PROVIDER POLICIES & PROCEDURES

GENE-BASED EXON-SKIPPING THERAPY FOR DUCHENNE MUSCULAR DYSTROPHY (DMD)

The primary purpose of this document is to assist providers enrolled in the Connecticut Medical Assistance Program (CMAP) with the information needed to support a medical necessity determination for gene-based exon-skipping therapy for Duchenne muscular dystrophy (DMD). By clarifying the information needed for prior authorization of services, HUSKY Health hopes to facilitate timely review of requests so that individuals obtain the medically necessary care they need as quickly as possible.

This policy applies to the following gene-based exon-skipping therapies for DMD: **Amondys 45®** (casimersen), **Exondys 51** (eteplirsen), **Viltepso®** (viltolarsen), and **Vyondys 53®** (golodirsen).

Duchenne muscular dystrophy is caused by a defective DMD gene located on the X chromosome that is responsible for the production of dystrophin. Progressive muscle damage and degeneration occurs in people with DMD, resulting in muscle weakness, associated motor delays, loss of ambulation, respiratory impairment, and cardiomyopathy.

Exon-skipping therapies are designed to skip the specified exon on the genetic sequence, thereby correcting the out-of-frame mutation that causes a lack of functional dystrophin. This results in the production of an internally truncated dystrophin protein. Each of the four therapies listed below have been approved by the U.S. Food and Drug Administration (FDA) under accelerated approval based on an increase in dystrophin in skeletal muscle observed in treated patients.

Amondys 45 (casimersen) is an antisense oligonucleotide indicated for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 45 skipping.

Exondys 51 (eteplirsen) is an antisense oligonucleotide indicated for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping.

Viltepso (viltolarsen) is an antisense oligonucleotide indicated for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping.

Vyondys 53 (golodirsen) is an antisense oligonucleotide indicated for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping.

CLINICAL GUIDELINE

Coverage guidelines for gene-based exon-skipping therapy for DMD will be made in accordance with the DSS definition of Medical Necessity. The following criteria are guidelines *only*. Coverage guidelines are based on an assessment of the individual and their unique clinical needs. If the guidelines conflict with the definition of Medical Necessity, the definition of Medical Necessity shall prevail. The guidelines are as

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follows:

A. Amondys 45 (casimersen)

Initial Authorization

An initial 6-month trial of Amondys 45 for DMD may be considered medically necessary when ALL of the following criteria are met:

1. Submission of genetic testing confirming the diagnosis of DMD with a mutation amenable to exon 45 skipping ; **AND**
2. The treatment is prescribed by or in consultation with a physician who specializes in the treatment of DMD; **AND**
3. Submission of medical records confirming ALL of the following:
    a. The individual is ambulatory; **AND**
    b. Baseline age-appropriate motor function tests have been performed (e.g., 6-minute walk test (6MWT)); **AND**
    c. The individual is able to walk a distance of at least 300 meters independently over 6 minutes; **AND**
    d. Baseline pulmonary function tests have been performed, and the forced vital capacity (FVC) ≥50% predicted; **AND**
4. ONE of the following apply:
    a. The individual has not previously received gene replacement therapy for DMD (e.g., Elevidys); **OR**
    b. The individual has previously received gene replacement therapy for DMD and has experienced a worsening in clinical status; **AND**
5. The individual is only requesting one DMD gene-based exon-skipping therapy; **AND**
6. The treating provider will follow all FDA approved labeling for dosing, administration, and monitoring of Amondys 45.

Reauthorization

Continuation of treatment with Amondys 45 may be considered medically necessary at 6-month intervals when there is clinical documentation from the treating provider supporting a beneficial clinical response.

B. Exondys 51 (eteplirsen)

Initial Authorization

An initial 6-month trial of Exondys 51 for DMD may be considered medically necessary when ALL of the following criteria are met:

1. Submission of genetic testing confirming the diagnosis of DMD with a mutation amenable to exon 51 skipping; **AND**
2. The treatment is prescribed by or in consultation with a physician who specializes in the treatment of DMD; **AND**
3. Submission of medical records confirming ALL of the following:
    a. Baseline age-appropriate motor function tests have been performed (e.g., 6-minute walk test (6MWT), Upper limb function (ULM) test); **AND**

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b. The individual retains meaningful voluntary motor function; **AND**
c. Baseline pulmonary function tests have been performed, and the forced vital capacity (FVC) ≥50% predicted; **AND**

4. ONE of the following apply:
   a. The individual has not previously received gene replacement therapy for DMD (e.g., Elevidys); **OR**
   b. The individual has previously received gene replacement therapy for DMD and has experienced a worsening in clinical status; **AND**
5. The individual is only requesting one DMD gene-based exon-skipping therapy; **AND**
6. The treating provider will follow all FDA approved labeling for dosing, administration, and monitoring of Exondys 51.

