PROVIDER POLICIES & PROCEDURES

ELEVIDYS® (DELANDISTROGENE MOXEPARVOVEC-ROKL)

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 Elevidys, a gene-based 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.

Duchenne muscular dystrophy is caused by mutations in the DMD gene that result in the absence of dystrophin protein. Essential for muscle integrity, dystrophin is expressed in all muscles and provides protection from damage during normal muscle contraction.

Elevidys (delandistrogene moxeparvovec-rokl) is a one-time infused adeno-associated virus vector-based gene therapy indicated for the treatment of Duchenne muscular dystrophy in ambulatory patients who are at least 4 years of age and have a confirmed mutation in the DMD gene.

Note:

- In June 2024, the Food and Drug Administration (FDA) granted accelerated approval to Elevidys for the treatment of non-ambulatory patients aged 4 years and older.
- The DMD indication in non-ambulatory patients has been approved under accelerated approval based on expression of Elevidys micro-dystrophin. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial.
- Current data for patients over the age of 7 and non-ambulatory patients is limited. A clear association between micro-dystrophin expression and clinical outcome assessment (assessed by change from baseline on the Performance of Upper Limb version 2) in non-ambulatory patients has not been established. Clinical trials primarily focused on ambulatory patients ages 4 to 7; additional confirmatory trials for non-ambulatory patients and those over the age of 7 are ongoing.

Elevidys aims to address the lack of dystrophin by delivering a transgene encoding Elevidys micro-dystrophin, an engineered protein that consists of selected domains of wild-type dystrophin. Studies have demonstrated that Elevidys micro-dystrophin localizes to the sarcolemma, the critical membrane involved in muscular contraction.

Coverage guidelines for gene-based exon-skipping therapies for DMD are available **here**.

CLINICAL GUIDELINE

Coverage decisions for the use of Elevidys 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 follows:

Treatment with Elevidys (delandistrogene moxeparvovec-rokl) for DMD may be considered medically necessary when ALL of the following criteria are met:

A. Submission of genetic testing confirming **both** of the following:
    1. The individual has a diagnosis of DMD with a mutation in the DMD gene; **AND**
    2. The individual does not have a deletion in exon 8 and/or exon 9 in the DMD gene; **AND**
B. If the individual has a deletion in the DMD gene in exon 1 to 17 and/or exons 59 to 71, the provider will attest to monitoring the individual for a severe immune-mediated myositis reaction; **AND**
C. The individual is 4 to 7 years of age at the time of the request; **AND**
D. Submission of medical records confirming that the individual is ambulatory; **AND**
E. Submission of medical records confirming that at least one of the following baseline motor assessments have been completed:
    1. North Star Ambulatory Assessment (NSAA); **OR**
    2. Time to Rise (TTR); **OR**
    3. 10-meter walk/run test (10MWR); **OR**
    4. Time to ascend 4 steps; **OR**
    5. 100-meter walk/run test (100MWR); **AND**
F. **Both** of the following apply:
    1. Baseline liver function tests have been completed and confirm that the individual does not have preexisting hepatic impairment, acute liver disease (i.e., acute hepatic viral infection), chronic hepatic disease or elevated gamma-glutamyl transferase (GGT); **AND**
    2. Provider attests that liver function (clinical exam, GGT, and total bilirubin) will be monitored after Elevidys infusion in accordance with the FDA approved labeling; **AND**
G. **All** of the following apply:
    1. The individual does not have a left ventricle ejection fraction (LVEF) < 40%; **AND**
    2. Baseline troponin-I levels have been obtained; **AND**
    3. Provider attests that troponin-I will be monitored after Elevidys infusion in accordance with the FDA approved labeling; **AND**
H. **Both** of the following apply:
    1. Baseline platelet counts have been obtained; **AND**
    2. Provider attests that platelet counts will continue to be monitored after Elevidys infusion in accordance with the FDA approved labeling; **AND**
I. Submission of medical records confirming that the individual does not have an elevated anti-AAVrh74 total binding antibody titer ≥ 1:400; **AND**
J. The individual has not received Elevidys in the past; **AND**
K. Elevidys will not be used in combination with exon-skipping therapies (i.e., casimersen, eteplirsen, golodirsen, viltolarsen); **AND**
L. The individual does not currently have an active infection; **AND**
M. The individual is up to date on all CDC recommended childhood vaccines; **AND**
N. The individual will receive a corticosteroid regimen prior to and following receipt of Elevidys infusion as per the FDA approved labeling; **AND**
O. Elevidys is prescribed by or in consultation with a physician who specializes in the treatment of DMD (i.e., pediatric neurologist, neuromuscular specialist); **AND**
P. The treating provider will follow all FDA approved labeling for dosing, administration, and monitoring for Elevidys.

Investigational and Not Medically Necessary

Elevidys used for any other indication is considered investigational and not medically necessary.

