Clinical Policy: Vincristine Sulfate Liposome Injection (Marqibo)

Reference Number: PA.CP.PHAR.315
Effective Date: 01/2018
Last Review Date: 10/2023

Coding Implications
Revision Log

Description
Vincristine sulfate liposome injection (Marqibo®) is a vinca alkaloid.

FDA Approved Indication(s)
Marqibo is indicated for the treatment of adult patients with Philadelphia chromosome-negative (Ph-) acute lymphoblastic leukemia (ALL) in second or greater relapse or whose disease has progressed following two or more anti-leukemia therapies.

This indication is approved under accelerated approval based on overall response rate. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.

* On May 2, 2022, the FDA withdrew approval of Marqibo after a postmarking clinical trial failed to verify the clinical benefit of the drug. Updated NCCN guidance (Acute Lymphoblastic Leukemia v2.2023) does not support usage.

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

I. Initial Approval Criteria

A. Acute Lymphoblastic Leukemia (must meet all):

1. Authorization is not permitted due to lack of FDA and NCCN support. Member may not initiate therapy with Marqibo. If member is currently using Marqibo proceed to Section II. A. Acute Lymphoblastic Leukemia for continued therapy criteria (*see Appendix D*).

Approval duration: Not applicable

B. Other diagnoses/indications: Refer to PA.CP.PMN.53

II. Continued Approval

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

1. Currently receiving medication via PA Health & Wellness benefit or member is currently receiving the medication for the treatment of acute lymphoblastic leukemiaor the Continuity of Care policy (PA.LTSS.PHAR.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 2.25 mg/m^2 every 7 days;
   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.LTSS.PHAR.01) applies; or
2. Refer to 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 policies – PA.CP.PMN.53;
- **B.** Patients with the demyelinating form of Charcot-Marie-Tooth syndrome.

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
Not Applicable

Appendix C: Contraindications/Boxed Warnings
- Contraindication(s):
  - Patients with demyelinating conditions including Charcot-Marie-Tooth syndrome
  - Hypersensitivity to vincristine sulfate or any of the other components of Marqibo (vinCRIStine sulfate LIPOSOME injection)
  - Intrathecal administration
- Boxed warning(s): for intravenous use only – fatal if given by other routes; dosage recommendations differ from vincristine sulfate, verify drug name and dose to avoid overdosage

Appendix D: General Information
- On May 2, 2022, the FDA withdrew approval of Marqibo after a postmarking clinical trial failed to verify the clinical benefit of the drug. The manufacturer voluntarily withdrew its new drug application and drug approval was subsequently withdrawn.
- The NCCN no longer recommends Marqibo per its Acute Lymphoblastic Leukemia Guidelines Version 2.2023.

V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
ALL (off-label)	2.25 mg/m^2 IV over 1 hour once every 7 days	See dosing regimen

VI. Product Availability

Marqibo Kit containing the following:

- Vial: vincristine sulfate injection, USP 5 mg/5 mL (1 mg/mL)
- Vial: sphingomyelin/cholesterol liposome injection 103 mg/mL
- Vial: sodium phosphate injection 355 mg/25 mL (14.2 mg/mL)

VII. References

1. Marqibo Prescribing Information. East Windsor, NJ: Acrotech Biopharma, LLC; March 2022. https://www.accessdata.fda.gov/drugsatfda_docs/label/2022/202497Orig1s013lbl.pdf . Accessed July 7, 2023..
2. National Comprehensive Cancer Network Drugs and Biologics Compendium. Available at https://www.nccn.org/professionals/drug_compendium/content/. Accessed August 8, 2023.
3. National Comprehensive Cancer Network. Acute Lymphoblastic Leukemia Version 2.2023. Available at: https://www.nccn.org/professionals/physician_gls/pdf/all.pdf. Accessed August 8, 2023.
4. Food and Drug Administration, HHS. Acrotech Biopharm LLC; Withdrawal of approval of new drug application for marqibo (vincristine sulfate liposome injection), 5 miligrams/ 5 milliliters. Fderal Register. May 2, 2022. Available at https://www.federalregister.gov/documents/2022/05/02/2022-09235/acrotech-biopharma-llc-withdrawal-of-approval-of-new-drug-application-for-marqibo-vincristine. Accessed August 8, 2023.

Coding Implications

Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
J9371	Injection, vincristine sulfate liposome, 1 mg

Reviews, Revisions, and Approvals	Date	Approval Date
4Q 2018 annual review: no significant changes; added prescriber restrictions; references reviewed and updated.	07/2018	
4Q 2019 annual review: No changes per Statewide PDL implementation 01-01-2020	10/2019	
4Q 2020 annual review Ph- anti-leukemia therapy examples added to Appendix B; FDA/NCCN dosing limitation added; references reviewed and updated.	10/2020	
4Q 2021 annual review: added requirement for use as a single agent per NCCN and pivotal trial; updated Appendix C to include hypersensitivity contraindication; references reviewed and updated.	10/2021	

Reviews, Revisions, and Approvals	Date	Approval Date
4Q 2022 annual review: changed to off-label usage for ALL due to FDA withdrawal but still supported by NCCN; references reviewed and updated.	10/2022	
4Q 2023 annual review: removed initial approval criteria for ALL as use is not supported by the FDA and NCCN; removed Appendix B table; updated Appendix D with NCCN reference; references reviewed and updated.	10/2023	