

SPECIALTY GUIDELINE 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 Indications

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 are considered experimental/investigational and are not a covered benefit.

II. REQUIRED DOCUMENTATION

The following information is necessary to initiate the prior authorization review:

Laboratory confirmation of DMD diagnosis with a *DMD* gene mutation that is amenable to exon 51 skipping (refer to Appendix)

III. CRITERIA FOR INITIAL APPROVAL

Duchenne Muscular Dystrophy

Indefinite authorization may be granted for treatment of DMD when all of the following criteria are met:

1. Genetic testing was conducted to confirm the diagnosis of DMD and to identify the specific type of *DMD* gene mutation
2. The *DMD* gene mutation is amenable to exon 51 skipping (refer to Appendix)

IV. CONTINUATION OF THERAPY

All members (including new members) requesting authorization for continuation of therapy must meet all initial authorization criteria.

V. DOSAGE AND ADMINISTRATION

Approvals may be subject to dosing limits in accordance with FDA-approved labeling, accepted compendia, and/or evidence-based practice guidelines.

VI. 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

VII. REFERENCES

1. Exondys 51 [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc.; September 2016.
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-Gomez 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.