NC Division of Health Benefits **Effective Date:**
Outpatient Pharmacy Prior Approval Criteria
Elevidys

DRAFT

Therapeutic Class Code: Z16
Therapeutic Class Description: GENE THERAPY AGENTS-PROTEIN DEFICIENCY

Medication
Elevidys

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

a. that is unsafe, ineffective, or experimental/investigational.
b. 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

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

Criteria for Coverage for Elevidys:

- Beneficiary has a Diagnosis of Duchenne muscular dystrophy (DMD);
  AND
- Beneficiary is aged ≥4 years and < ~~6~~–~~8~~ years old;
  AND
- Medication is prescribed by or in consultation with a Pediatric Neuromuscular Specialist;
  AND
- Beneficiary has a confirmed mutation of the DMD gene;
  AND
- Beneficiary does not have any deletion in exon 8 and/or exon 9 in the DMD gene;
  AND
- Beneficiary must have a baseline anti-AAVrh74 total binding antibody titer of < 1:400 as measured by ELISA;
  AND
- Beneficiary is ambulatory as confirmed by the North Star Ambulatory Assessment (NSAA) scale (score of ≥ 1);
  AND
- Beneficiary is not on concomitant therapy with DMD-directed antisense oligonucleotides (e.g., golodirsen, casimersen, viltolarsen, eteplirsen);
  AND
- Beneficiary has not received a DMD-directed antisense oligonucleotide within the past 30 days;
  AND
- Beneficiary does not have an active infection, including clinically important localized infections;
  AND
- Beneficiary has been on a stable dose of a corticosteroid, unless contraindicated or intolerance, prior to start of therapy and will be used concomitantly with a corticosteroid regimen pre- and postinfusion (refer to the package insert for recommended corticosteroid dosing during therapy);
  AND
- Beneficiary's troponin-I levels will be monitored at baseline and subsequently as clinically indicated;
  AND
- Beneficiary will have liver function and platelet count assessed prior to and following therapy for at least 3 months and as indicated; (to include at a minimum GGT and total bilirubin)
  AND
- Beneficiary must meet FDA-approved label for use (e.g., **use outside of studied population will be considered investigational**).

- While there are ongoing trials, the State is not aware of a resulted trial for ages above 8 so even though the FDA approval is not age based, since it hasn't been studied in older ages, the State considers usage in older ages to be investigational.
- Use should be avoided where the benefits of therapy versus risk are less well known. The indication for non-ambulatory patients is approved by the FDA under accelerated approval based on expression of microdystrophin in skeletal muscle. Continued approval by the FDA for this indication may be contingent upon verification of a clinical benefit in confirmatory trials. Use in non-ambulatory members is considered to be investigational until confirmatory trials are published.
- The ENVISION trial NCT05881408 will confirm clinical benefit for non-ambulatory patients and is expected to be completed January 2027.
- The provider attests that they are reasonably certain Elevidys can be administered before the beneficiary turns 8 years old.

- Approval duration: One time approval

References:

1. Elevidys [package insert]. Cambridge, MA; Sarepta; June 2023.

Criteria Change Log

xx/xx/xxxx	New Criteria