Mass General Brigham
Health Plan

Exondys (eteplirsen)
Effective 01/01/2024

Plan	☐ MassHealth UPPL ☒Commercial/Exchange	**Program Type**	☒ Prior Authorization ☐ Quantity Limit ☐ Step Therapy
Benefit	☐ Pharmacy Benefit ☒ Medical Benefit		
Specialty Limitations	N/A		
Contact Information	**Medical and Specialty Medications** All Plans | Phone: 877-519-1908 | Fax: 855-540-3693 **Non-Specialty Medications** All Plans | Phone: 800-711-4555 | Fax: 844-403-1029		
Exceptions	N/A		

Overview

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 not medically necessary.

Coverage Guidelines

Authorization may be granted for members new to the plan who are currently receiving treatment with the requested medication, excluding when the product is obtained as samples or via manufacturer's patient assistance programs.

OR

Authorization may be granted when the following criteria is met:

1. Member has a diagnosis of Duchenne Muscular Dystrophy (DMD).
2. Genetic testing was conducted to confirm the diagnosis of DMD and to identify the specific type of *DMD* gene mutation.
3. Submission of documents supporting that the *DMD* gene mutation is amenable to exon 51 skipping (refer to examples in Appendix).
4. Treatment with Exondys 51 is initiated before the age of 14.
5. Member is able to achieve an average distance of at least 180 meters while walking independently over 6 minutes.
6. Member will not exceed a dose of 30 mg/kg.

7. The requested medication is prescribed by or in consultation with a physician who specializes in treatment of DMD.

Continuation of Therapy

Reauthorization may be granted for members requesting continuation of therapy when both of the following criteria are met:

1. The member has demonstrated a documented response to therapy as evidenced by remaining ambulatory (e.g., able to walk with or without assistance, not wheelchair dependent).
2. The member will not exceed a dose of 30 mg/kg.

Limitations

1. Initial approvals will be granted for 6 months.
2. Reauthorizations will be granted for 12 months.

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

References

1. Exondys 51 [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc.; October 2018.
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.

Review History

12/13/2023: Reviewed at Dec P&T, switched from SGM to Custom. Effective 1/1/2024