Evolent Clinical Guideline 3166 for Mylotarg™ (gemtuzumab ozogamicin)

Guideline Number: Evolent_CG_3166	**Applicable Codes**	
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STATEMENT

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.

INDICATIONS

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

- The member has not experienced disease progression on the requested medication AND
- The requested medication was used within the last year without a lapse of more than 30 days of having an active authorization AND
- Additional medication(s) are not being added to the continuation request.

Acute Myeloid Leukemia (AML)

- 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.
- NOTE: The following regimens containing Mylotarg (gemtuzumab ozogamicin) are not supported by Evolent Policy:
    - Induction therapy, less than 60 years of age: Fludarabine + HiDAC + idarubicin + G-CSF + gemtuzumab ozogamicin
    - Induction/Consolidation therapy, greater than or equal to 60 years of age: Single agent Mylotarg (gemtuzumab ozogamicin).
    - 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 **Evolent Pathways.**

CONTRAINDICATIONS/WARNINGS

- Contraindications
  - Hypersensitivity to gemtuzumab ozogamicin or any component of the formulation.
- US Boxed Warning
  - Hepatotoxicity, including severe or fatal hepatic veno-occlusive disease (VOD), also known as sinusoidal obstruction syndrome (SOS), has been reported in association with the use of gemtuzumab ozogamicin as a single agent, and as part of a combination chemotherapy regimen. Monitor frequently for signs and symptoms of VOD after treatment with gemtuzumab ozogamicin.

EXCLUSION CRITERIA

- Disease progression on or following Mylotarg (gemtuzumab ozogamicin) or Mylotarg (gemtuzumab ozogamicin) containing regimen.
- 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.
- Dosing in pediatric members 1 month and older exceeds single dose limit of Mylotarg (gemtuzumab ozogamicin):
  - 3 mg/m^2 for pediatric members with body surface area (BSA) greater than or equal to 0.6 m^2
  - 0.1 mg/kg for pediatric members with BSA less than 0.6 m^2
- 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:
  - Whether the clinical characteristics of the patient and the cancer are adequately represented in the published evidence.
  - Whether the administered chemotherapy/biologic therapy/immune therapy/targeted therapy/other oncologic therapy regimen is adequately represented in the published evidence.
  - 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.
  - 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).

- 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.
- That case reports are generally considered uncontrolled and anecdotal information and do not provide adequate supportive clinical evidence for determining accepted uses of drugs.
- 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.

CODING AND STANDARDS

Codes

- J9203 - Injection, gemtuzumab ozogamicin, 0.1 mg

Applicable Lines of Business

☐	CHIP (Children's Health Insurance Program)
☒	Commercial
☒	Exchange/Marketplace
☒	Medicaid
☐	Medicare Advantage

POLICY HISTORY

Date	Summary
August 2026	• Updated references
August 2025	• Converted to new Evolent guideline template • This guideline replaces UM ONC_1325 Mylotarg (gemtuzumab ozogamicin) • Updated indication section • Updated references
August 2024	• Updated dosing limits in exclusion criteria • Added new references • Updated NCH verbiage to Evolent

LEGAL AND COMPLIANCE

Guideline Approval

Committee

Reviewed / Approved by Evolent Specialty Services Clinical Guideline Review Committee

Disclaimer

Evolent Clinical Guidelines do not constitute medical advice. Treating health care professionals are solely responsible for diagnosis, treatment, and medical advice. Evolent uses Clinical Guidelines in accordance with its contractual obligations to provide utilization management. Coverage for services varies for individual members according to the terms of their health care coverage or government program. Individual members' health care coverage may not utilize some Evolent Clinical Guidelines. Evolent clinical guidelines contain guidance that requires prior authorization and service limitations. A list of procedure codes, services or drugs may not be all inclusive and does not imply that a service or drug is a covered or non-covered service or drug. Evolent reserves the right to review and update this Clinical Guideline in its sole discretion. Notice of any changes shall be provided as required by applicable provider agreements and laws or regulations. Members should contact their Plan customer service representative for specific coverage information.

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