

**NC Division of Health Benefits
Outpatient Pharmacy
Prior Approval Criteria
Zolgensma**

**Medicaid
Effective Date: January 25, 2021
Amended Date: March 1, 2024**

Therapeutic Class Code: Z0K

Therapeutic Class Description: Gene Therapy Agents - SMN Protein Deficiency

Medication
Zolgensma Kit

Eligible Beneficiaries

NC Medicaid (Medicaid) beneficiaries 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.

EPSDT Special Provision: Exception to Policy Limitations for Beneficiaries 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 beneficiaries 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 clinician). This means EPSDT covers most of the medical or remedial care a child needs to improve or maintain his/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

- that is unsafe, ineffective, or experimental/investigational.
- 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/or 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

- 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 *Basic Medicaid Billing Guide*, sections 2 and 6, and on the EPSDT provider page. The Web

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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/medicaid/get-started/find-programs-and-services-right-you/medicaid-benefit-children-and-adolescents>

Criteria for Coverage:

1. Diagnosis of SMA with bi-allelic mutations in the survival motor neuron 1 (SMN1) gene. (please attach documentation)
2. Genetic testing confirms the presence of one of the following (a, b, or c): (please attach documentation)
 - a. Homozygous deletions of SMN1 gene (e.g., absence of the SMN1 gene);
 - b. Homozygous mutation in the SMN1 gene (e.g., biallelic mutations of exon 7);
 - c. Compound heterozygous mutation in the SMN1 gene (e.g., deletion of SMN1 exon 7 (allele 1) and mutation of SMN1 (allele 2));
3. Prescribed by or in consultation with a neurologist;
4. Age < 2 years;
5. Documentation of one of the following baseline scores (a or b): (please attach documentation) (exemption from this requirement for infants beginning treatment based on Newborn Screening results indicating baby has SMA)
 - a. Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorder (CHOP-INTEND) score;
 - b. Hammersmith Infant Neurological Examination (HINE) Section 2 motor milestone score;
6. Documentation of both of the following (a and b): (please attach documentation)
 - a. Baseline laboratory tests demonstrating Anti-AAV9 antibody titers $\leq 1:50$ as determined by ELISA binding immunoassay;
 - b. Baseline liver function test, platelet counts, INR, and troponin-I;
7. Beneficiary 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)
8. Beneficiary has not been previously treated with Zolgensma;
9. Zolgensma is not prescribed concurrently with Spinraza[®];
10. Member does not have an active viral infection
11. Total dose does not exceed 1.1×10^{14} vector genomes (vg) per kilogram (kg) body weight
12. Zolgensma must be given in conjunction with pre and post infusion parenteral corticosteroids.

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Approval duration: 4 weeks (one-time infusion per lifetime)

References

1. Zolgensma Prescribing Information, AveXis, Inc., Bannockburn, IL., 2019. Revised February 2023.

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Criteria Change Log

01/25/2021	Criteria effective date
02/01/2022	Added exemption from providing (CHOP-INTEND) score or HINE score for infants initiating treatment based on Newborn Screening results indicating baby has SMA)
03/01/2024	Addition of INR testing to baseline testing due to blackbox warning regarding acute liver failure and removed NC Health Choice language