Exondys 51™ (eteplirsen)

Humana

Pharmacy Coverage Policy

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Line of Business: Medicare, Exchanges, Puerto Rico, Commercial, Medicaid
Policy Type: Prior Authorization

Disclaimer	Background
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Disclaimer

Description

Exondys 51 (eteplirsen) is an antisense oligonucleotide of the phosphorodiamidate morpholino oligomer (PMO) subclass.

Exondys 51 is designed to bind to exon 51 of dystrophin pre-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.

Exondys 51 (eteplirsen) 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.
Eteplirsen is available as Exondys 51100 mg/2mL injection solution in a single-dose vial

and 500 mg/10mL injection solution in a single-dose vial.

Coverage Determination

Please note the following regarding medically accepted indications:

All reasonable efforts have been made to ensure consideration of medically accepted indications in this policy. Medically accepted indications are defined by CMS as those uses of a covered Part D drug that are approved under the federal Food, Drug and Cosmetic Act, or the use of which is supported by one or more citations included or approved for inclusion in any of the compendia described in section 1927(g)(1)(B)(i) of the Act. These compendia guide review of off-label and off-evidence prescribing and are subject to minimum evidence standards for each compendium. Currently, this review includes the following references when applicable and may be subject to change per CMS:

- American Hospital Formulary Service-Drug Information (AHFS-DI)
- National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium
- Truven Health Analytics Micromedex DrugDEX
- Elsevier/Gold Standard Clinical Pharmacology
- Wolters Kluwer Lexi-Drugs

Exondys 51 (eteplirsen) will require prior authorization. This agent may be considered medically necessary when the following criteria are met:

Duchenne Muscular Dystrophy

- The member must have a diagnosis of Duchenne Muscular Dystrophy with a confirmed mutation of DMD gene that is amenable to exon 51 skipping documented by:
  - Multiplex ligation-dependent probe amplification (MLPA), OR
  - array comparative genomic hybridization (array CGH), OR
  - DMD gene sequencing
- The member must be ambulatory (e.g. able to walk with assistance, not wheelchair

dependent)

Recommended dosing: 30 mg/kg of body weight once weekly

Continuation of Therapy

- The member remains ambulatory (e.g. able to walk with assistance, not wheelchair dependent)

Intial approval: Exondys 51 (eteplirsen) will be approved for six months or as determined through clinical review.
Continuation of care: Exondys 51 (eteplirsen) will be approved for one year or as determined through clincal review.

Coverage Limitations

Exondys 51 (eteplirsen) therapy is not considered medically necessary for members with the following concomitant conditions:

- Experimental/Investigational Use – Indications not supported by CMS recognized compendia or acceptable peer reviewed literature.

Background

This is a prior authorization policy about Exondys 51 (eteplirsen):

Duchenne muscular dystrophy (DMD) is a progressive, genetic, life-threatening, degenerative neuromusclar disase that causes disabiling muscle weakness. DMD is caused by several gene mutations that disrupt the translational reading frame of the dystrophin mRNA and thus in the production of an unstable, nonfunctional dystrophin molecule. Dystrophin is thought to maintain the structural integrity of the muscle cell membrane by connecting the cytoskeleton to the surrounding extracellular matrix. The disease usually manifests in boys 3 to 7 years of age and commonly become wheelchair

dependent between 11 and 13 years of age. Eventually, respiratory failure occurs and patients usually develop cardiomyopathy before the age of 30. Premature death from respiratory or cardiac failure occur in the second to fourth decade. The estimated incidence of DMD is approximately 1 in 3500 to 5000 males born worldwide. The estimated prevalence of DMD was 1.02 per 10,000 males age 5 to 24 in 2010. Corticosteroid therapy is considered standard of care, delaying loss of ambulation and respiratory decline by several years.

The most common adverse reaction (incidence ≥ 35% and higher than placebo) were balance disorder and vomiting.

Provider Claims Codes

There are no provider claim codes associated with this policy.

Medical Terms

Exondys 51; eteplirsen; Duchenne Muscular Dystrophy; intravenous; pharmacy

References

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4. DRUGDEX® System [Internet database]. Greenwood Village, Colo: Thompson Reuters (Healthcare) Inc. Updated periodically. 2016.
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Duchenne Muscular Dystrophy. Ann Neurol 2013;74:647-647