NC Division of Medical Assistance **Outpatient Pharmacy Prior Approval Criteria** **Agents for Duchenne Muscular Dystrophy**	**Medicaid and Health Choice** **Effective Date: May 1, 2017** **Amended Date:**

Therapeutic Class Code: Z1R
Therapeutic Class Description: GENETIC D/O TX-EXON SKIPPING ANTISENSE OLIGONUCLEOTIDE

Medication
Amondys 45
Exondys 51
Viltepso
Vyondys 53

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.

NC Health Choice (NCHC) beneficiaries, ages 6 through 18 years of age, shall be enrolled on the date of service to be eligible, and must meet policy coverage criteria, unless otherwise specified. **EPSDT does not apply to NCHC beneficiaries**.

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

Health Choice Special Provision: Exceptions to Policy Limitations for Health Choice Beneficiaries ages 6 through 18 years of age

EPSDT does not apply to NCHC beneficiaries. If a NCHC beneficiary does not meet the clinical coverage criteria within **the Outpatient Pharmacy prior approval** clinical coverage criteria, the NCHC beneficiary shall be denied services. Only services included under the Health Choice State Plan and the DMA clinical coverage policies, service definitions, or billing codes shall be covered for NCHC beneficiaries.

Criteria for Initial Coverage for Amondys 45:

- The beneficiary has a diagnosis of Duchenne Muscular Dystrophy;
  AND
- Medical records are submitted (ex: chart notes, laboratory values) that confirm the mutation of the Duchenne Muscular Dystrophy gene is amenable to exon 45 skipping;
  AND
- Medication is prescribed by or in consultation with a neurologist;
  AND
- The beneficiary retains meaningful voluntary motor function (beneficiary is able to speak, manipulate objects using upper extremities, ambulate, etc.);
  AND
- The beneficiary has been assessed for any physical therapy and/or occupational therapy needs;
  AND
- The beneficiary's serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio (UPCR) have been measured prior to starting therapy;
  AND
- Prescriber attestation that serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio will be measured during treatment (monthly urine dipstick with serum cystatin C and urine protein-to-creatinine ratio every 3 months);
  AND
- Baseline documentation of ≥ 1 of the following:
  - Dystrophin level
  - 6-minute walk test (6WMT) or other timed function tests

  - Upper limb function (ULM) test
  - North Star Ambulatory Assessment (NSAA)
  - Forced Vital Capacity (FVC) % predicted
  - Performance of Upper Limb (PUL);

AND

- The beneficiary is not receiving concomitant therapy with other DMD-directed antisense oligonucleotides (e.g., eteplirsen, gogodirsen, viltolarsen;

AND

- Amondys 45 dosing for Duchenne Muscular Dystrophy is in accordance with the USFDA approved labeling: maximum dosing of 30mg/kg once weekly;

AND

- Maximum length of initial approval: 6 months

Criteria for Initial Coverage for Exondys 51:

- The beneficiary has a diagnosis of Duchenne Muscular Dystrophy;

AND

- Medical records are submitted (ex: chart notes, laboratory values) that confirm the mutation of the Duchenne Muscular Dystrophy gene is amenable to exon 51 skipping;

AND

- Medication is prescribed by or in consultation with a neurologist;

AND

- The beneficiary retains meaningful voluntary motor function (beneficiary is able to speak, manipulate objects using upper extremities, ambulate, etc.);

AND

- The beneficiary has been assessed for any physical therapy and/or occupational therapy needs;

AND

- Baseline documentation of ≥ 1 of the following:
  - Dystrophin level
  - 6-minute walk test (6WMT) or other timed function tests
  - Upper limb function (ULM) test
  - North Star Ambulatory Assessment (NSAA)
  - Forced Vital Capacity (FVC) % predicted
  - Performance of Upper Limb (PUL);

AND

- The beneficiary is not taking Exondys 51 with any other RNA antisense agent, or any other gene therapy;

AND

- Exondys 51 dosing for Duchenne Muscular Dystrophy is in accordance with the USFDA

- approved labeling: maximum dosing of 30mg/kg once weekly;

AND

- Maximum length of initial approval: 6 months

Criteria for Initial Coverage for Vyondys 53:

- The beneficiary has a diagnosis of Duchenne Muscular Dystrophy;

AND

- Medical records are submitted (ex: chart notes, laboratory values) that confirm the mutation of the Duchenne Muscular Dystrophy gene is amenable to exon 53 skipping;

