



Clinical Policy: Zolgensma - Pharmacy Prior Approval Criteria

Reference Number: WNC.CP.302

Last Review Date: 02/2025

[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.

Description¹ - This policy discusses medical necessity criteria for Zolgensma (onasemnogene abeparvovec-xioi) Pharmacy Prior Approval Criteria.

Therapeutic Class Code: Z0K

Therapeutic Class Description: Gene Therapy Agents - SMN Protein Deficiency

Policy/Criteria¹

- I. WellCare of North Carolina[®] shall cover Zolgensma when **ALL** the following requirements are met:
 - A. Member shall be enrolled on the date of service and may have service restrictions due to their eligibility category that would make them ineligible for this service;
 - B. Diagnosis of SMA with bi-allelic mutations in the survival motor neuron 1 (SMN1) gene. **(Please attach documentation)**
 - C. Genetic testing confirms the presence of **one** of the following (1, 2, **OR** 3): **(please attach documentation)**
 1. Homozygous deletions of SMN1 gene (e.g., absence of the SMN1 gene);
 2. Homozygous mutation in the SMN1 gene (e.g., biallelic mutations of exon 7);
 3. Compound heterozygous mutation in the SMN1 gene (e.g., deletion of SMN1 exon 7 (allele 1) and mutation of SMN1 (allele 2));
 - D. Prescribed by or in consultation with a neurologist;
 - E. Age < 2 years;
 - F. Documentation of one of the following baseline scores (1 **OR** 2): **(please attach documentation)** (exemption from this requirement for infants beginning treatment based on Newborn Screening results indicating baby has SMA)
 1. Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorder (CHOP-INTEND) score;
 2. Hammersmith Infant Neurological Examination (HINE) Section 2 motor milestone score;
 - G. Documentation of both of the following (1 **AND** 2): **(please attach documentation)**
 1. Baseline laboratory tests demonstrating Anti-AAV9 antibody titers \leq 1:50 as determined by ELISA binding immunoassay;

CLINICAL POLICY WNC.CP.302
ZOLGENSMA - PHARMACY PRIOR APPROVAL CRITERIA

- 2. Baseline liver function test, platelet counts, INR, and troponin-I;
 - H. Member does not have advanced SMA (e.g., complete paralysis of limbs, permanent ventilator dependence, tracheostomy, non-invasive ventilation beyond the use for sleep); **(please attach documentation)**
 - I. Member has not been previously treated with Zolgensma;
 - J. Zolgensma is not prescribed concurrently with Spinraza®;
 - K. Member does not have an active viral infection;
 - L. Total dose does not exceed 1.1 x 10¹⁴ vector genomes (vg) per kilogram (kg) body weight;
 - M. Zolgensma must be given in conjunction with pre and post infusion parenteral corticosteroids.
- II. WellCare of North Carolina’s® Duration of Approval is 4 weeks **(one-time infusion per lifetime)**.

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 2024, 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.

CPT®* Codes	Description
J3399	Zolgensma - onasemnogene abeparvovec-xioi

Reviews, Revisions, and Approval	Reviewed Date	Approval Date
Original Approval Date	02/2025	02/2025

References

1. State of North Carolina Medicaid Outpatient Pharmacy Prior Approval Criteria - Zolgensma. [Program Specific Clinical Coverage Policies | NC Medicaid \(ncdhhs.gov\)](https://www.ncdhhs.gov/program-specific-clinical-coverage-policies). Published March 1, 2024. Accessed January 10, 2025.

North Carolina Guidance
Eligibility Requirements

CLINICAL POLICY WNC.CP.302
ZOLGENSMA - PHARMACY PRIOR APPROVAL CRITERIA

1. An eligible beneficiary shall be enrolled in the NC Medicaid Program (Medicaid is NC Medicaid program, unless context clearly indicates otherwise);
2. Provider(s) shall verify each Medicaid beneficiary's eligibility each time a service is rendered.
3. 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

- 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:

- I. that is unsafe, ineffective, or experimental or investigational.
- II. 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

CLINICAL POLICY WNC.CP.302
ZOLGENSMA - PHARMACY PRIOR APPROVAL CRITERIA

- 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.
- **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:

- i. meet Medicaid qualifications for participation;
- ii. have a current and signed Department of Health and Human Services (DHHS) Provider Administrative Participation Agreement; and
- iii. 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:

- 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.
- 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.

CLINICAL POLICY WNC.CP.302

ZOLGENSMA - PHARMACY PRIOR APPROVAL CRITERIA

- 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.

- Modifiers - Providers shall follow applicable modifier guidelines.
- Billing Units - Provider(s) shall report the appropriate code(s) used which determines the billing unit(s).
- Co-payments -
For Medicaid refer to Medicaid State Plan:
<https://medicaid.ncdhhs.gov/get-involved/nc-health-choice-state-plan>
- 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, exclusions, and limitations of the coverage documents (e.g., evidence of coverage, certificate of coverage, policy,

CLINICAL POLICY WNC.CP.302
ZOLGENSMA - PHARMACY PRIOR APPROVAL CRITERIA



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.

This clinical policy is the property of the Health Plan. Unauthorized copying, use, and distribution of this clinical policy or any information contained herein are strictly prohibited. Providers, members/enrollees, and their representatives are bound to the terms and conditions expressed herein through the terms of their contracts. Where no such contract exists, providers, members/enrollees and their representatives agree to be bound by such terms and conditions by providing services to members/enrollees and/or submitting claims for payment for such services.

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