Reauthorization

Continuation of treatment with Exondys 51 may be considered medically necessary at 6-month intervals when there is clinical documentation from the treating provider supporting a beneficial clinical response.

C. Viltepso (viltolarsen)

Initial Authorization

An initial 6-month trial of Viltepso for DMD may be considered medically necessary when ALL of the following criteria are met:

1. Submission of genetic testing confirming the diagnosis of DMD with a mutation amenable to exon 53 skipping; **AND**
2. The treatment is prescribed by or in consultation with a physician who specializes in the treatment of DMD; **AND**
3. Submission of medical records confirming ALL of the following:
   a. The individual is ambulatory; **AND**
   b. At least ONE of the following baseline age-appropriate motor function tests have been performed:
      1. Time to Stand Test (TTSTAND); **OR**
      2. Time to Run/Walk 10 Meters Test (TTRW); **OR**
      3. Six-minute Walk Test (6MWT); **OR**
      4. North Star Ambulatory Assessment (NSAA);**OR**
      5. Time to Climb 4 Steps Test (TTCLIMB); **OR**
      6. Hand-held dynamometer (elbow extension, elbow flexion, knee extension and knee flexion on the dominant side only); **AND**
   c. Baseline pulmonary function tests have been performed, and the forced vital capacity (FVC) ≥50% predicted; **AND**
4. ONE of the following apply:
   a. The individual has not previously received gene replacement therapy for DMD (e.g., Elevidys); **OR**
   b. The individual has previously received gene replacement therapy for DMD and has experienced a worsening in clinical status; **AND**
5. The individual is only requesting one DMD gene-based exon-skipping therapy; **AND**
6. The treating provider will follow all FDA approved labeling for dosing, administration, and

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monitoring for Viltepso.

Reauthorization

Continuation of treatment with Viltepso may be considered medically necessary at 6-month intervals when there is clinical documentation from the treating provider supporting a beneficial clinical response.

D. Vyondys 53 (golodirsen)

Initial Authorization

An initial 6-month trial of Vyondys 53 for DMD may be considered medically necessary when ALL of the following criteria are met:

1. Submission of genetic testing confirming the diagnosis of DMD with a mutation amenable to exon 53 skipping; **AND**
2. The treatment is prescribed by or in consultation with a physician who specializes in the treatment of DMD; **AND**
3. Submission of medical records confirming ALL of the following:
    a. The individual is ambulatory; **AND**
    b. Baseline age-appropriate motor function tests have been performed (e.g., 6-minute walk test (6MWT), North Star Ambulatory Assessment (NSAA)); **AND**
    c. The individual is able to walk an average distance of 250 meters independently over 6 minutes; **AND**
    d. Baseline pulmonary function tests have been performed and the forced vital capacity (FVC) ≥50% predicted; **AND**
4. ONE of the following apply:
    a. The individual has not previously received gene replacement therapy for DMD (e.g., Elevidys); **OR**
    b. The individual has previously received gene replacement therapy for DMD and has experienced a worsening in clinical status; **AND**
5. The individual is only requesting one DMD gene-based exon-skipping therapy; **AND**
6. The treating provider will follow all FDA approved labeling for dosing, administration, and monitoring of Vyondys 53.

Reauthorization

Continuation of treatment with Vyondys 53 may be considered medically necessary at 6-month intervals when there is clinical documentation from the treating provider supporting a beneficial clinical response.

Investigational and Not Medically Necessary

Any other indication for the use of Amondys 45, Exondys 51, Viltepso, and Vyondys 53, is considered investigational and not medically necessary.