Elevidys is a one-time therapy; repeat administration of Elevidys 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 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 Elevidys Prior Authorization Request form (to include physician's order and signature);
2. Clinical documentation supporting the medical necessity of treatment with Elevidys should include the following:
    a. Genetic testing confirming:
        i. A mutation in the DMD gene; **AND**
        ii. The individual does not have a deletion in exon 8 and/or exon 9 in the DMD gene; **AND**
    b. Medical record documentation that the individual is ambulatory; **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 ascend 4 steps; **OR**
        v. 100-meter walk/run test (100MWR); **AND**
    d. The following baseline labs confirming that above criteria are met, and that the individual is an appropriate candidate and safe to initiate treatment:
        i. Liver function tests (i.e., gamma-glutamyl transferase [GGT], and total bilirubin); **AND**
        ii. Platelet count; **AND**
        iii. Troponin-I; **AND**
        iv. LVEF; **AND**
        v. Anti-AAVrh74 total binding antibody titer; **AND**
    e. Signed provider attestation confirming the following:
        i. If there is a deletion in the DMD gene in exon 1 to 17 and/or exons 59 to 71, the provider will monitor the individual for a severe immune-mediated myositis reaction; **AND**
        ii. The provider will continue to monitor liver function tests, platelet count and troponin-I

after Elevidys infusion as per FDA approved labeling; **AND**

iii. Elevidys will not be used in combination with exon-skipping therapies; **AND**
iv. The individual does not currently have an active infection; **AND**
v. The individual is up to date on all CDC recommended childhood vaccines; **AND**
vi. The individual will receive a corticosteroid regimen prior to and following receipt of Elevidys infusion as per the FDA approved labeling; **AND**

3. Other information as requested by CHNCT.

Initial Authorization
When criteria, as outlined above, are met, authorization for Elevidys will be granted for 90 days.

Reauthorization
N/A

EFFECTIVE DATE
This Policy for individuals covered under the HUSKY Health Program is effective August 1, 2025.

LIMITATIONS
N/A

CODES:

Code	Definition
J1413	Injection, delandistrogene moxeparvovec-rokl, per therapeutic dose

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 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:

- A Gene Transfer Therapy Study to Evaluate the Safety and Efficacy of Delandistrogene Moxeparvovec (SRP-9001) in Participants With Duchenne Muscular Dystrophy (DMD) (EMBARK). ClinicalTrials.gov identifier: NCT05096221. Updated December 10, 2024. Accessed April 8 ,2025. https://classic.clinicaltrials.gov/ct2/show/NCT05096221.
- A Gene Transfer Therapy Study to Evaluate the Safety of and Expression From Delandistrogene Moxeparvovec (SRP-9001) in Participants With Duchenne Muscular Dystrophy (DMD) (ENDEAVOR). ClinicalTrials.gov identifier: NCT04626674. Updated August 26, 2024. Accessed April 29, 2025. https://clinicaltrials.gov/study/NCT04626674.
- A Gene Transfer Therapy Study to Evaluate the Safety of Delandistrogene Moxeparvovec (SRP-9001) in Participants With Duchenne Muscular Dystrophy (DMD). ClinicalTrials.gov identifier: NCT03375164. Updated November 14, 2024. Accessed April 29, 2025. https://clinicaltrials.gov/study/NCT03375164
- A Randomized, Double-blind, Placebo-controlled Study of Delandistrogene Moxeparvovec (SRP-9001) for Duchenne Muscular Dystrophy (DMD). ClinicalTrials.gov identifier: NCT03769116. Updated November 14, 2024. Accessed April 8, 2025. https://classic.clinicaltrials.gov/ct2/show/NCT03769116.
- Birnkrant DJ, Bushby K, Bann CM, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management [published correction appears in Lancet Neurol. 2018 Apr 4;:]. *Lancet Neurol*. 2018;17(3):251-267. doi:10.1016/S1474-4422(18)30024-3
- Bushby K, Finkel R, Birnkrant DJ, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and pharmacological and psychosocial management. *Lancet Neurol*. 2010;9(1):77-93. doi:10.1016/S1474-4422(09)70271-6
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- Darras BT. Duchenne and Becker muscular dystrophy: glucocorticoid and disease-modifying treatment. In: UpToDate, Shefner JM, Dashe JF (Eds), Wolters Kluwer. Updated August 7, 2024. Accessed April 8, 2025.
- Elevidys [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc.; Revised August 2024

- Mendell JR, Muntoni F, McDonald CM, et al. AAV gene therapy for Duchenne muscular dystrophy: the EMBARK phase 3 randomized trial. *Nat Med*. 2025;31(1):332-341. doi:10.1038/s41591-024-03304-z
- Mendell JR, Proud C, Zaidman CM, et al. Practical Considerations for Delandistrogene Moxeparvovec Gene Therapy in Patients With Duchenne Muscular Dystrophy. *Pediatr Neurol*. 2024;153:11-18. doi:10.1016/j.pediatrneurol.2024.01.003
- Mendell JR, Sahenk Z, Lehman KJ, et al. Long-term safety and functional outcomes of delandistrogene moxeparvovec gene therapy in patients with Duchenne muscular dystrophy: A phase 1/2a nonrandomized trial. *Muscle Nerve*. 2024;69(1):93-98. doi:10.1002/mus.27955
- Mendell JR, Shieh PB, McDonald CM, et al. Expression of SRP-9001 dystrophin and stabilization of motor function up to 2 years post-treatment with delandistrogene moxeparvovec gene therapy in individuals with Duchenne muscular dystrophy. *Front Cell Dev Biol*. 2023;11:1167762. Published 2023 Jul 11. doi:10.3389/fcell.2023.1167762
- Zaidman CM, Proud CM, McDonald CM, et al. Delandistrogene Moxeparvovec Gene Therapy in Ambulatory Patients (Aged ≥4 to <8 Years) with Duchenne Muscular Dystrophy: 1-Year Interim Results from Study SRP-9001-103 (ENDEAVOR). *Ann Neurol*. 2023;94(5):955-968. doi:10.1002/ana.26755

PUBLICATION HISTORY

Status	Date	Action Taken
Original publication	May 2025	Approved at the May 28, 2025, CHNCT Medical Reviewer meeting. Approved by the CHNCT Clinical Quality Subcommittee on June 16, 2025. Approved by DSS on July 9, 2025.