

# Clinical Policy: Allogeneic Hematopoietic Stem-Cell Transplantation for Myelodysplastic Syndromes & Myeloproliferative Neoplasms

Reference Number: WNC.CP.244

Last Review Date: 11/2025

Coding Implications

Revision Log

See Important Reminder at the end of this policy for important regulatory and legal information.

**Note:** When state Medicaid coverage provisions conflict with the coverage provisions in this clinical policy, state Medicaid coverage provisions take precedence. Please refer to the state Medicaid manual for any coverage provisions pertaining to this clinical policy.

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## Description<sup>1</sup>

This policy describes the medical necessity criteria for Allogeneic Stem-Cell Transplantation for Myelodysplastic Syndromes & Myeloproliferative Neoplasms.

## Policy/Criteria<sup>1</sup>

- I. WellCare of North Carolina<sup>®</sup> shall cover allogeneic hematopoietic stem-cell transplantation (HSCT) for the treatment of myelodysplastic syndromes and myeloproliferative neoplasms;
- II. WellCare of North Carolina<sup>®</sup> shall cover reduced-intensity conditioning (RIC) allogeneic HSCT for the treatment of myelodysplastic syndromes and myeloproliferative neoplasm for members who for medical reasons would be unable to tolerate a myeloablative conditioning regime.
- III. Donor lymphocyte infusion is considered medically necessary and, therefore, covered following allogeneic HSCT that is medically necessary for the treatment of myelodysplastic syndromes that have relapsed or are refractory, to prevent relapse in the setting of a high risk of relapse, or to convert an individual from mixed to full donor chimerism.
- IV. WellCare of North Carolina<sup>®</sup> **shall not** cover allogeneic HSCT for myelodysplastic syndromes and myeloproliferative neoplasms when the criteria and guidelines outlined in Sections I-III of this policy are not met.

**CLINICAL POLICY WNC.CP.244****ALLOGENEIC HEMATOPOIETIC STEM-CELL TRANSPLANTATION FOR  
MYELODYSPLASTIC SYNDROMES & MYELOPROLIFERATIVE NEOPLASMS****Background<sup>1</sup>**

Hematopoietic stem-cell transplantation refers to a procedure in which hematopoietic stem cells are infused to restore bone marrow function in cancer patients who receive bone marrow-toxic doses of cytotoxic drugs with or without whole body radiation therapy. Hematopoietic stem cells may be obtained from the transplant recipient (autologous HSCT) or from a donor (allogeneic HSCT). They can be harvested from bone marrow, peripheral blood, or umbilical cord blood shortly after delivery of neonates. Although cord blood is an allogeneic source, the stem cells in it are antigenically naïve and thus are associated with a lower incidence of rejection or graft-versus-host disease (GVHD).

Immunologic compatibility between infused hematopoietic stem cells and the recipient is not an issue in autologous HSCT. However, immunologic compatibility between donor and patient is a critical factor for achieving a good outcome of allogeneic HSCT. Compatibility is established by typing of human leukocyte antigens (HLA) using cellular, serologic, or molecular techniques. HLA refers to the tissue type expressed at the HLA A, B, and DR loci on each arm of chromosome 6. Depending on the disease being treated, an acceptable donor will match the patient at all or most of the HLA loci.

**Myelodysplastic syndromes** refer to a heterogeneous group of clonal hematopoietic disorders characterized by impaired maturation of hematopoietic cells and a tendency to transform into acute myelocytic leukemia (AML). MDS can occur as a primary (idiopathic) disease, or be secondary to cytotoxic therapy, ionizing radiation, or other environmental insult. Chromosomal abnormalities are seen in 40%–60% of patients, frequently involving deletions of chromosome 5 or 7, or an extra chromosome as in trisomy 8. Signs and symptoms of anemia, often complicated by infections or bleeding, are common in MDS; some patients exhibit systemic symptoms or features of autoimmunity that may be indicative of their disease pathogenesis. The vast majority of MDS diagnoses occur in individuals over the age of 55–60 years, with an age adjusted incidence of about 62% among individuals over age 70 years. Patients either succumb to disease progression to AML or to complications of pancytopenia. Patients with higher blast counts or complex cytogenetic abnormalities have a greater likelihood of progressing to AML than do other patients.

