Pharmacy Medical Necessity Guidelines: Exondys 51™ (eteplirsen)

Effective: February 9, 2021

Prior Authorization Required	√	Type of Review – Care Management	
Not Covered		Type of Review – Clinical Review	√
Pharmacy (RX) or Medical (MED) Benefit	MED/ RX	Department to Review	PRECERT/ MM

These pharmacy medical necessity guidelines apply to the following:

Commercial Products

☒ Tufts Health Plan Commercial products – large group plans
☒ Tufts Health Plan Commercial products – small group and individual plans
☒ Tufts Health Freedom Plan products – large group plans
☒ Tufts Health Freedom Plan products – small group plans
- CareLink℠ – Refer to CareLink Procedures, Services and Items Requiring Prior Authorization

Tufts Health Public Plans Products

☒ Tufts Health Direct – A Massachusetts Qualified Health Plan (QHP) (a commercial product)
☒ Tufts Health Together – MassHealth MCO Plan and Accountable Care Partnership Plans
☒ Tufts Health RITogether – A Rhode Island Medicaid Plan

Fax Numbers:

All plans except Tufts Health Public Plans:
PRECERT: 617.972.9409

Tufts Health Public Plans:
MM: 888.415.9055

Note: This guideline does not apply to Medicare Members (includes dual eligible Members).

OVERVIEW

FOOD AND DRUG ADMINISTRATION (FDA)-APPROVED INDICATIONS

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

This indication is approved under accelerated approval based on an increase in dystrophin in skeletal muscle observed in some patients treated with Exondys 51 (eteplirsen). A clinical benefit of Exondys 51 (eteplirsen) has not been established. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

DMD, a form of muscular dystrophy, is a genetic disorder characterized by progressive muscle degeneration and weakness that predominantly affects males. DMD is caused by a deficiency of dystrophin, a protein that helps strengthen muscle fibers and protect them from injury. DMD is the most severe form of muscular dystrophy and without intervention the mean age at death is around 19 years. Currently there is no cure for DMD and therapies are supportive in nature. Glucocorticoids are the only medication currently available that slows the decline in muscle strength and function in DMD. Additional benefits of glucocorticoids are reduction in the risk of scoliosis and stabilization of pulmonary function.

Exondys 51 (eteplirsen) is the first FDA-approved medication for the treatment of DMD. Accelerated approval was based on a surrogate endpoint of increased dystrophin levels that was deemed reasonably likely to predict clinical benefit. Currently, there is limited clinical trial data available that demonstrates a statistically significant clinical benefit of Exondys 51 (eteplirsen) compared to placebo in the treatment of DMD.

COVERAGE GUIDELINES

The plan may authorize initial coverage of Exondys 51 (eteplirsen) for Members, when the following criteria are met:

Initial Therapy

1. Documented diagnosis of Duchenne muscular dystrophy with medical records confirming a mutation of the Duchenne muscular dystrophy gene that is amenable to exon 51 skipping

Note: *Common Duchenne muscular dystrophy deletions that are theoretically amenable to exon 51 skipping include: 50, 52, 43-50, 45-50, 47-50, 48-50, and 49-50.*

AND

2. The prescribing physician is a neurologist or a provider who specializes in the treatment of Duchenne muscular dystrophy

AND

3. Documentation of one of the following:
    a. Member has been receiving a stable dose of corticosteroids for a period of at least 6 months
    b. Member has a contraindication to corticosteroids

Reauthorization Criteria

1. Documented diagnosis of Duchenne muscular dystrophy with medical records confirming a mutation of the Duchenne muscular dystrophy gene that is amenable to exon 51 skipping

Note: *Common Duchenne muscular dystrophy deletions that are theoretically amenable to exon 51 skipping include: 50, 52, 43-50, 45-50, 47-50, 48-50, and 49-50.*

AND

2. The prescribing physician is a neurologist or a provider who specializes in the treatment of Duchenne muscular dystrophy

AND

3. Documentation of one of the following:
    a. Member continues to utilize corticosteroids in combination with Exondys 51 (eteplirsen)
    b. Member has a contraindication to corticosteroids

AND

4. Documentation that based on the prescriber's assessment, the Member continues to benefit from Exondys 51 (eteplirsen), documented by a standardized assessment of motor function or respiratory function

