Policy Title: Exondys 51 (eteplirsen) (Intravenous)

Department: PHA

Effective Date: 12/04/2019

Review Date: 12/4/2019, 1/29/20, 4/29/2021

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

Scope: Medicaid, Commercial, 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
- 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; AND
- Patient is not on concomitant therapy with other DMD-directed antisense oligonucleotides (e.g., eteplirsen, golodirsen, viltolarsen, etc.); AND
- Patient retains meaningful voluntary motor function (e.g., patient is able to speak, manipulate objects using upper extremities, ambulate, etc.); AND
- Patient should be receiving physical and/or occupational therapy; AND
- Baseline documentation of one or more of the following:
- Dystrophin level
- 6-minute walk test (6MWT) or other timed function tests
- Upper limb function (ULM) test
- North Star Ambulatory Assessment (NSAA)
- Forced Vital Capacity (FVC) percent predicted

- MMP members who have previously received this medication within the past 365 days are not subject to Step Therapy Requirements

**Continuation of Therapy Criteria:**

- Patient meets all initial criteria such as concomitant therapy requirements (not including prerequisite therapy); AND
- Exondys 51 is prescribed by, or in consultation with, a neurologist with expertise in the treatment of DMD; AND
- Patient has responded to therapy compared to pretreatment baseline in one or more of the following (not all-inclusive):
  - Increase in dystrophin level
  - Stability, improvement, or slowed rate of decline in 6MWT or other timed function tests
  - Stability, improvement, or slowed rate of decline in ULM test
  - Stability, improvement, or slowed rate of decline in NSAA
  - Stability, improvement, or slowed rate of decline in FVC% predicted
  - Improvement in quality of life; 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

<table>
<thead>
<tr>
<th>Indication</th>
<th>Dose</th>
<th>Maximum dose (1 billable unit = 10 mg)</th>
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<tbody>
<tr>
<td>Duchenne muscular dystrophy</td>
<td>30 mg/kg via intravenous infusion once weekly</td>
<td>350 billable units every week</td>
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### 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:

<table>
<thead>
<tr>
<th>HCPCS/CPT Code</th>
<th>Description</th>
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<tbody>
<tr>
<td>J1428</td>
<td>Injection, eteplirsen, 10 mg</td>
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### References: