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 3/27/2019

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. Prescriber is a neurologist or has submitted documentation of a formal consultation with a neurologist; **AND**

4. Patient must be currently taking a corticosteroid OR have a contraindication to corticosteroids; **AND**

5. Appropriate and validated baseline function test results must be submitted with the initial request for therapy. These tests may include any of the following:

    a. Ambulatory patients: Six-minute walk test (6MWDT) of > 180 meters.
    b. Non-ambulatory patients: Brooke Upper Extremity Function Scale (of 5 or less) AND a Forced Vital Capacity of ≥ 30% of predicted value.

Other function tests may be accepted on a case-by-case basis but must include supporting documentation describing the test as well as peer-reviewed literature references.

Prior authorization approvals will be for 6 months. Continuation requests must provide clinical documentation of efficacy as evidenced by improvement or stabilization of functions compared to baseline measures.

BMT updated 03/26/2019
DUR Board Approval: 05/31/2018

REFERENCES

1.) Exondys 51 Package Insert (Sarepta Therapeutics) – Revised 2/2018
2.) Lexicomp monograph for Exondys 51 – reviewed 5/10/2018
3.) 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
4.) Mendell, JR et al. Ann Neurol 2016;79:257–271. Longitudinal Effect of Eteplirsen versus Historical Control on Ambulation in Duchenne Muscular Dystrophy
5.) 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
6.) 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.
7.) 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