Clinical Policy: Mercaptopurine (Purixan)

Reference Number: PA.CP.PHAR.447
Effective Date: 01/2020
Last Review Date: 04/2025

Description
Mercaptopurine (Purixan®) is a nucleoside metabolic inhibitor that is an analogue of the purine bases adenine and hypoxanthine.

FDA Approved Indication(s)
Purixan is indicated for the treatment of patients with acute lymphoblastic leukemia (ALL) as part of a combination chemotherapy maintenance regimen.

Policy/Criteria
Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

It is the policy of PA Health & Wellness® that Purixan is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria z

A. Acute Lymphoblastic Leukemia or Acute Myeloid Leukemia (must meet all)**:**

1. Diagnosis of ALL or acute promyelocytic leukemia;
2. Prescribed by or in consultation with an oncologist or hematologist;
3. Member must use mercaptopurine tablets, unless one of the following (a, b or c):
   a. Mercaptopurine tablets are contraindicated or clinically significant adverse effects are experienced;
   b. Member has a documented swallowing disorder or an inability to swallow tablets or capsules;
   c. Request is for Stage IV or metastatic cancer;
4. Request meets one of the following (a or b):
   a. Dose does not exceed 2.5 mg/kg or 75 mg/m^2 per day;
   b. Dose is supported by practice guidelines or peer-reviewed literature for the relevant off-label use (*prescriber must submit supporting evidence*).

Approval duration: 6 months

B. Other diagnoses/indications

1. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53

II. Continued Therapy

A. Acute Lymphoblastic Leukemia or Acute Myeloid Leukemia (must meet all)**:**

1. Currently receiving medication via PA Health & Wellness benefit and documentation supports positive response to therapy or the Continuity of Care policy (PA.PHARM.01) applies;
2. Member is responding positively to therapy;

3. If request is for a dose increase, request meets one of the following (a or b):
   a. New dose does not exceed 2.5 mg/kg or 75 mg/m^2 per day;
   b. New dose is supported by practice guidelines or peer-reviewed literature for the relevant off-label use (*prescriber must submit supporting evidence*).

Approval duration: 12 months

B. Other diagnoses/indications (must meet 1 or 2)**:**
1. Currently receiving medication via PA Health & Wellness benefit and documentation supports positive response to therapy or the Continuity of Care policy (PA. PHARM.01) applies.
   Approval duration: Duration of request or 6 months (whichever is less); or
2. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53

III. Diagnoses/Indications for which coverage is NOT authorized:

A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policy – PA.CP.PMN.53

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key
ALL: acute lymphoblastic leukemia
FDA: Food and Drug Administration
NCCN: National Comprehensive Cancer Network

Appendix B: Therapeutic Alternatives
This table provides a listing of preferred alternative therapy recommended in the approval criteria. The drugs listed here may not be a formulary agent and may require prior authorization.

Drug	Dosing Regimen	Dose Limit/Maximum Dose
mercaptopurine (Purinethol®)	1.5 to 2.5 mg/kg (50 to 75 mg/m^2) PO QD	Dose should be adjusted to maintain an absolute neutrophil count (ANC) at a desirable level

Therapeutic alternatives are listed as Brand name® (generic) when the drug is available by brand name only and generic (Brand name®) when the drug is available by both brand and generic.

Appendix C: Contraindications/Boxed Warnings
None reported

Appendix D: General Information
- Typical maintenance therapy regimen consists of daily 6-mercaptopurine, weekly methotrexate, and monthly vincristine/prednisone pulses for 2-3 years.
- Oral mercaptopurine can have highly variable drug and metabolite concentrations as many factors (e.g. thiopurine S-methyl transferase (TPMT) polymorphisms and drug-drug-interactions with other chemotherapeutic agents) can affect bioavailability and impact the ability of maintenance regimens to prevent disease relapse.

- Mercaptopurine dose adjustments may be needed to manage clinically significant adverse effects (e.g. myelosuppression including anemia, neutropenia, lymphopenia and thrombocytopenia). Mercaptopurine oral suspension may be more amendable to dose adjustments in patients who continue to have poor clinical response despite dose adjustments with the tablet form.
- Micromedex lists mercaptopurine for Crohn's disease as a Class I recommendation for adults and Class Ia for pediatrics. Ulcerative colitis has a Class IIb recommendation for both adult and pediatrics.
- NCCN treatment guidelines for ALL state that lymphoblastic lymphoma is indistinguishable from ALL based on morphologic, genetic, and immunophenotypic features. Patients with lymphoblastic lymphoma generally benefit from treatment with ALL-like regimens.

V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
ALL	1.5 to 2.5 mg/kg (50 to 75 mg/m^2) PO QD	2.5 mg/kg/day or 75 mg/m^2/day

VI. Product Availability

Oral suspension: 2000 mg/100 mL (20 mg/mL)

VII. References

1. Purixan Prescribing Information. Leicester, UK: Nova Laboratories Ltd; October 2024. Available at: https://www.accessdata.fda.gov/drugsatfda_docs/label/2024/205919s007lbl.pdf.. Accessed January 17, 2025.
2. National Comprehensive Cancer Network Drugs and Biologics Compendium. Available at: http://www.nccn.org/professionals/drug_compendium. Accessed February 10, 2025.
3. DRUGDEX® System [Internet database]. Greenwood Village, Colo: Thomson Healthcare. Updated periodically. Accessed February 10, 2025.
4. National Comprehensive Cancer Network. Acute Lymphoblastic Leukemia Version 3.2024. Available at: https://www.nccn.org/professionals/physician_gls/pdf/all.pdf. Accessed February 10, 2025.
5. National Comprehensive Cancer Network. Pediatric Acute Lymphoblastic Leukemia Version 2.2025. Available at: https://www.nccn.org/professionals/physician_gls/pdf/ped_all.pdf. Accessed February 10, 2025.
6. National Comprehensive Cancer Network. Acute Myeloid Leukemia Version 2.2025. Available at: https://www.nccn.org/professionals/physician_gls/pdf/aml.pdf. Accessed February 10, 2025.

Reviews, Revisions, and Approvals	Date
New Policy Created	01/2020
1Q 2021 annual review: no significant changes; references reviewed and updated.	01/2021
2Q 2021 annual review: corrected dosing typo; references reviewed and updated.	04/2021

Reviews, Revisions, and Approvals	Date
2Q 2022 annual review: modified redirection language from "medical justification" to "member must use"; references reviewed and updated.	04/2022
2Q 2023 annual review: added by-passing of redirection not allow step therapy in certain oncology settings; references reviewed and updated.	04/2023
2Q 2024 annual review: no significant changes; references reviewed and updated.	04/2024
2Q 2025 annual review: no significant changes; references reviewed and updated.	04/2025