Exondys 51
(eteplirsen injection)

Date of Origin: 1/16/18 **Last Review Date: 1/24/18** **Effective Date: 1/24/18**

Dates Reviewed: 1/24/18

Developed By: Medical Criteria Committee

I. Length of Authorization

N/A

II. Dosing Limits

N/A

III. Initial Approval Criteria

The use of eteplirsen (Exondys 51) for Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping† does not meet the definition of medical necessity, defined as those services and supplies that are required for diagnosis or treatment of a medical condition and are:

a. Appropriate and consistent with the symptoms or diagnosis of a member's condition
b. Established as the standard treatment by the medical community in the service area in which they are received
c. Not primarily for the convenience of a member or a provider
d. The least costly of the alternative supplies or levels of service that can be safely provided to a member.

The fact that a provider prescribes, orders, recommends, or approves a service or supply does not, of itself, make the service medically necessary or a covered service.

†FDA Approved Indication(s), ‡ Compendia recommended indication

IV. Renewal Criteria

N/A

V. Dosage/Administration

N/A

VI. Billing Code/Availability Information

Jcode:

- J1428 – injection, eteplirsen: 1 billable unit = 10 mg

NDC:

- Exondys 51 100 mg/2mL (50 mg/mL) single-dose vial for injection: 60923-363-xx
- Exondys 51 500 mg/10mL (50 mg/mL) single-dose vial for injection: 60923-284-xx

VII. References

1. Exondys 51 [package insert]. Cambridge, MA; Septra Therapeutics, Inc; September 2016. Accessed January 2018.
2. 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. PMID: 23907995
3. 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. PMID: 26573217
4. FDA CDER: Summary Review 206488Orig1s000. Available from: http://www.accessdata.fda.gov/drugsatfda_docs/nda/2016/206488Orig1s000SumR.pdf
5. FDA CDER: Medical Review 206488Orig1s000. Available from: http://www.accessdata.fda.gov/drugsatfda_docs/nda/2016/206488Orig1s000MedR.pdf