NOTE: EPSDT Special Provision
Early and Periodic Screening, Diagnosis, and Treatment (EPSDT) is a federal Medicaid requirement that requires the Connecticut Medical Assistance Program (CMAP) to cover services, products, or procedures for

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Medicaid enrollees under 21 years of age where the service or good is medically necessary health care to correct or ameliorate a defect, physical or mental illness, or a condition identified through a screening examination. The applicable definition of medical necessity is set forth in Conn. Gen. Stat. Section 17b-259b (2011) [ref. CMAP Provider Bulletin PB 2011-36].

PROCEDURE
Prior authorization is required. Coverage determinations will be based upon a review of requested and/or submitted case-specific information.

The following information is needed to review requests for the above therapies:

1. Fully completed State of Connecticut, Department of Social Services HUSKY Health Program Gene-Based Exon-Skipping Therapy Prior Authorization Request form (to include physician's order and signature);
2. Clinical documentation supporting the medical necessity of treatment with Gene-Based Exon-Skipping Therapies should include the following:
    a. Genetic testing confirming:
        i. A mutation in the DMD gene amenable to exon skipping as appropriate for each therapy; **AND**
    b. Medical record documentation that the individual is ambulatory (for Amondys 45, Viltepso, and Vyondys 53 ONLY); **AND**
    c. Results from at least one of the following baseline motor assessments:
        i. North Star Ambulatory Assessment (NSAA); **OR**
        ii. Time to Rise (TTR); **OR**
        iii. 10-meter walk/run test (10MWR); **OR**
        iv. Time to Climb 4 steps; **OR**
        v. 100-meter walk/run test (100MWR); **OR**
        vi. Six-minute Walk Test (6MWT); **OR**
        vii. Hand-held dynamometer (elbow extension, elbow flexion, knee extension and knee flexion on the dominant side only);**OR**
        viii. Upper limb function (ULM) test; **AND**
    d. Baseline pulmonary function test confirming FVC ≥50% predicted; **AND**
    e. Signed provider attestation confirming the following:
        i. Gene-Based Exon Skipping Therapy will not be used in combination with gene replacement therapy for DMD; **AND**
        ii. Only ONE DMD gene-based exon-skipping therapy will be used (no concurrent administration); **AND**
3. Other information as requested by CHNCT.

Initial Authorization

When criteria, as outlined above, are met, initial approval for DMD gene-based exon skipping therapy will be granted for 6 months.

Reauthorization

When criteria, as outlined above, are met, reauthorization for DMD gene-based exon skipping therapy will be granted for 6 months.

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EFFECTIVE DATE
This Policy for individuals covered under the HUSKY Health Program is effective February 1, 2020.

LIMITATIONS
Not Applicable

CODES:

Code	Definition
J1426	Injection, casimersen, 10mg
J1427	Injection, viltolarsen, 10mg
J1428	Injection, eteplirsen, 10 mg
J1429	Injection, golodirsen, 10mg

DEFINITIONS

1. **HUSKY A**: Connecticut children and their parents or a relative caregiver; and pregnant women may qualify for HUSKY A (also known as Medicaid). Income limits apply.
2. **HUSKY B**: Uninsured children under the age of 19 in higher income households may be eligible for HUSKY B (also known as the Children's Health Insurance Program) depending on their family income level. Family cost-sharing may apply.
3. **HUSKY C**: Connecticut residents who are age 65 or older or residents who are ages 18-64 and who are blind, or have another disability, may qualify for Medicaid coverage under HUSKY C (this includes Medicaid for Employees with Disabilities (MED-Connect), if working). Income and asset limits apply.
4. **HUSKY D**: Connecticut residents who are ages 19-64 without dependent children and who: (1) do not qualify for HUSKY A; (2) do not receive Medicare; and (3) are not pregnant, may qualify for HUSKY D (also known as Medicaid for the Lowest-Income populations).
5. **HUSKY Health Program**: The HUSKY A, HUSKY B, HUSKY C, HUSKY D and HUSKY Limited Benefit programs, collectively.
6. **HUSKY Limited Benefit Program or HUSKY, LBP**: Connecticut's implementation of limited health insurance coverage under Medicaid for individuals with tuberculosis or for family planning purposes and such coverage is substantially less than the full Medicaid coverage.
7. **Medically Necessary or Medical Necessity**: (as defined in Connecticut General Statutes § 17b-259b) Those health services required to prevent, identify, diagnose, treat, rehabilitate or ameliorate an individual's medical condition, including mental illness, or its effects, in order to attain or maintain the individual's achievable health and independent functioning provided such services are: (1) Consistent with generally-accepted standards of medical practice that are defined as standards that are based on (A) credible scientific evidence published in peer-reviewed medical literature that is generally recognized by the relevant medical community, (B)recommendations of a physician-specialty society, (C) the views of physicians practicing in relevant clinical areas, and (D) any other relevant factors; (2) clinically appropriate in terms of type, frequency, timing, site, extent and duration and considered effective for the individual's illness, injury or disease; (3) not primarily for the convenience of the individual, the individual's health care provider or other health care providers; (4) not more costly than an alternative service or sequence of services at least as likely to produce

