RX.PA.009.MPC Exondys 51® (Eteplirsen) and Vyondys 53 (golodirsen)

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.

Vyondys 53 (golodirsen) 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 53 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.

The drugs, Exondys 51 (eteplirsen) and Vyondys 53 (golodirsen), are subject to the prior authorization process.

PROCEDURE
Initial Authorization Criteria:
Must meet all of the criteria listed below:

- **All products:**
  - Must be prescribed by a neurologist who specializes in the treatment of muscular dystrophy
  - Must be male sex assigned at birth
  - Must have a diagnosis of Duchenne muscular dystrophy (DMD)
  - 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
  - Must be prescribed at a dose within the manufacturer's dosing guidelines (based on diagnosis, weight, etc) listed in the FDA approved labeling
- **Exondys 51:**
  - Must be age 7 years or older
  - Must have a mutation of the Duchenne muscular gene that is amenable to exon 51 skipping. Documentation of lab result confirming mutation is required.
- **Vyondys 53:**
  - Must be age 6 years or older
  - Must have a mutation of the Duchenne muscular gene that is amenable to exon 53 skipping. Documentation of lab result confirming mutation is required.
  - Must include baseline renal function tests (GFR)

Reauthorization Criteria:

All prior authorization renewals are reviewed on an annual basis to determine the Medical Necessity for continuation of therapy. Authorization may be extended at 1-year based upon chart documentation from the prescriber that the member is still a candidate for treatment with the requested product based upon the prescriber's assessment while on therapy. For Vyondys 53 request, must also include documentation of recent renal function tests (GFR).

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, if required for the plan and level of request.

HCPCS Code(s):

Code	Description
J1428	Injection, eteplirsen, 10 mg

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.
4. Vyondys 53 [prescribing information]. Sarepta Therapeutics, Cambridge, MA, December 2019.