



PROVIDER POLICIES & PROCEDURES

GENE BASED THERAPY FOR DUCHENNE MUSCULAR DYSTROPHY (DMD) ETEPLIRSEN (EXONDYS 51®), GOLODIRSEN (VYONDYS 53®), VILTOLARSEN (VILTEPSO®) AND CASIMERSEN (AMONDYS 45™)

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 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.

Eteplirsen is a prescription medication used to treat DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping.

Golodirsen and viltolarsen are prescription medications used to treat DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping.

Casimersen is a prescription medication used to treat DMD in patients who have confirmed mutation of the DMD gene that is amenable to exon 45 skipping.

CLINICAL GUIDELINE

Coverage guidelines for gene based 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 follows:

Initial Authorization

Gene based therapy for DMD may be considered medically necessary if the following criteria is met:

- A. The individual has a diagnosis of DMD with mutation amenable to exon 51 skipping (eteplirsen), exon 53 skipping (golodirsen and viltolarsen), or exon 45 skipping (casimersen) confirmed by genetic testing (ordering physician MUST provide results of genetic testing); AND
- B. Must be prescribed by or in consultation with a physician who specializes in the treatment of DMD; AND
- C. The individual is currently receiving treatment with corticosteroids unless contraindicated or not tolerated; AND
- D. Baseline age appropriate motor-function and pulmonary function tests have been performed; AND
- E. The physician will follow all FDA recommendations for dosing, administration and monitoring.

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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Note: There is currently no established efficacy for concomitant therapy with two or more DMD directed antisense oligonucleotides. Requests for treatment with more than one agent will be reviewed on a case by case basis.

Reauthorization

Continuation of treatment may be considered medically necessary at 6 month intervals if the individual is benefiting from therapy (a signed letter from the individual's ordering physician is required).

Investigational and Not Medically Necessary

Any other indication for the use of eteplirsen, golodirsen, viltolarsen, and casimersen 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 Gene Therapy for DMD Prior Authorization Request form (to include physician's order and signature);
2. Baseline age appropriate motor-function and pulmonary function tests (initial authorization requests);
3. Letter from ordering physician documenting the benefits patient is receiving from treatment (reauthorization requests); and
4. Other information as requested by CHNCT.

Requesting Authorization

Requests must be submitted by the ordering physician and faxed to the number listed on the request form. Questions regarding this form should be directed to the HUSKY Health Program Utilization Management Department at 1.800.440.5071 (select option for medical authorizations).

Initial Authorization

Initial approval of gene based therapy for DMD will be for 6 months.

Re-authorization

Requests for continuation of gene based therapy for DMD will be reviewed at 6 month intervals.

EFFECTIVE DATE

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

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LIMITATIONS

Not Applicable

CODES:

Code	Definition
C9071	Injection, viltolarsen 10mg
C9075	Injection, casimersen, 10mg (effective July 1, 2021)
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 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

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service is medically necessary.

ADDITIONAL RESOURCES AND REFERENCES:

