

Clinical Oversight Review Board (CORB) Criteria for Prescribing Eteplirsen (Exondys 51)

Non-Formulary **eteplirsen (Exondys 51)** requires a clinical review. Appropriateness of therapy will be based on the following criteria:

Initiation (new start) criteria: Non-formulary **eteplirsen (Exondys 51)** will be covered on the prescription drug benefit for 12 months when the following criteria are met:

- Prescribed by or in consultation with a Pediatric Neurologist or a Pediatric Geneticist
- Documented Duchenne muscular dystrophy (DMD) diagnosis on the Problem List
- Documented DMD gene mutation that is expected to benefit from exon 51 skipping[&]
- Patient is 4 years old or older
- Patient can walk and is not dependent on a wheelchair (cane or walker use acceptable)
- Minimum distance for unassisted 6-minute walk test (6MWT) of 180 meters documented in medical record
- Patient is on a stable dose of glucocorticoid for at least 6 months
- If patient is 6 years old or older and able to complete spirometry testing, forced vital capacity (FVC; lung function test) of 50% or higher
- Patient can breathe without ventilator support (including without nocturnal BiPAP; nocturnal CPAP acceptable)
- Left ventricle ejection fraction of 50% or higher
- QTc[#] interval of 449 milliseconds or less
- Average heart rate at or below normal range for age as assessed via Holter monitor
- Patient has not received, or is planning to receive gene therapy for treatment of DMD
- Patient has been reviewed by the Kaiser Permanente Interregional Consultative Physician Panel, with recommendation to use medication

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Continued use criteria: Continued use will be contingent upon demonstrated response to therapy (to be reviewed every 12 months while on treatment). **Discontinuation is recommended in the following situations:**

- Ambulation level* is limited home or below for ambulation test
- Dependent level for sit to stand test**
- Patient requires ventilator support, including BiPAP (nocturnal CPAP acceptable)
- Patient has progressed to some level of wheelchair dependency (cane or walker use acceptable)
- Patient is non-adherent to Pulmonology tests^ (completed annually)
- Patient is non-adherent to echocardiogram (completed 6 months after initiation, then annually)

Notes:

- *Ambulation levels include: unable, limited home, home, limited community, or community independent
- ** Sit to stand test levels: dependent, moderate assist, or independent
- ^ Age 6 years or more: spirometry, pulse oximetry, End-Tidal CO₂ (ETCO₂), MIP/MEP, and cough peak flows; Age 5 years or less: pulse oximetry and ETCO