Clinical Policy: Eteplirsen

Reference Number: PA.CP.PHAR.288
Effective Date: 01/2018
Last Review Date: 07/18 Revision Log

Description
The intent of the criteria is to ensure that patients follow selection elements established by Pennsylvania Health and Wellness® clinical policy for eteplirsen (Exondys 51™).

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.

B. 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.

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.

Formulations:

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

FDA Approved Indications:

Exondys 51 (eteplirsen) is an antisense oligonucleotide / intravenous infusion indicated for:

- Treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping.

Limitations 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.

Appendices

Appendix A: Abbreviation Key

6MWT: 6-minute walk test
DMD: Duchenne muscular dystrophy
FDA: Food and Drug Administration
mRNA: messenger ribonucleic acid

Appendix B: Corticosteroid Regimens Used in DMD

- Prednisone 0.3-0.75 mg/kg/day
- Prednisone 10 mg/kg/weekend
- Deflazacort* 0.6 mg/kg/day for the first 20 days of each month
- Deflazacort* 0.9-1 mg/kg/day

**Deflazacort is not FDA-approved and is currently only available through an expanded access program.*

Reviews, Revisions, and Approvals	Date	Approval Date
References reviewed and updated.		

References

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iv. 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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2. Pennsylvania Medical Assistance Bulletin – Prior Authorization of Pharmaceutical Services - Exondys 51 (eteplirsen) dated June 6, 2017.