The French American-British (FAB) system has been used to classify MDS into 5 subtypes as follows: 1) refractory anemia (RA); 2) refractory anemia with ringed sideroblasts (RARS); 3) refractory anemia with excess blasts (RAEB); 4) refractory anemia with excess blasts in transformation (RAEBT); and 5) chronic myelomonocytic leukemia (CMML). The FAB system has been supplanted by that of the World Health Organization (WHO), which records the number of lineages in which dysplasia is seen (unilineage versus multilineage), separates the 5q-syndrome, and reduces the threshold maximum blast percentage for the diagnosis of MDS from 30% to 20% (see Policy Guidelines for WHO classification scheme for myeloid neoplasms).

Several prognostic scoring systems for MDS have been proposed; the most commonly used is the International Prognostic Scoring System (IPSS). The IPSS groups patients into one of four

**CLINICAL POLICY WNC.CP.244****ALLOGENEIC HEMATOPOIETIC STEM-CELL TRANSPLANTATION FOR  
MYELODYSPLASTIC SYNDROMES & MYELOPROLIFERATIVE NEOPLASMS**

prognostic categories based on the number of cytopenias, cytogenetic profile and the percentage blasts in the bone marrow (see Policy Guidelines). This system underweights the clinical importance of severe, life-threatening neutropenia and thrombocytopenia in therapeutic decisions and does not account for the rate of change in critical parameters, such as peripheral blood counts or blast percentage. However, the IPSS has been useful in comparative analysis of clinical trial results and its utility confirmed at many institutions. A second prognostic scoring system incorporates the WHO subgroup classification that accounts for blast percentage, cytogenetics, and severity of cytopenias as assessed by transfusion requirements. The WPSS uses a 6-category system which allows more precise prognostication of overall survival duration as well as risk for progression to AML. This system, however, is not yet in widespread use in clinical trials.

Treatment of smoldering or non-progressing MDS has in the past involved best supportive care including red blood cell (RBC) and platelet transfusions and antibiotics. Active therapy was given only when MDS progressed to AML or resembled AML with severe cytopenias. A diverse array of therapies are now available to treat MDS, including hematopoietic growth factors (e.g., erythropoietin, darbepoetin, granulocyte colony-stimulating factor), transcriptional-modifying therapy (e.g., U.S. Food and Drug Administration-approved hypomethylating agents, nonapproved histone deacetylase inhibitors), immunomodulators (e.g., lenalidomide, thalidomide, antithymocyte globulin, cyclosporine A), low-dose chemotherapy (e.g., cytarabine), and allogeneic HSCT. Given the spectrum of treatments available, the goal of therapy must be decided upfront, whether it is to improve anemia, thrombocytopenia, or neutropenia; eliminate the need for RBC transfusion; achieve complete remission (CR); or cure the disease. Allogeneic HSCT is the only approach with curative potential, but its use is governed by patient age, performance status, medical comorbidities, the patient's risk preference, and severity of MDS at presentation.

**Myeloproliferative Neoplasms** are characterized by the slow but relentless expansion of a clone of cells with the potential evolution into a blast crisis similar to AML. They share a common stem cell-derived clonal heritage, with phenotypic diversity attributed to abnormal variations in signal transduction as the result of a spectrum of mutations that affect protein tyrosine kinases or related molecules. The unifying characteristic common to all MPNs is effective clonal myeloproliferation resulting in peripheral granulocytosis, thrombocytosis, or erythrocytosis that is devoid of dyserythropoiesis, granulocytic dysplasia, or monocytosis.

