Clinical Policy: Eteplirsen

Reference Number: PA.CP.PHAR.288
Effective Date: 01/2018
Last Review Date: 01/19 Revision Log

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

FDA Approved Indication(s)
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. 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.

Policy/Criteria

It is the policy of Pennsylvania Health and Wellness® that eteplirsen (Exondys 51) is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria (must meet all):ju

A. Duchenne muscular dystrophy (DMD)

1. Diagnosis of Duchenne muscular dystrophy;
2. Prescribed by or in consultation with a neurologist with experience treating DMD;
3. The beneficiary has documentation of a baseline evaluation, including a standardized assessment of motor functions, by a neurologist with experience treating DMD;
4. The beneficiary will receive concurrent corticosteroids unless contraindicated or intolerant ;
5. The beneficiary does not meet the clinical review guidelines listed above, but, it the professional judgement of the physician reviewer, the services are medically necessary to meet he medical needs of the beneficiary.

Approval duration: 6 months

II. Continued Approval Criteria (must meet all):

A. Duchenne muscular dystrophy (DMD

1. Currently receiving medication via Pennyslvania Health and Wellness benefit, or member has previously met initial approval criteria; or the Continuity of Care policy (PA.LTSS.PHAR.01) applies;

2. Eteplirsen is prescribed by or in consultation with a neurologist with experience treating Duchenne muscular dystrophy;

3. The beneficiary has documentation of an annual evaluation, including an assessment of motor function ability, by a neurologist with experience treating DMD;
4. Based on the prescriber's assessment, the beneficiary continues to benefit from eteplirsen;
5. The beneficiary will receive concurrent corticosteroids unless contraindicated or intolerant;
6. The beneficiary does not meet the clinical review guidelines listed above, but, in the professional judgment of the physician reviewer, the services are medically necessary to meet the medical needs of the beneficiary.

III. Appendices/General Information

Appendix A: Abbreviation/Acronym Key
6MWT: 6-minute walk test
DMD: Duchenne muscular dystrophy
FDA: Food and Drug Administration

Appendix B: Therapeutic Alternatives
This table provides a listing of preferred alternative therapy recommended in the approval criteria. The drugs listed here may not be a formulary agent for all relevant lines of business and may require prior authorization.

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 QD	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
None reported

Background
Description/Mechanism of Action:
Eteplirsen is designed to bind to exon 51 of dystrophin pre-messenger ribonucleic acid (mRNA), resulting in exclusion of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 51 skipping. Exon skipping is intended to allow for production of an internally truncated dystrophin protein.

IV. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
DMD	30 mg/kg IV once weekly	30 mg/kg

V. Product Availability

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

Reviews, Revisions, and Approvals	Date	Approval Date
References reviewed and updated.		
1Q 2019 annual review: references reviewed and updated.	01/19	

References

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4. Mendell JR, Rodino-Klapac LR, Sahenk Z, et al. Eteplirsen for the treatment of Duchenne muscular dystrophy. Ann Neurol. 2013; 74: 637-647.
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