

<b>Clinical Policy Title:</b>	eteplirsen
<b>Policy Number:</b>	RxA.605
<b>Drug(s) Applied:</b>	Exondys 51™
<b>Original Policy Date:</b>	03/06/2020
<b>Last Review Date:</b>	12/07/2020
<b>Line of Business Policy Applies to:</b>	All lines of business

## Background

Eteplirsen (Exondys 51™) is an antisense oligonucleotide. 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.

Limitation(s) of use: This indication is approved under accelerated approval based on an increase in dystrophin in skeletal muscle observed in some patients treated with Exondys 51™. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

## Dosing Information

Drug Name	Indication	Dosing Regimen	Maximum Dose
eteplirsen (Exondys 51™)	DMD	30 mg/kg IV once weekly	30 mg/kg

## Dosage Forms

- Single-dose vial for injection: 100 mg/2 mL (50 mg/mL) and 500 mg/10 mL (50 mg/mL)

## Clinical Policy

Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

### I. Initial Approval Criteria

#### A. Duchenne Muscular Dystrophy (must meet all):

1. Diagnosis of DMD with mutation amenable to exon 51 skipping confirmed by genetic testing;
2. Prescribed by or in consultation with a neurologist;
3. Age 13 years or less at therapy initiation;
4. Member has all the following assessed within the last 30 days (a, b, and c):
  - a. Ambulatory function (e.g., ability to walk with or without assistive devices, not wheelchair dependent) with a 6-minute walk test (6MWT) distance  $\geq$  200 m;
  - b. Stable cardiac function with left ventricular ejection fraction (LVEF)  $>$  40%;
  - c. Stable pulmonary function with predicted forced vital capacity (FVC)  $\geq$  50%;
5. Inadequate response (as evidenced by a significant decline in 6MWT, LVEF, or FVC) despite adherent use of an oral corticosteroid (e.g., prednisone, Emflaza™) for  $\geq$  6 months, unless contraindicated or clinically

This clinical policy has been developed to authorize, modify, or determine coverage for individuals with similar conditions. Specific care and treatment may vary depending on individual need and benefits covered by the plan. This policy is not intended to dictate to providers how to practice medicine, nor does it constitute a contract or guarantee regarding payment or results. This document may contain prescription brand name drugs that are trademarks of pharmaceutical manufacturers that are not affiliated with RxAdvance.

- significant adverse effects are experienced;
- 6. Exondys 51™ is prescribed concurrently with an oral corticosteroid, unless contraindicated or clinically significant adverse effects are experienced;
- 7. Exondys 51™ is not prescribed concurrently with other exon-skipping therapies (e.g., Vyondys 53™);
- 8. Dose does not exceed 30 mg/kg per week.

**Approval Duration**

**Commercial:** 6 months

**Medicaid:** 6 months

**II. Continued Therapy Approval**

**A. Duchenne Muscular Dystrophy (must meet all):**

1. Member is currently receiving medication that has been authorized by RxAdvance, or member has previously met initial approval criteria listed in this policy;
2. Member is responding positively to therapy as evidenced by all of the following assessed within the last 30 days (a, b, and c):
  - a. Ambulatory function (e.g., ability to walk with or without assistive devices, not wheelchair dependent) with a 6-minute walk test (6MWT) distance  $\geq$  200 m;
  - b. Stable cardiac function with LVEF  $>$  40%;
  - c. Stable pulmonary function with predicted FVC  $\geq$  50%;
3. Exondys 51™ is prescribed concurrently with an oral corticosteroid, unless contraindicated or clinically significant adverse effects are experienced;
4. Exondys 51™ is not prescribed concurrently with other exon-skipping therapies (e.g., Vyondys 53);
5. If request is for a dose increase, new dose does not exceed 30 mg/kg per week.

**Approval Duration**

**Commercial:** 6 months

**Medicaid:** 6 months

**Appendices**

**APPENDIX A: Abbreviation/Acronym Key**

6MWT: 6-minute walk test  
 DMD: Duchenne muscular dystrophy  
 FDA: Food and Drug Administration  
 LVEF: Left ventricular ejection fraction  
 FVC: Forced vital capacity

**APPENDIX B: Therapeutic Alternatives**

*Below are suggested therapeutic alternatives based on clinical guidance. Please check drug formulary for preferred agents and utilization management requirements.*

Drug Name	Dosing Regimen	Dose Limit/ Maximum Dose
prednisone*	0.3-0.75 mg/kg/day or 10 mg/kg/weekend PO	Based on weight
Emflaza™ (deflazacort)	0.9 mg/kg PO once daily	Based on weight

*Therapeutic alternatives are listed as Brand name® (generic) when the drug is available by brand name only and generic (Brand name®) when the drug is available by both brand and generic. \*Off-label*

**APPENDIX C: Contraindications/Boxed Warnings**

- Contraindication(s):

- None reported
- Boxed Warning(s):
  - None reported

**APPENDIX D: General Information**

- Hypersensitivity reactions, including rash and urticaria, pyrexia, flushing, cough, dyspnea, bronchospasm, and hypotension, have occurred in patients who were treated with Exondys 51™. If a hypersensitivity reaction occurs, institute appropriate medical treatment and consider slowing the infusion or interrupting the Exondys 51™ therapy.

**References**

1. Exondys 51™ Prescribing Information. Cambridge, MA: Sarepta Therapeutics, Inc; July 2020. Available at [www.exondys51.com](http://www.exondys51.com). Accessed October 02, 2020.
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Review/Revision History	Review/Revision Date	P&T Approval Date
Policy established.	03/2019	03/06/2020
Policy was reviewed: <ol style="list-style-type: none"> <li>1. Policy title table was updated: Line of business policy applies was updated to All lines of business.</li> <li>2. Background was updated.</li> <li>3. Clinical criteria (both initial and continued) were added.</li> <li>4. Appendix A: LVEF and FVC</li> </ol>	10/02/2020	12/07/2020

<p>were added.</p> <ol style="list-style-type: none"><li>5. Appendix B was updated: Pre table phrase was updated to <i>“Below are suggested therapeutic alternatives..”</i>.</li><li>6. Appendix D: General Information was added.</li><li>7. References were updated.</li></ol>		
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