As a group, about 8,400 MPNs are diagnosed annually in the U.S. Like MDS, MPNs occur primarily in older individuals, with about 67% reported in patients aged 60 years and older. In indolent, non-progressing cases, therapeutic approaches are based on relief of symptoms. Myeloablative allogeneic HSCT has been considered the only potentially curative therapy, but because most patients are of advanced age with attendant comorbidities, its use is limited to those who can tolerate the often-severe treatment-related adverse effects of this procedure. However, the use of RIC regimens for allogeneic HSCT has extended the potential benefits of this procedure to selected individuals with these disorders.

## CLINICAL POLICY WNC.CP.244

# ALLOGENEIC HEMATOPOIETIC STEM-CELL TRANSPLANTATION FOR MYELOYDYSPLASTIC SYNDROMES & MYELOPROLIFERATIVE NEOPLASMS

**Donor Lymphocyte Infusion** is a type of therapy in which lymphocytes from the blood of a donor are given to a member who has already received a stem cell transplant from the same donor. The donor lymphocytes may kill remaining cancer cells.

### **Coding Implications**

This clinical policy references Current Procedural Terminology (CPT®). CPT® is a registered trademark of the American Medical Association. All CPT codes and descriptions are copyrighted 2025, American Medical Association. All rights reserved. CPT codes and CPT descriptions are from the current manuals and those included herein are not intended to be all-inclusive and are included for informational purposes only. 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.

Reviews, Revisions, and Approvals	Reviewed Date	Approval Date
Original approval date	04/21	05/21
Reviewed CPT codes.	12/21	02/22
Annual Review. Added “Hematopoietic” to title	11/22	11/22
NCHC verbiage removed from NC Guidance Verbiage.	04/23	04/23
Annual Review. CPT codes removed.	11/23	11/23
Annual Review. Changed ‘beneficiary’ to member. Removed ‘Medicaid and health choice’ verbiage from References.	11/24	11/24
Annual Review. Under NC Guidance/Claims related information, updated state web address.	11/25	11/25

### **References**

1. State of North Carolina Medicaid Clinical Coverage Policy No: 11A-9 Allogeneic Hematopoietic Stem-Cell Transplantation for Myelodysplastic Syndromes & Myeloproliferative Neoplasms. [Program Specific Clinical Coverage Policies | NC Medicaid \(ncdhhs.gov\)](https://www.ncdhhs.gov/Program-Specific-Clinical-Coverage-Policies/NC-Medicaid). Published August 15, 2023. Accessed July 2, 2025.

### **North Carolina Guidance**

#### *Eligibility Requirements*

- a. An eligible beneficiary shall be enrolled in the NC Medicaid Program (Medicaid is NC Medicaid program, unless context clearly indicates otherwise);
- b. Provider(s) shall verify each Medicaid beneficiary’s eligibility each time a service is rendered.
- c. The Medicaid beneficiary may have service restrictions due to their eligibility category that would make them ineligible for this service.

## CLINICAL POLICY WNC.CP.244

### ALLOGENEIC HEMATOPOIETIC STEM-CELL TRANSPLANTATION FOR MYELODYSPLASTIC SYNDROMES & MYELOPROLIFERATIVE NEOPLASMS

#### *EPSDT Special Provision: Exception to Policy Limitations for a Medicaid Beneficiary under 21 Years of Age*

- a. 42 U.S.C. § 1396d(r) [1905(r) of the Social Security Act]  
Early and Periodic Screening, Diagnostic, and Treatment (EPSDT) is a federal Medicaid requirement that requires the state Medicaid agency to cover services, products, or procedures for Medicaid beneficiary under 21 years of age if the service is medically necessary health care to correct or ameliorate a defect, physical or mental illness, or a condition [health problem] identified through a screening examination (includes any evaluation by a physician or other licensed practitioner).

