Policy Title:	Exondys 51 (eteplirsen) (Intravenous)		
		Department:	PHA
Effective Date:	12/04/2019		
Review Date:	12/4/2019		
Revision Date:	12/4/2019		

Purpose: To support safe, effective and appropriate use of Exondys 51 (eteplirsen).

Scope: Medicaid, Exchange, Medicare-Medicaid Plan (MMP)

Policy Statement:

Exondys 51 (eteplirsen) is covered under the Medical Benefit when used within the following guidelines. Use outside of these guidelines may result in non-payment unless approved under an exception process.

Procedure:

Coverage of Exondys 51 (eteplirsen) will be reviewed prospectively via the prior authorization process based on criteria below.

Initial Criteria:

- Patient has a diagnosis of Duchenne muscular dystrophy (DMD) by, or in consultation with, a neurologist with expertise in the diagnosis of DMD; AND
- Exondys 51 is prescribed by, or in consultation with, a neurologist with expertise in the treatment of DMD; AND
- Provider submits medical records (e.g., chart notes, laboratory values) confirming the mutation of the DMD gene is amenable to exon 51 skipping; AND
- Exondys 51 will only be covered for patients with the mutation of DMD gene that is amenable to the exon 51 skipping and Exondys 51 will NOT be covered for other forms of muscular dystrophy; AND
- Patient has been on a stable dose of corticosteroids for at least 6 months; AND
- Submission of medical records (e.g., chart notes, laboratory values) confirming that the patient has a 6- Minute Walk Time (6MWT) ≥ 300 meters while walking independently (e.g., without side-by-side assist, cane, walker, wheelchair, etc.) prior to beginning Exondys 51 therapy; AND
- Exondys 51 dosing for DMD is in accordance with the United States Food and Drug Administration approved labeling: maximum dosing of 30 mg/kg infused once weekly

Continuation of Therapy Criteria:

- Exondys 51 is prescribed by, or in consultation with, a neurologist with expertise in the treatment of DMD; AND
- Documentation of medical records (e.g., chart notes, laboratory values) demonstrating that the patient continues to have a 6-Minute Walk Time (6MWT) ≥ 300 meters while walking independently (e.g., without side-by-side assist, cane, walker, wheelchair, etc.). This must be measured no earlier than 4 weeks prior to a continuation request; AND
- Exondys 51 dosing for DMD is in accordance with the United States Food and Drug Administration approved labeling: maximum dosing of 30 mg/kg infused once weekly

Coverage durations:

- Initial coverage: 6 months
- Continuation of therapy coverage: 6 months

*** Requests will also be reviewed to National Coverage Determination (NCD) and Local Coverage Determinations (LCDs) if applicable.***

Dosage/Administration:

Indication	**Dose**	**Maximum dose (1 billable unit = *10* mg)**
Duchenne muscular dystrophy	30 mg/kg via intravenous infusion once weekly	350 billable units every week

Investigational use: All therapies are considered investigational when used at a dose or for a condition other than those that are recognized as medically accepted indications as defined in any one of the following standard reference compendia: American Hospital Formulary Service Drug information (AHFS-DI), Thomson Micromedex DrugDex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs, or Peer-reviewed published medical literature indicating that sufficient evidence exists to support use. Neighborhood does not provide coverage for drugs when used for investigational purposes.

Applicable Codes:
Below is a list of billing codes applicable for covered treatment options. The below tables are provided for reference purposes and may not be all inclusive. Requests received with codes from tables below do not guarantee coverage. Requests must meet all criteria provided in the procedure section.

The following HCPCS/CPT codes are:

HCPCS/CPT Code	Description
J1428	Injection, eteplirsen, 10 mg;

References:

1. Exondys 51 [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc, October 2018.
2. Anthony K, Feng L, Arechavala-Gomeza V, et al. Exon skipping quantification by quantitative reverse-transcription polymerase chain reaction in Duchenne muscular dystrophy patients treated with the antisense oligomer eteplirsen. Hum Gene Ther Methods. 2012 Oct;23(5):336-45.
3. Bushby K, Finkel R, Birnkrant DJ, Case LE, Clemens PR, Cripe L, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and pharmacological and psychosocial management. Lancet Neurol; 2010 Jan; 9(1):77-93.
4. Bushby K, Finkel R, Birnkrant DJ, et al. (2010) Diagnosis and management of Duchenne muscular dystrophy, part 2: implementation of multidisciplinary care. Lancet Neurol; 2010 Jan; 9(2):177-189.
5. Gold Standard, Inc. Exondys 51. Clinical Pharmacology [database online]. Available at: http://www.clinicalpharmacology.com. Accessed: February 8, 2018.
6. Mendell JR, Rodino-Klapac LR, Sahenk Z, et al. Eteplirsen for the treatment of Duchenne muscular dystrophy. Ann Neurol. 2013 Nov;74(5):637-47.
7. Sarepta Therapeutics. Confirmatory Study of Eteplirsen in DMD Patients (PROMOVI). In: ClinicalTrials.gov [Internet]. Bethesda (MD): National Library of Medicine (US). 2000- [cited 2017 Jan 27]. Available from: https://clinicaltrials.gov/show/NCT 02255552. NLM Identifier: NCT 02255552.
8. Mendell JR, Goemans N, Lowes LP, et al. Longitudinal effect of eteplirsen versus historical control on ambulation in Duchenne muscular dystrophy. Ann Neurol. 2016 Feb;79(2):257-71.
9. Kinane TB, Mayer OH, Duda PW, et al. Long-Term Pulmonary Function in Duchenne Muscular Dystrophy: Comparison of Eteplirsen-Treated Patients to Natural History. J Neuromuscul Dis. 2018;5(1):47-58.