Premier Health Plan

POLICY AND PROCEDURE

POLICY TITLE: ***Exondys 51 (eteplirsen)***
DEPARTMENT: **Clinical Pharmacy Services- Utilization Management**
ORIGINAL DATE: ***December 2016***

Last P & T Committee Approval Date: *February 2018*

Product Applicability: *mark all applicable products below:*

COMMERCIAL	[ ] HMO	[ ] PPO	*Products*: [ ] Small [ ] Indiv. [ ] Large	*Exchange*: [ ] Shop [ ] Indiv.	[x] All
OTHER	[x] Self-funded/ASO				

PURPOSE

The purpose of this policy is to define the prior authorization process for Exondys 51 (eteplirsen).

Exondys 51 (eteplirsen) is indicated for Duchenne muscular dystrophy (DMD) in patients with a confirmed mutation of the DMD gene that is amenable to exon 51 skipping.

DEFINITIONS

Duchenne muscular dystrophy (DMD) - is a rare, X-linked, recessive, life-threatening, degenerative neuromuscular disease affecting males. It is attributed to mutations in the DMD gene (chromosome Xp21), which is responsible for producing the protein dystrophin. Dystrophin is needed for proper muscle functioning and provides mechanical stability to muscle fibers during muscle contraction. The absence of or defect in this protein, leads to progressive muscle degeneration with loss of independent ambulation, as well as respiratory and cardiac complications.

POLICY

It is the policy of the Health Plan to maintain a prior authorization process that promotes

appropriate utilization of specific drugs with potential for misuse or limited indications. This process involves a review using Food and Drug Administration (FDA) criteria to make a determination of Medical Necessity, and approval by the Pharmacy & Therapeutics Committee of the criteria for prior authorization, as described in RX.002 Pharmacy and Therapeutics Committee and RX.003-Prior Authorization Process.

The drug, Exondys 51 (eteplirsen), is subject to the prior authorization process.

PROCEDURE

Initial Authorization Criteria:

Must meet all of the criteria listed below:

- Must be prescribed by a neurologist who specializes in the treatment of muscular dystrophy
- Must be male sex assigned at birth
- Must be age 7 years or older
- Must have a diagnosis of Duchenne muscular dystrophy
- Must have a mutation of the Duchenne muscular gene that is amenable to exon 51 skipping. Documentation of lab result confirming mutation is required.
- Must be ambulatory and able to walk 180-440 meters on the 6-minute walk test
- Must have an adequate trial of at least 1 year of corticosteroids or significant side effects/toxicity or have a contraindication to this therapy

Reauthorization Criteria:

All prior authorization renewals are reviewed on an <time frame> basis to determine the Medical Necessity for continuation of therapy. Authorization may be extended at one year based upon chart documentation from the prescriber that the member is still a candidate for treatment with Exondys 41 based upon the prescriber's assessment while on therapy.

Limitations:

Length of Authorization (if above criteria met)	
Initial Authorization	Up to 6 months
Reauthorization	Up to 1 year

If the established criteria are not met, the request is referred to a Medical Director for review.

REFERENCES

1. Exondys 51 [prescribing information]. Cambridge, MA Sarepta Therapeutics, Inc.; 2016.
2. Mendell JR, et al. Eteplirsen for the treatment of Duchenne muscular dystrophy. *Ann Neurol*. 2013;74(5):637-647.
3. Mendell JR, et al. . Longitudinal effect of eteplirsen versus historical control on ambulation in Duchenne muscular dystrophy. *Ann Neurol.* 2016;79(2):257-271.

RECORD RETENTION

Records Retention for Evolent Health documents, regardless of medium, are provided within the Evolent Health records retention policy and as indicated in CORP.028.E Records Retention Policy and Procedure.

REVIEW HISTORY

DESCRIPTION OF REVIEW / REVISION	DATE APPROVED
New policy	*12/16*
Annual Review	*02/17, 02/18*