This means EPSDT covers most of the medical or remedial care a child needs to improve or maintain his or her health in the best condition possible, compensate for a health problem, prevent it from worsening, or prevent the development of additional health problems.

Medically necessary services will be provided in the most economic mode, as long as the treatment made available is similarly efficacious to the service requested by the beneficiary's physician, therapist, or other licensed practitioner; the determination process does not delay the delivery of the needed service; and the determination does not limit the beneficiary's right to a free choice of providers.

EPSDT does not require the state Medicaid agency to provide any service, product, or procedure:

1. that is unsafe, ineffective, or experimental or investigational.
2. that is not medical in nature or not generally recognized as an accepted method of medical practice or treatment.

Service limitations on scope, amount, duration, frequency, location of service, and other specific criteria described in clinical coverage policies may be exceeded or may not apply as long as the provider's documentation shows that the requested service is medically necessary "to correct or ameliorate a defect, physical or mental illness, or a condition" [health problem]; that is, provider documentation shows how the service, product, or procedure meets all EPSDT criteria, including to correct or improve or maintain the beneficiary's health in the best condition possible, compensate for a health problem, prevent it from worsening, or prevent the development of additional health problems.

#### **EPSDT and Prior Approval Requirements**

1. If the service, product, or procedure requires prior approval, the fact that the beneficiary is under 21 years of age does NOT eliminate the requirement for prior approval.
2. **IMPORTANT ADDITIONAL INFORMATION** about EPSDT and prior approval is found in the *NCTracks Provider Claims and Billing Assistance Guide*, and on the EPSDT provider page. The Web addresses are specified below:

*NCTracks Provider Claims and Billing Assistance Guide:*

<https://www.nctracks.nc.gov/content/public/providers/provider-manuals.html>

*EPSDT provider page:* <https://medicaid.ncdhhs.gov/>

**CLINICAL POLICY WNC.CP.244****ALLOGENEIC HEMATOPOIETIC STEM-CELL TRANSPLANTATION FOR  
MYELODYSPLASTIC SYNDROMES & MYELOPROLIFERATIVE NEOPLASMS***Provider(s) Eligible to Bill for the Procedure, Product, or Service*

To be eligible to bill for the procedure, product, or service related to this policy, the provider(s) shall:

- a. meet Medicaid qualifications for participation;
- b. have a current and signed Department of Health and Human Services (DHHS) Provider Administrative Participation Agreement; and
- c. bill only for procedures, products, and services that are within the scope of their clinical practice, as defined by the appropriate licensing entity.

*Compliance*

Provider(s) shall comply with the following in effect at the time the service is rendered:

- a. All applicable agreements, federal, state, and local laws and regulations including the Health Insurance Portability and Accountability Act (HIPAA) and record retention requirements; and
- b. All NC Medicaid's clinical (medical) coverage policies, guidelines, policies, provider manuals, implementation updates, and bulletins published by the Centers for Medicare and Medicaid Services (CMS), DHHS, DHHS division(s) or fiscal contractor(s).

*Claims-Related Information*

Provider(s) shall comply with the NC Tracks Provider Claims and Billing Assistance Guide, Medicaid bulletins, fee schedules, NC Medicaid's clinical coverage policies and any other relevant documents for specific coverage and reimbursement for Medicaid:

