# Drug Policy:

# Mylotarg™ (gemtuzumab ozogamicin)

| | | | |
|---|---|---|---|
| **POLICY NUMBER**<br>UM ONC_1325 | **SUBJECT**<br>Mylotarg™ (gemtuzumab ozogamicin) | | **DEPT/PROGRAM**<br>UM Dept | **PAGE 1 of 3** |
| **DATES COMMITTEE REVIEWED**<br>09/13/17, 09/21/18, 08/14/19, 12/11/19, 08/12/20, 08/11/21, 11/15/21, 05/11/22, 08/10/22, 12/14/22, 03/08/23, 05/10/23, 08/09/23, 08/14/24 | **APPROVAL DATE**<br>August 14, 2024 | **EFFECTIVE DATE**<br>August 30, 2024 | **COMMITTEE APPROVAL DATES**<br>09/13/17, 09/21/18, 08/14/19, 12/11/19, 08/12/20, 08/11/21, 11/15/21, 05/11/22, 08/10/22, 12/14/22, 03/08/23, 05/10/23, 08/09/23, 08/14/24 | |
| **PRIMARY BUSINESS OWNER:** UM | | **COMMITTEE/BOARD APPROVAL**<br>Utilization Management Committee | | |
| **NCQA STANDARDS**<br>UM 2 | | **ADDITIONAL AREAS OF IMPACT** | | |
| **CMS REQUIREMENTS** | **STATE/FEDERAL REQUIREMENTS** | | **APPLICABLE LINES OF BUSINESS**<br>Commercial, Exchange, Medicaid | |

## I. PURPOSE

To define and describe the accepted indications for Mylotarg (gemtuzumab ozogamicin) usage in the treatment of cancer, including FDA approved indications, and off-label indications.

Evolent is responsible for processing all medication requests from network ordering providers. Medications not authorized by Evolent may be deemed as not approvable and therefore not reimbursable.

The use of this drug must be supported by one of the following: FDA approved product labeling, CMS-approved compendia, National Comprehensive Cancer Network (NCCN), American Society of Clinical Oncology (ASCO) clinical guidelines, or peer-reviewed literature that meets the requirements of the CMS Medicare Benefit Policy Manual Chapter 15.

## II. INDICATIONS FOR USE/INCLUSION CRITERIA

### A. Continuation requests for a not-approvable medication shall be exempt from this Evolent policy provided:

1. The requested medication was used within the last year, AND
2. The member has not experienced disease progression and/or no intolerance to the requested medication, AND
3. Additional medication(s) are not being added to the continuation request.

### B. Acute Myeloid Leukemia (AML)

1. The member has CD33-positive AML and Mylotarg (gemtuzumab ozogamicin) is being used as a single agent OR in combination with chemotherapy for members with newly diagnosed AML (age 1 month and older) or for relapsed/refractory AML (age 2 years and older) who have not received Mylotarg (gemtuzumab ozogamicin) previously.

2. NOTE: The following regimens containing Mylotarg (gemtuzumab ozogamicin) are not supported by Evolent Policy:
    a. Induction therapy, less than 60 years of age: Fludarabine + HiDAC + idarubicin + G-CSF + gemtuzumab ozogamicin
    b. Induction/Consolidation therapy, greater than or equal to 60 years of age: Single agent Mylotarg (gemtuzumab ozogamicin).
    c. The above policy position is based on the lack of Level 1 Evidence (randomized clinical trials and/or meta-analyses) to show superior outcomes compared to Evolent recommended alternatives agents/regimens, including but not limited to regimens at http://pathways.newcenturyhealth.com.

## III. EXCLUSION CRITERIA

A. Disease progression on or following Mylotarg (gemtuzumab ozogamicin) or Mylotarg (gemtuzumab ozogamicin) containing regimen.

B. Dosing in adult members exceeds single dose limit of Mylotarg (gemtuzumab ozogamicin) combination therapy 3 mg/m$^2$ (max dose is 4.5 mg) or 6 mg/m$^2$ as single agent.

C. Dosing in pediatric members 1 month and older exceeds single dose limit of Mylotarg (gemtuzumab ozogamicin):
    1. 3 mg/m2 for pediatric members with body surface area (BSA) greater than or equal to 0.6 m2
    2. 0.1 mg/kg for pediatric members with BSA less than 0.6 m2

D. Investigational use of Mylotarg (gemtuzumab ozogamicin) with an off-label indication that is not sufficient in evidence or is not generally accepted by the medical community. Sufficient evidence that is not supported by CMS recognized compendia or acceptable peer reviewed literature is defined as any of the following:
    1. Whether the clinical characteristics of the patient and the cancer are adequately represented in the published evidence.
    2. Whether the administered chemotherapy/biologic therapy/immune therapy/targeted therapy/other oncologic therapy regimen is adequately represented in the published evidence.
    3. Whether the reported study outcomes represent clinically meaningful outcomes experienced by patients. Generally, the definitions of Clinically Meaningful outcomes are those recommended by ASCO, e.g., Hazard Ratio of less than 0.80 and the recommended survival benefit for OS and PFS should be at least 3 months.
    4. Whether the experimental design, considering the drugs and conditions under investigation, is appropriate to address the investigative question. (For example, in some clinical studies, it may be unnecessary or not feasible to use randomization, double blind trials, placebos, or crossover).
    5. That non-randomized clinical trials with a significant number of subjects may be a basis for supportive clinical evidence for determining accepted uses of drugs.
    6. That case reports are generally considered uncontrolled and anecdotal information and do not provide adequate supportive clinical evidence for determining accepted uses of drugs.
    7. That abstracts (including meeting abstracts) without the full article from the approved peer-reviewed journals lack supporting clinical evidence for determining accepted uses of drugs.

## IV. MEDICATION MANAGEMENT

A. Please refer to the FDA label/package insert for details regarding these topics.

## V. APPROVAL AUTHORITY

A. Review – Utilization Management Department

B. Final Approval – Utilization Management Committee

## VI. ATTACHMENTS

A. None

## VII. REFERENCES


A. Mylotarg prescribing information. Pfizer Inc, Philadelphia, PA 2021.

B. Lambert J, et al. Gemtuzumab ozogamicin for *de novo* acute myeloid leukemia: final efficacy and safety updates from the open-label, phase III ALFA-0701 trial. Haematologica. 2019 Jan;104(1):113-119. doi: 10.3324/haematol.2018.188888

C. Gamis AS, et al. Gemtuzumab ozogamicin in children and adolescents with de novo acute myeloid leukemia improves event-free survival by reducing relapse risk: results from the randomized phase III Children's Oncology Group trial AAML0531. J Clin Oncol. 2014 Sep 20;32(27):3021-32. doi: 10.1200/JCO.2014.55.3628

D. Clinical Pharmacology Elsevier Gold Standard 2024.

E. Micromedex® Healthcare Series: Micromedex Drugdex Ann Arbor, Michigan 2024

F. National Comprehensive Cancer Network. Cancer Guidelines and Drugs and Biologics Compendium 2024.

G. AHFS Drug Information. American Society of Health-Systems Pharmacists or Wolters Kluwer Lexi-Drugs. Bethesda, MD 2024.

H. Ellis LM, et al. American Society of Clinical Oncology perspective: Raising the bar for clinical trials by defining clinically meaningful outcomes. J Clin Oncol. 2014 Apr 20;32(12):1277-80.

I. Medicare Benefit Policy Manual Chapter 15 Covered Medical and Other Health Services: https://www.cms.gov/Regulations-and-Guidance/Guidance/Manuals/Downloads/bp102c15.pdf.