Exondys 51
(eteplirsen injection)

Date of Origin: 1/16/18 **Last Review Date: 12/23/19** **Effective Date: 1/24/18**

Dates Reviewed: 1/24/18, 12/23/19

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 delivery of a service by a qualified healthcare provider, exercising prudent clinical judgement that meets all of the following:

a. Is for the purpose of preventing, evaluating, diagnosing or treating a medical condition or its symptoms
b. Is in accordance with generally accepted standards of medical practice
c. Is proven to be effective in producing intended effects on health outcomes (e.g., morbidity, mortality, quality of life, symptom control, function) associated with the member's medical condition or its symptoms
d. Has beneficial effects on health outcomes that outweigh the potential harmful effects
e. Is clinically appropriate in terms of type, frequency, extent, site and duration
f. Is not primarily for the convenience of the patient or healthcare provider
g. Is at least as likely to produce equivalent therapeutic or diagnostic results for the diagnosis or treatment of that patient's medical condition or its symptoms as an alternative service, including no intervention, and is not more costly than an alternative service or sequence of services.

For these purposes, "generally accepted standards of medical practice" are standards based on reliable scientific evidence published in peer-reviewed medical literature generally recognized by the relevant medical community, physician specialty society recommendations, and the views of physicians practicing in relevant clinical areas, and other relevant factors. For new treatments, effectiveness is determined by reliable scientific evidence that is published in peer-reviewed medical literature. For existing treatments, effectiveness is determined first by scientific evidence, then by professional standards, then by expert opinion. The fact that services were furnished, prescribed or approved by a physician or other qualified provider does not in itself mean that services are medically necessary. The fact that a service is FDA-approved does not in itself mean that the service is medically necessary.

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; October 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