- a. Claim Type - as applicable to the service provided:  
Professional (CMS-1500/837P transaction)  
Institutional (UB-04/837I transaction)  
Unless directed otherwise, Institutional Claims must be billed according to the National Uniform Billing Guidelines. All claims must comply with National Coding Guidelines.
- b. International Classification of Diseases and Related Health Problems, Tenth Revisions, Clinical Modification (ICD-10-CM) and Procedural Coding System (PCS) - Provider(s) shall report the ICD-10-CM and Procedural Coding System (PCS) to the highest level of specificity that supports medical necessity. Provider(s) shall use the current ICD-10 edition and any subsequent editions in effect at the time of service. Provider(s) shall refer to the applicable edition for code description, as it is no longer documented in the policy.
- c. Code(s) - Provider(s) shall report the most specific billing code that accurately and completely describes the procedure, product or service provided. Provider(s) shall use the Current Procedural Terminology (CPT), Health Care Procedure Coding System (HCPCS), and UB-04 Data Specifications Manual (for a complete listing of valid revenue codes) and any subsequent editions in effect at the time of service. Provider(s) shall refer to the applicable edition for the code description, as it is no longer documented in the policy. If no such specific CPT or HCPCS code exists, then the provider(s) shall report the procedure, product or service using the appropriate unlisted procedure or service code.

*Unlisted Procedure or Service*

**CLINICAL POLICY WNC.CP.244****ALLOGENEIC HEMATOPOIETIC STEM-CELL TRANSPLANTATION FOR  
MYELOYDYSPLASTIC SYNDROMES & MYELOPROLIFERATIVE NEOPLASMS**

CPT: The provider(s) shall refer to and comply with the Instructions for Use of the CPT Codebook, Unlisted Procedure or Service, and Special Report as documented in the current CPT in effect at the time of service.

HCPCS: The provider(s) shall refer to and comply with the Instructions For Use of HCPCS National Level II codes, Unlisted Procedure or Service and Special Report as documented in the current HCPCS edition in effect at the time of service

- d. Modifiers - Providers shall follow applicable modifier guidelines.
- e. Billing Units - Provider(s) shall report the appropriate code(s) used which determines the billing unit(s).
- f. Co-payments -  
For Medicaid refer to Medicaid State Plan:  
<https://medicaid.ncdhhs.gov/meetingsnotices/medicaid-state-plan-public-notices>
- g. Reimbursement - Provider(s) shall bill their usual and customary charges. For a schedule of rates, refer to: <https://medicaid.ncdhhs.gov/>.

**Important Reminder**

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information. The Health Plan makes no representations and accepts no liability with respect to the content of any external information used or relied upon in developing this clinical policy. This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved. “Health Plan” means a health plan that has adopted this clinical policy and that is operated or administered, in whole or in part, by Centene Management Company, LLC, or any of such health plan’s affiliates, as applicable.

The purpose of this clinical policy is to provide a guide to medical necessity, which is a component of the guidelines used to assist in making coverage decisions and administering benefits. It does not constitute a contract or guarantee regarding payment or results. Coverage decisions and the administration of benefits are subject to all terms, conditions, conditions, exclusions, and limitations of the coverage documents (e.g., evidence of coverage, certificate of coverage, policy, contract of insurance, etc.), as well as to state and federal requirements and applicable Health Plan-level administrative policies and procedures.

This clinical policy is effective as of the date determined by the Health Plan. The date of posting may not be the effective date of this clinical policy. This clinical policy may be subject to applicable legal and regulatory requirements relating to provider notification. If there is a discrepancy between the effective date of this clinical policy and any applicable legal or regulatory requirement, the requirements of law and regulation shall govern. The Health Plan

**CLINICAL POLICY WNC.CP.244****ALLOGENEIC HEMATOPOIETIC STEM-CELL TRANSPLANTATION FOR  
MYELOYDYSPLASTIC SYNDROMES & MYELOPROLIFERATIVE NEOPLASMS**

retains the right to change, amend or withdraw this clinical policy, and additional clinical policies may be developed and adopted as needed, at any time.

This clinical policy does not constitute medical advice, medical treatment, or medical care. It is not intended to dictate to providers how to practice medicine. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members/enrollees. This clinical policy is not intended to recommend treatment for members/enrollees. Members/enrollees should consult with their treating physician in connection with diagnosis and treatment decisions.

Providers referred to in this clinical policy are independent contractors who exercise independent judgment and over whom the Health Plan has no control or right of control. Providers are not agents or employees of the Health Plan.

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