

## Prior Authorization Criteria - Exondys 51 (eteplirsen)

### Approval Criteria

#### Initial 6-Month Approval Criteria:

1. Participant has a diagnosis of Duchenne Muscular Dystrophy (DMD) with a confirmed mutation of a DMD gene that is amenable to exon 51 skipping (provide genetic testing results). The treatment regimen prescribed is not for an indication outside of the FDA approved labeling, and no contraindications or significant drug interactions to treatment exist as specified in the product labeling.
2. Prescriber is either board certified in one of the following adult or pediatric specialties or subspecialties: Neurology, orthopedics, physical medicine and rehabilitation, neuromuscular medicine, neurodevelopmental disabilities, or provides a consultation report from one of such specialists or subspecialists who has recommended treatment with eteplirsen.
3. The following baseline documentation is provided:
  - a. Current weight.
  - b. 6-Minute Walk Test (6MWT) if participant is ambulatory.
  - c. Brooke Upper Extremity (BUE) Function score of  $\leq 5$ . OR
  - d. Stable pulmonary function with Forced Vital Capacity (FVC)  $\geq 30\%$  predicted.
  - e. Urinalysis showing absence of proteinuria.
  - f. Blood Urea Nitrogen (BUN) and Serum Cr.
4. Documentation of treatment with standard corticosteroid therapy for a minimum of 6 months, with evidence of adherence, or justification for discontinuation of standard therapy is supplied.
5. Prescriber must provide goals of therapy.
6. Dose does not exceed 30mg/kg body weight once weekly.

#### Renewal Criteria

Additional approvals may be granted for 6 months if the following criteria continue to be met:

1. Adherence to eteplirsen therapy is confirmed through pharmacy claims or submission of infusion records. Repeated nonadherence may result in denial of renewal request.

2. Clinic notes and objective evidence demonstrating clinical response to therapy are provided, including current:
  - a. Weight.
  - b. 6MWT if participant is ambulatory.
  - c. BUE Function score of  $\leq 5$ . OR
  - d. FVC  $\geq 30\%$  predicted.
  - e. Urinalysis showing absence of proteinuria.
  - f. BUN and Serum Cr.
  
3. Documentation of continued use of standard therapies previously initiated, if appropriate/tolerated, is provided.