STATE OF WEST VIRGINIA
DEPARTMENT OF HEALTH AND HUMAN RESOURCES
BUREAU FOR MEDICAL SERVICES

Office of Pharmacy Service
Prior Authorization Criteria

EXONDYS 51® (eteplirsen)
Effective 02/19/2020

EXONDYS 51 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 was approved under the accelerated approval process and was based on an increase in dystrophin in skeletal muscle observed in some patients treated with EXONDYS 51. **A clinical benefit of EXONDYS 51 has not been established.** Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

Exondys 51 may be billed as a Medical ("Buy & Bill") claim OR as a Pharmacy Point-of-Sale (POS) claim:

- Medical "Buy & Bill" under J1428 - Contact KEPRO, tel: (304) 343-9663/ fax (866) 209-9632
- Pharmacy POS - Contact RDTP, tel: (800) 847-3859/ fax (800) 531-7787

All requests require review by the Medical Director and may be approvable once the following criteria are met:

1. Patient must have a confirmed mutation of a DMD gene that is amenable to exon 51 skipping (chart notes required); **AND**

2. The patient must meet all label requirements as recommended by the FDA and the manufacturer; **AND**

3. Request must either be from a neurologist or from a physician who has provided documentation of a formal consultation with a neurologist; **AND**

   Patient must be stabilized on corticosteroid therapy for at least 6 months prior to the request for coverage of Exondys 51. Documentation must be supplied detailing the prescribed steroid therapy and the patient must continue this therapy while receiving Exondys 51
   NOTE: If the patient cannot take steroid therapy, clinical justification must be provided by the physician, otherwise the prior authorization request shall be immediately denied;

   AND

4. The results of appropriate and validated baseline functional tests must be submitted with the initial request for therapy. These results will be considered valid only if taken after the patient has received corticosteroid therapy for at least 6 months.
   Acceptable tests may include, **but are not limited to**, any of the following:
   a. Ambulatory patients: Six-minute walk test (6MWDT) (patient must achieve > 180 meters for approval.
   b. Non-ambulatory patients: Brooke Upper Extremity Function Scale (of 5 or less) **AND** a Forced Vital Capacity of ≥ 30% of predicted value are required for approval.

Other functional assessment tests may be accepted on a case-by-case basis at the discretion of the Medical Director. These tests must be quantitative in nature and accompanied with supporting documentation and references describing the test.

All prior authorization approvals are limited to 6 months at time and continuation of coverage requires the following conditions to be met:

1) Follow-up functional test results must show stabilization or improvement of patient function compared to baseline measures; **AND**
2) Patient must maintain 100% compliance with all scheduled therapy - Exondys 51 must be dosed once per week and maintenance steroid therapy must continue as prescribed by the physician. Failure to maintain compliance with prescribed therapy shall result in immediate discontinuation of coverage unless the disruption can be medically justified by the prescribing physician.

REFERENCES

1.) Exondys 51 Package Insert (Sarepta Therapeutics) – Revised 2/2018
2.) Lexicomp monograph for Exondys 51 – reviewed 5/10/2018
3.) Measures of Clinical Assessment in Patients with Duchenne Muscular Dystrophy (DMD) https://sarepta.appdataroom.com/download/v/bd16dff387135c4643386085c1bdcbc58fc508ef490459?ts=1582134595&sig=35e5f191eed1241b58063411fc49504f65bf63e7&trackingGuid=CDB061BF-A492-4090-A22E-2FADB6F9B100&groupId=4411
4.) Birnrant et al. Lancet Neurol. 2018 March; 17(3): 251-267. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management
5.) Mendell, JR et al. Ann Neurol 2016;79:257–271. Longitudinal Effect of Eteplirsen versus Historical Control on Ambulation in Duchenne Muscular Dystrophy
6.) Kinane, TB et al. Journal of Neuromuscular Diseases 5 (2018) 47–58 Long-Term Pulmonary Function in Duchenne Muscular Dystrophy: Comparison of Eteplirsen-Treated Patients to Natural History
7.) Kenji Rowel Q Lim, Rika Maruyama and Toshifumi Yokota Drug Des Devel Ther. 2017; 11: 533–545. Eteplirsen in the treatment of Duchenne muscular dystrophy.
8.) Clinical Trials:
    a. https://clinicaltrials.gov/ct2/show/NCT01396239?term=eteplirsen&rank=6
    b. https://clinicaltrials.gov/ct2/show/NCT01540409
    c. **Ongoing confirmatory phase 3 trial:** An Open-Label, Multi-Center, Study With a Concurrent Untreated Control Arm to Evaluate the Efficacy and Safety of Eteplirsen in Duchenne Muscular Dystrophy (https://clinicaltrials.gov/ct2/show/NCT02255552)
    Estimated study completion date May 1, 2019