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equivalent therapeutic or diagnostic results as to the diagnosis or treatment of the individual's illness, injury or disease; and (5) based on an assessment of the individual and his or her medical condition.

8. **Prior Authorization**: A process for approving covered services prior to the delivery of the service or initiation of the plan of care based on a determination by CHNCT as to whether the requested service is medically necessary.

ADDITIONAL RESOURCES AND REFERENCES:

- Alfano LN, Charleston JS, Connolly AM, et al. Long-term treatment with eteplirsen in nonambulatory patients with Duchenne muscular dystrophy. *Medicine (Baltimore).* 2019;98(26):e15858. doi:10.1097/MD.0000000000015858
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PUBLICATION HISTORY

Status	Date	Action Taken
Original publication	December 2020	Approved at the October 28, 2020 CHNCT Medical Reviewer meeting. Approved by the CHNCT Clinical Quality Subcommittee on December 21, 2020. Approved by DSS on January 7, 2021.
Update	March 2021	Title change. Added coverage guidelines for AMONDYS-45, a new therapy for individuals with DMD that is amenable to exon 45 skipping. Coding update. Changes approved at the March 10, 2021 CHNCT Medical Reviewer meeting. Approved by the CHNCT Clinical Quality Subcommittee on March 15, 2021. Approved by DSS on March 22, 2021.
Update	June 2021	New code C9075 - casimersen, 10mg added. Code effective July 1, 2021. Approved by DSS on July 2, 2021.
Review	June 2022	Reviewed and approved without changes at the June 8, 2022 CHNCT Medical Reviewer meeting. Reviewed and approved without changes by the CHNCT Clinical Quality Subcommittee on June 20, 2022. Approved by DSS on July 5, 2022.
Update	September 2023	Added elevidys to the policy. Minor formatting and grammatical changes made, and additional references added. Reviewed and approved at the CHNCT Medical Reviewer meeting October 11, 2023. Reviewed and approved by the CHNCT Clinical Quality Subcommittee on December 18, 2023. Approved by DSS on January 03, 2023.

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Update	October 2024	Updated age and ambulation eligibility criteria for elevidys based on updated FDA indication. Removed requirement for corticosteroid regimen for both types of gene therapy. Updated verbiage in exon-skipping therapy reauthorization criteria. Added J1413 code, removed J3590. Updated references. Changes approved at the CHNCT Medical Reviewer meeting October 9, 2024. Approved by the CHNCT Clinical Quality Subcommittee on December 16, 2024. Approved by DSS on December 27, 2024.
Update	May 2025	Removed Elevidys from this policy, this is now a standalone policy. Updated introduction and indications for each medication to match FDA approved labeling. Added ambulatory requirement for Amondys 45, Vyondys 53, and Viltepso. Added a minimal 6MWT distance criteria for Amondys 45 and Vyondys 53. Added criteria to Exondys 51 requiring that the individual retains meaningful voluntary motor function. Added requirement of FVC ≥50% predicted for all treatments. Added criteria addressing concomitant use with gene replacement therapy. Added baseline motor function test options to Viltepso section. Reworded reauthorization sections. Updated Procedure section to clearly reflect requirements for authorization. Removed Requesting Authorization section. Updated references. Changes approved at the CHNCT Medical Reviewer meeting May 28, 2025. Changes approved by the CHNCT Clinical Quality Subcommittee on June 16, 2025. Approved by DSS on July 9, 2025.
Update	July 2025	Removed reference to Elevidys from Introduction and Procedure sections. FDA discontinued distribution of Elevidys on July 18, 2025. Change approved by DSS on July 23, 2025.

Please note that authorization is based on medical necessity at the time the authorization is issued and is not a guarantee of payment. Payment is based on the individual having active coverage, benefits and policies in effect at the time of service.

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