EXONDYS 51™ (eteplirsen)

LENGTH OF AUTHORIZATION: SIX MONTHS

REVIEW CRITERIA:

- Patient must be at least 4 years of age.
- Patient must have the diagnosis of Duchenne muscular dystrophy (DMD).
- Submission of medical records (e.g., chart notes, laboratory values) as genetic test is required to confirm that a patient’s mutation of the DMD gene is amenable to exon 51 skipping.
- Medication is prescribed by or in consultation with a neurologist or a physician who specializes in treatment of DMD (i.e. pediatric neurologist, cardiologist or pulmonary specialist).
- If the patient is ambulatory, functional level determination of baseline assessment of ambulatory function (six-minute walk test) is required.
- If not ambulatory, patient must have a Brooke Upper Extremity Function Scale of five or less documented and a Forced Vital Capacity of 30% or more.

CONTINUATION OF THERAPY:

Documentation of improvement or maintenance:

- For ambulatory patients- Submission of six-minute walk test.
- For non-ambulatory patients- Submission of Brooke Upper Extremity Function Scale (five or less) documented and a Forced Vital Capacity documented (30% or more).

DOSSING: Maximum dose of 30mg/kg once weekly