AND

- Medication is prescribed by or in consultation with a neurologist;
  AND
- The beneficiary retains meaningful voluntary motor function (beneficiary is able to speak, manipulate objects using upper extremities, ambulate, etc.);
  AND
- The beneficiary has been assessed for any physical therapy and/or occupational therapy needs;
- **AND**
- The beneficiary's serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio has been measured prior to the start of therapy;
  AND
- Prescriber attestation that serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio will be measured during treatment (monthly urine dipstick with serum cystatin C and urine protein-to-creatinine ratio every 3 months);
  AND
- Baseline documentation of ≥ 1 of the following:
  - Dystrophin level
  - 6-minute walk test (6WMT) or other timed function tests
  - Upper limb function (ULM) test
  - North Star Ambulatory Assessment (NSAA)
  - Forced Vital Capacity (FVC) % predicted;
  - Performance of Upper Limb (PUL);

  AND

- The beneficiary is not taking Vyondys 53 with any other RNA antisense agent, or any other gene therapy;
  AND
- Vyondys 53 dosing for Duchenne Muscular Dystrophy is in accordance with the USFDA approved labeling: maximum dosing of 30mg/kg once weekly;
  AND
- Maximum length of initial approval: 6 months

Criteria for Initial Coverage for Viltepso

- The beneficiary has a diagnosis of Duchenne Muscular Dystrophy;
  AND
- Medical records are submitted (ex chart notes, laboratory values) demonstrating a mutation on the DMD gene that is amenable to exon 53 skipping;
  AND
- Medication is prescribed by or in consultation with a neurologist;
  AND
- The beneficiary retains meaningful voluntary motor function (e.g. beneficiary is able to speak, manipulate objects using upper extremities, ambulate);
  AND
- The beneficiary has been assessed for any physical therapy and/or occupational therapy needs;
  AND
- The beneficiary is not on concomitant therapy with other DMD-directed antisense oligonucleotides

- (e.g., eteplirsen, golodirsen);
  AND

- The beneficiary's serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio has been measured prior to the start of therapy;
  AND
- Prescriber attestation that serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio will be measured and during treatment (monthly urine dipstick with serum cystatin C and urine protein-to-creatinine ratio every 3 months);
  AND
- Baseline documentation of ≥ 1 of the following:
  - Dystrophin level; **OR**
  - 6-minute walk test (6MWT) or other timed function tests; **OR**
  - Upper limb function (ULM) test; **OR**
  - North Star Ambulatory Assessment (NSAA); **OR**
  - Forced Vital Capacity (FVC) percent predicted
  - Performance of Upper Limb (PUL);
- Maximum length of initial approval: 6 months

Criteria for Renewal of Coverage

- The beneficiary must continue to meet initial approval criteria;
  AND
- The beneficiary has demonstrated a response to therapy compared to pretreatment baseline in of ≥ 1 of the following (not all-inclusive):
  - Increase in dystrophin level; **OR**
  - Stability, improvement, or slowed rate of decline in 6MWT or other timed function tests; **OR**
  - Stability, improvement, or slowed rate of decline in ULM test; **OR**
  - Stability, improvement, or slowed rate of decline in NSAA; **OR**
  - Stability, improvement, or slowed rate of decline in FVC% predicted; **OR**
  - Improvement in quality of life;

  AND
- The beneficiary has not experienced any treatment-restricting adverse effects (e.g. renal toxicities, proteinuria);
- Maximum length of renewal approval: 6 months

References

1. Prescriber Information Exondys 51 ® (eteplirsen) Sarepta Therapeutics, Inc, Cambridge, MA 02142. September 2016.
2. Vyondys 53 [package insert]. Cambridge, MA. Sarepta Therapeutics, Inc. December 2019.
3. Viltepso [package insert]. Paramus, NJ; NS Pharma; August 2020.
4. Amondys 45 [package insert]. Cambridge, MA; Sarepta Therapeutics; February 2021.

Criteria Change Log

05/01/2017	Criteria effective date
03/01/2021	Vyondys 53 added Viltepso
x/xxx/xxxx	For Vyondys 53-Added serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio has been measured prior to the start of therapy; and Prescriber attestation that serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio will be measured during treatment (monthly urine dipstick with serum cystatin C and urine protein-to-creatinine ratio every 3 months)
xx/xx/xxxx	Add Amondys 45