

Clinical Policy: Allogeneic Hematopoietic Stem Cell Transplantation for Genetic Diseases and Acquired Anemias

Reference Number: WNC.CP.241

Coding Implications

Last Review Date:

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.

Description¹

This policy describes the medical necessity criteria for Allogeneic Hematopoietic Transplant for Genetic Diseases and Acquired Anemias.

Policy/Criteria¹

- I. WellCare of North Carolina® shall cover Allogeneic Hematopoietic Stem Cell Transplantation for Genetic Diseases and Acquired Anemias with the following disorders:
 - A. Hemoglobinopathies
 1. Sickle Cell Anemia for children or young adults (defined as under age 40) with either a history of prior stroke or at increased risk of stroke or end-organ damage.
 2. Homozygous Beta-Thalassemia (i.e., Thalassemia Major).
 - B. Bone marrow failure syndromes
 1. Aplastic anemia including hereditary (e.g., Fanconi anemia, dyskeratosis congenita, Shwachman-Diamond, Diamond-Blackfan) or acquired (e.g., secondary to drug or toxin exposure) forms.
 - C. Primary immunodeficiencies
 1. Absent or defective T-cell function (e.g., severe combined immunodeficiency, Wiskott-Aldrich syndrome, X-linked lymphoproliferative syndrome).
 2. Absent or defective natural killer function (e.g., Chediak-Higashi syndrome).
 3. Absent or defective neutrophil function (e.g., Kostmann syndrome, chronic granulomatous disease, leukocyte adhesion defect).
 - D. Inherited metabolic disease
 1. Lysosomal and peroxisomal storage disorders **except** Hunter, Sanfilippo, and Morquio syndromes.
 - E. Genetic disorders affecting skeletal tissue
 1. Infantile malignant osteopetrosis (Albers-Schonberg disease or marble bone disease).
- II. WellCare of North Carolina® **shall not** cover Allogeneic Hematopoietic Stem Cell Transplantation for Genetic Diseases and Acquired Anemias in the following situations:
 - A. For diagnoses other than those covered in Section I; **OR**

CLINICAL POLICY WNC.CP.241

ALLOGENEIC HEMATOPOIETIC STEM CELL TRANSPLANTATION FOR GENETIC DISEASES AND ACQUIRED ANEMIAS

- B. When the member's psychosocial history limits the member's ability to comply with pre- and post-transplant medical care; **OR**
- C. When current member or caretaker non-compliance would make compliance with a disciplined medical regime improbable.

Background¹

Hematopoietic stem-cell transplantation refers to a procedure in which hematopoietic stem cells are infused to restore bone marrow function in patients who receive bone marrow toxic doses of cytotoxic drugs with or without whole body radiation therapy. Allogeneic HSCT refers to the use of hematopoietic progenitor cells obtained from a donor. They can be harvested from bone marrow, peripheral blood, or umbilical cord blood and placenta shortly after delivery of neonates.

Immunologic compatibility between infused stem cells and the recipient 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 Class I and Class II loci on chromosome 6. Depending on the disease being treated, an acceptable donor will match the patient at all or most of the HLA loci (with the exception of umbilical cord blood).

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 code.	12/21	02/22
Annual Review	11/22	11/22
NCHC verbiage removed from NC Guidance Verbiage.	04/23	04/23
Annual Review. CPT code removed.	11/23	11/23
Annual Review. Updated title to include 'Stem Cell Transplantation.' Removed "Medicaid and health choice" verbiage from References. Changed 'beneficiary' to member.	11/24	11/24

ALLOGENEIC HEMATOPOIETIC STEM CELL TRANSPLANTATION FOR GENETIC DISEASES AND ACQUIRED ANEMIAS

Reviews, Revisions, and Approvals	Reviewed Date	Approval Date
Annual Review. Under NC Guidance/Claims related information, updated state web address.		

References

1. State of North Carolina Medicaid Clinical Coverage Policy No: 11A-5 Allogeneic Hematopoietic Stem Cell Transplantation for Genetic Diseases and Acquired Anemias. [Program Specific Clinical Coverage Policies | NC Medicaid \(ncdhhs.gov\)](#). Published August 15, 2023. Accessed July 1, 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.

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.

CLINICAL POLICY WNC.CP.241

ALLOGENEIC HEMATOPOIETIC STEM CELL TRANSPLANTATION FOR GENETIC DISEASES AND ACQUIRED ANEMIAS

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/>

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:

CLINICAL POLICY WNC.CP.241

ALLOGENEIC HEMATOPOIETIC STEM CELL TRANSPLANTATION FOR GENETIC DISEASES AND ACQUIRED ANEMIAS

- 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

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-notice>
- 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

CLINICAL POLICY WNC.CP.241**ALLOGENEIC HEMATOPOIETIC STEM CELL TRANSPLANTATION FOR GENETIC DISEASES AND ACQUIRED ANEMIAS**

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 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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CLINICAL POLICY WNC.CP.241**ALLOGENEIC HEMATOPOIETIC STEM CELL TRANSPLANTATION FOR GENETIC DISEASES AND ACQUIRED ANEMIAS**

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