, de Revel T, Thomas X, Contentin N, et al. High efficacy and safety profile of fractionated doses of Mylotarg as induction therapy in patients with relapsed acute myeloblastic leukemia: a prospective study of the alfa group. Leukemia. 2007 Jan;21(1):66-71. Epub 2006 Oct 19.

Review History

5/17/2022 – Created and Reviewed June P&T; matched MH UPPL. Created criteria to be in compliance with Masshealth criteria. Effective 8/1/22.
04/10/24 – Reviewed and updated for P&T. Mylotarg has been removed from pharmacy benefit and will be managed through medical benefit only. Expanded provider specialty to include consult notes. Effective 5/6/24
06/11/25 – Reviewed and updated for P&T. Part of annual UM review. Updated formatting and references. For newly diagnosed CD33-positive AML, criteria will require either combination therapy or clinical rationale why combination is not appropriate or if member is ≥ 60 years of age. Effective 7/1/25