Draft Revision Policy: Do Not Implement

Eteplirsen (Exondys 51™)

Does Not Apply to BlueCare

IMPORTANT REMINDER

We develop Medical Policies to provide guidance to Members and Providers. This Medical Policy relates only to the services or supplies described in it. The existence of a Medical Policy is not an authorization, certification, explanation of benefits or a contract for the service (or supply) that is referenced in the Medical Policy. For a determination of the benefits that a Member is entitled to receive under his or her health plan, the Member's health plan must be reviewed. If there is a conflict between the medical policy and a health plan or government program (e.g., TennCare), the express terms of the health plan or government program will govern.

The proposal is to add text/statements in red and to delete text/statements with strikethrough:

POLICY

INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications
Exondys 51 is 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. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

All other indications are considered experimental/investigational and not medically necessary.

DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:

- Initial requests:
  - Laboratory confirmation of Duchenne muscular dystrophy (DMD) diagnosis with a DMD gene mutation that is amenable to exon 51 skipping (refer to examples in Appendix).
  - If applicable, medical records confirming a worsening in clinical status since receiving gene replacement therapy.
  - Chart notes or medical record documentation demonstrating the member can ambulate an average distance between 200-300 meters during a 6 minute walk test (6 MWT).
- Continuation of therapy requests: documentation (e.g., chart notes) of response to therapy.

PRESCRIBER SPECIALTIES

This medication must be prescribed by or in consultation with a physician who specializes in treatment of Duchenne muscular dystrophy (DMD).

COVERAGE CRITERIA

Duchenne Muscular Dystrophy

Authorization of 6 months may be granted for treatment of DMD when all of the following criteria are met:

- Genetic testing was conducted to confirm the diagnosis of DMD and to identify the specific type of DMD gene mutation.
- The DMD gene mutation is amenable to exon 51 skipping (refer to examples in Appendix).
- Member is 7-13 years of age ~~Treatment with Exondys 51 is initiated before the age of 14~~.
- Documentation is provided that demonstrates the member is able to walk an average distance between 200-300 ~~Member is able to achieve an average distance of at least 180~~ meters while walking independently ~~over~~ during a 6MWT ~~6 minutes~~.
- Member meets one of the following:
  - Member is using a corticosteroid.
  - Member has a history of intolerance to corticosteroids or a contraindication to corticosteroid use.
- Member meets one of the following criteria:
  - Member has not previously received gene replacement therapy for DMD (e.g., Elevidys).
  - Member has previously received gene replacement therapy for DMD (e.g., Elevidys) and has experienced a worsening in clinical status since receiving gene replacement therapy (e.g., decline in ambulatory function).
- Member will not exceed a dose of 30 mg/kg once weekly.

CONTINUATION OF THERAPY

Note: Members who were previously established on Exondys 51 and subsequently administered gene replacement therapy (e.g., Elevidys) must meet all requirements in the coverage criteria section prior to re-starting Exondys 51.

Authorization of 12 months may be granted for members requesting continuation of therapy when both of the following criteria are met:

- The member has demonstrated a response to therapy as evidenced by remaining ambulatory (e.g., able to walk with or without assistance, not wheelchair dependent).
- The member will not exceed a dose of 30 mg/kg once weekly.

APPENDIX

Examples of DMD gene mutations (exon deletions) amenable to exon 51 skipping (not an all-inclusive list):

- Deletion of exon 50
- Deletion of exon 52
- Deletion of exons 45-50
- Deletion of exons 47-50
- Deletion of exons 48-50
- Deletion of exons 49-50

APPLICABLE TENNESSEE STATE MANDATE REQUIREMENTS

BlueCross BlueShield of Tennessee's Medical Policy complies with Tennessee Code Annotated Section 56-7-2352 regarding coverage of off-label indications of Food and Drug Administration (FDA) approved drugs when the off-label use is recognized in one of the statutorily recognized standard reference compendia or in the published peer-reviewed medical literature.

ADDITIONAL INFORMATION

For appropriate chemotherapy regimens, dosage information, contraindications, precautions, warnings, and monitoring information, please refer to one of the standard reference compendia (e.g., the NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) published by the National Comprehensive Cancer Network®, Drugdex Evaluations of Micromedex Solutions at Truven Health, or The American Hospital Formulary Service Drug Information).

REFERENCES

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3. Cirak S, Arechavala-Gomeza V, Guglieri M, et al. Exon skipping and dystrophin restoration in patients with Duchenne muscular dystrophy after systemic phosphorodiamidate morpholino oligomer treatment: an open-label, phase 2, dose-escalation study. Lancet. 2011;378(9791):595-605.
4. Mendell JR, Goemans N, Lowes LP, et al; Eteplirsen Study Group and Telethon Foundation DMD Italian Network. Longitudinal effect of eteplirsen versus historical control on ambulation in Duchenne muscular dystrophy. Ann Neurol. 2016;79(2):257-271.
5. Randeree L, Eslick GD. Eteplirsen for pediatric patients with Duchenne muscular dystrophy: A pooled-analysis. J Clin Neurosci. 2018;49:1-6.
6. ClinicalTrials.gov. Efficacy Study of AVI-4658 to Induce Dystrophin Expression in Selected Duchenne Muscular Dystrophy Patients. ClinicalTrials.gov identifier: NCT01396239. Updated March 30, 2020. Accessed June 17, 2026. https://clinicaltrials.gov/study/NCT01396239
7. Lexi-Comp Online. (2026, May). AHFS DI. Eteplirsen. Retrieved June 2026 from Lexi-Comp Online with AHFS.
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EFFECTIVE DATE

ID_BT_2025