- AMONDYS 45 [Product Information]. Cambridge, Mass. Sarepta Therapeutics, Inc. Revised February 2021. [https://amondys45.com/Amondys45_\(casimersen\)_Prescribing_Information.pdf](https://amondys45.com/Amondys45_(casimersen)_Prescribing_Information.pdf).
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- 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
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- Bushby K, Finkel R, Birnkrant DJ, et al. Diagnosis and management of Duchenne muscular dystrophy, part 2: implementation of multidisciplinary care [published correction appears in *Lancet Neurol*. 2010 Mar;9(3):237]. *Lancet Neurol*. 2010;9(2):177-189. doi:10.1016/S1474-4422(09)70272-8
- Clemens PR, Rao VK, Connolly AM, et al. Safety, Tolerability, and Efficacy of Viltolarsen in Boys With Duchenne Muscular Dystrophy Amenable to Exon 53 Skipping: A Phase 2 Randomized Clinical Trial [published correction appears in doi: 10.1001/jamaneurol.2020.2025]. *JAMA Neurol*. 2020;77(8):982-991. doi:10.1001/jamaneurol.2020.1264
- ClinicalTrials.gov. Study of SRP-4045 and SRP-4053 in DMD patients (ESSENCE). Available at: <https://clinicaltrials.gov/ct2/show/NCT02500381>. Accessed on March 4, 2021.
- Exondys 51 [Product Information]. Cambridge, MA. Sarepta Therapeutics, Inc. Revised July 2020. <https://www.exondys51.com/modules/exondys/files/EXONDYS51PI.pdf>
- Frank DE, Mercuri E, Servais, L, et al. Golodirsen induces exon skipping leading to sarcolemmal dystrophin expression in patients with genetic mutations amenable to exon 53 skipping. Poster presented at: Annual Clinical Genetics Meeting of the American College of Medical Genetics and Genomics; April 2-6, 2019; Seattle, WA
- Institute for Clinical and Economic Review (ICER). Deflazacort, eteplirsen, and golodirsen for Duchenne muscular dystrophy: Effectiveness and value: Evidence Report. July 11, 2019. https://icer-review.org/wpcontent/uploads/2018/12/ICER_DMD_Evidence_Report_071119.pdf.
- Kinane TB, Mayer OH, Duda PW, Lowes LP, Moody SL, Mendell JR. Long-Term Pulmonary Function in Duchenne Muscular Dystrophy: Comparison of Eteplirsen-Treated Patients to Natural History. *J Neuromuscul Dis*. 2018;5(1):47-58. doi:10.3233/JND-170272
- Mendell JR, Goemans N, Lowes LP, et al. Longitudinal effect of eteplirsen versus historical control on ambulation in Duchenne muscular dystrophy. *Ann Neurol*. 2016;79(2):257-271. doi:10.1002/ana.24555
- Mendell JR, Rodino-Klapac LR, Sahenk Z, et al. Eteplirsen for the treatment of Duchenne muscular dystrophy. *Ann Neurol*. 2013;74(5):637-647. doi:10.1002/ana.23982
- Muntoni F, Frank DE, Morgan J, et al. Golodirsen induces exon skipping leading to sarcolemmal dystrophin expression in patients with genetic mutations amenable to exon 53 skipping [abstract]. *Neuromuscul Disord*. 2018;28:S5. Abstract D01.

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- Muntoni F, Frank DE, Sardone V, et al. SRP-4053 induces exon skipping leading to sarcolemmal dystrophin expression in Duchenne muscular dystrophy patients amenable to exon 53 skipping. Poster presented at: 22nd International Annual Congress of the World Muscle Society; October 3-7 2017; Saint Malo, France.
- Study of SRP-4045 and SRP-4053 in DMD Patients (ESSENCE) <https://clinicaltrials.gov/ct2/show/NCT02500381?term=golodirsen&cond=Duchenne+Muscular+Dystrophy&rank=3>.
- Study to Assess the Efficacy and Safety of Viltolarsen in Ambulant Boys with DMD (RACER53) <https://clinicaltrials.gov/ct2/show/NCT04060199?term=viltolarsen&draw=2&rank=1>.
- UptoDate. Duchenne and Becker muscular dystrophy: Glucocorticoid and disease-modifying treatment. Last updated September 1, 2020.
- Viltepso [Product Information]. Paramus, NJ. NS Pharma, Inc. Revised August 2020. <https://www.viltepso.com/prescribing-information>
- Vyondys 53 [Product Information]. Cambridge, MA. Sarepta Therapeutics, Inc. Revised August 2020. [https://www.vyondys53.com/Vyondys53_\(golodirsen\)_Prescribing_Information.pdf](https://www.vyondys53.com/Vyondys53_(golodirsen)_Prescribing_Information.pdf)

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.

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