LIMITATIONS

- Authorizations will be provided for 6 months.
- Members new to the plan stable on Exondys 51 (eteplirsen) should be reviewed against Reauthorization Criteria.
- The plan will not authorize the use of Exondys 51 (eteplirsen) in Members with Duchenne muscular dystrophy who do not have a confirmed mutation of the Duchenne muscular dystrophy gene that is amenable to exon 53 skipping. Common Duchenne muscular dystrophy deletions that are theoretically amenable to exon 51 skipping include: 50, 52, 43-50, 45-50, 47-50, 48-50, and 49-50.
- The plan will not authorize the use of Exondys 51 (eteplirsen) in combination with other disease modifying therapies for Duchenne muscular dystrophy as there no evidence to suggest combination therapy is safe or effective.

CODES

The following HCPCS/CPT code(s) are:

Code	Description
J1428	Injection, eteplirsen, 10 mg

REFERENCES

1. Exondys 51 (eteplirsen) [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc.; July 2020.
2. 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;9:77-93.

3. Bushby K, Finkel R, Birnkrant DJ, et al. Diagnosis and management of Duchenne muscular dystrophy, part 2: implementation of multidisciplinary care. Lancet Neurol. 2010;9:177-89.
4. Mendell JR, Rodino-Klapac LR, Sahenk Z, et al. Eteplirsen for the treatment of Duchenne muscular dystrophy. Annals of Neurology. 2013 Nov;74(5):637-47.
5. Mendell JR, Goemans N, Lowes LP, et al. Longitudinal effect of eteplirsen versus historical control on ambulation in Duchenne muscular dystrophy. Annals of Neurology. 2016 Feb;79(2):257-71.

APPROVAL HISTORY

March 14, 2017: Reviewed by Pharmacy & Therapeutics Committee.

Subsequent endorsement date(s) and changes made:

1. April 11, 2017: Administrative update. Effective 6/1/2017, Medical Necessity Guideline applies to Tufts Health RITogether.
2. January 1, 2018: Administrative update: Added new J code J1428 to Medical Necessity Guideline and removed expired C code C9484.
3. March 13, 2018: No changes
4. July 10, 2018: Effective10/1/18, added the requirement that treatment is/was initiated prior to age 14 years of age. Added the following coverage limitations: Members new to the plan stable on Exondys 51 (eteplirsen) should be reviewed against Initial Coverage Criteria for the first coverage request and coverage will not be authorized for Members who are wheelchair dependent. Updated the coverage limitation addressing the duration of approval for initial authorizations to apply to all members.
5. January 8, 2019: No changes
6. April 14, 2020: Effective July 1, 2020, modified reauthorization criteria and updated the Limitations so that Members new to the plan stable on Exondys 51 (eteplirsen) should be reviewed against Reauthorization Criteria. For Initial Therapy, removed the requirements that treatment is/was initiated prior to 14 years of age, for documentation the Member is ambulatory demonstrated by a baseline 6-minute walk test of at least 200 meters. Added the Limitation: "The plan will not authorize the use of Exondys 51 (eteplirsen) in combination with other disease modifying therapies for Duchenne muscular dystrophy as there no evidence to suggest combination therapy is safe or effective." Removed the following Limitations: "The plan will not authorize the use of Exondys (eteplirsen) for other forms of muscular dystrophy" and "Coverage will not be authorized for Members who are wheelchair dependent."
7. February 9, 2021: No changes. Added information throughout the Medical Necessity Guidelines about what DMD deletions that are theoretically amenable to exon 51 skipping.

BACKGROUND, PRODUCT AND DISCLAIMER INFORMATION

Pharmacy Medical Necessity Guidelines have been developed for determining coverage for plan benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. The plan makes coverage decisions on a case-by-case basis considering the individual member's health care needs. Pharmacy Medical Necessity Guidelines are developed for selected therapeutic classes or drugs found to be safe, but proven to be effective in a limited, defined population of patients or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in the service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. The plan revises and updates Pharmacy Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests needed revisions.

For self-insured plans, coverage may vary depending on the terms of the benefit document. If a discrepancy exists between a Pharmacy Medical Necessity Guideline and a self-insured Member's benefit document, the provisions of the benefit document will govern.

Treating providers are solely responsible for the medical advice and treatment of members. The use of this policy is not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to member eligibility and benefits on the date of service, coordination of benefits, referral/authorization and utilization management guidelines when applicable, and adherence to plan policies and procedures and claims editing logic.

Provider Services