

STANDARD MEDICARE PART B MANAGEMENT

EXONDYS 51 (eteplirsen)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indication

Exondys 51 is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the *DMD* gene that is amenable to exon 51 skipping.

This indication is approved under accelerated approval based on an increase in dystrophin in skeletal muscle observed in some patients treated with Exondys 51. A clinical benefit of Exondys 51 has not been established. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

All other indications will be assessed on an individual basis. Submissions for indications other than those enumerated in this policy should be accompanied by supporting evidence from Medicare approved compendia.

II. DOCUMENTATION

The following documentation must be available, upon request, for all submissions:

- A. Initial requests: laboratory confirmation of Duchenne muscular dystrophy (DMD) diagnosis with a *DMD* gene mutation that is amenable to exon 51 skipping (refer to examples in Appendix)
- B. Continuation of therapy requests: documentation (e.g., chart notes) of response to therapy

III. CRITERIA FOR INITIAL APPROVAL

Duchenne Muscular Dystrophy

Authorization of 6 months may be granted for treatment of DMD when all of the following criteria are met:

- A. Genetic testing was conducted to confirm the diagnosis of DMD and to identify the specific type of *DMD* gene mutation.
- B. The *DMD* gene mutation is amenable to exon 51 skipping (refer to examples in Appendix).
- C. Treatment with Exondys 51 is initiated before the age of 14.
- D. Member is able to achieve an average distance of at least 180 meters while walking independently over 6 minutes.
- E. Member will not exceed a dose of 30 mg/kg

IV. CONTINUATION OF THERAPY

All members (including new members) requesting authorization for continuation of therapy must be currently receiving therapy with the requested agent.

Authorization for 12 months may be granted when all of the following criteria are met:

- A. The member is currently receiving therapy with Exondys 51.
- B. Exondys 51 is being used to treat an indication enumerated in Section III.
- C. The member is receiving benefit from therapy.
- D. The member will not exceed a dose of 30 mg/kg

V. APPENDIX

Examples of *DMD* gene mutations (exon deletions) amenable to exon 51 skipping

- 1. Deletion of exon 50
- 2. Deletion of exon 52
- 3. Deletion of exons 45-50
- 4. Deletion of exons 47-50
- 5. Deletion of exons 48-50
- 6. Deletion of exons 49-50

VI. REFERENCES

- 1. Exondys 51 [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc.; July 2020.
- 2. Mendell JR, Rodino-Klapac LR, Sahenk Z, et al. Eteplirsen for the treatment of Duchenne muscular dystrophy. *Ann Neurol*. 2013;74(5):637-47.
- 3. Cirak S, Arechavala-Gomeza V, Guglieri M, et al. Exon skipping and dystrophin restoration in patients with Duchenne muscular dystrophy after systemic phosphorodiamidate morpholino oligomer treatment: an open-label, phase 2, dose-escalation study. *Lancet*. 2011;378(9791):595-605.
- 4. Mendell JR, Goemans N, Lowes LP, et al; Eteplirsen Study Group and Telethon Foundation DMD Italian Network. Longitudinal effect of eteplirsen versus historical control on ambulation in Duchenne muscular dystrophy. *Ann Neurol*. 2016;79(2):257-271.
- 5. Randeree L, Eslick GD. Eteplirsen for paediatric patients with Duchenne muscular dystrophy: A pooled-analysis. *J Clin Neurosci*. 2018;49:1-6.