RX.PA.009.MPC Amondys 45® (casimersen), Elevidys (delandistrogene moxeparvove), Exondys 51® (Eteplirsen), Vyondys 53 (golodirsen) and Viltepso (viltolarsen)

The purpose of this policy is to define the prior authorization process for Amondys 45 (casimersen), Elevidys (delandistrogene moxeparvove), Exondys 51 (eteplirsen), Vyondys 53 (golodirsen) and Viltepso (viltolarsen).

Amondys 45 (casimersen) 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 45 skipping.

Elevidys (delandistrogene moxeparvove) is indicated for the treatment of ambulatory pediatric patients aged 4 through 5 years with Duchenne muscular dystrophy (DMD) with a confirmed mutation in the DMD gene.

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.

Viltepso (viltolarsen) 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, Amondys (casimersen), Elevidys (delandistrogene moxeparvove), Exondys 51 (eteplirsen), Vyondys 53 (golodirsen) and Viltepso (viltolarsen), are subject to the prior authorization process.

PROCEDURE

A. 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
- **Amondys 45:**
  - Must be ≤ 13 years of age at therapy initiation
  - Must have a mutation of the Duchenne muscular gene that is amenable to exon 45 skipping. Documentation of lab result confirming mutation is required.
  - Member will not be receiving concomitant exon-skipping therapies or gene therapies for DMD
- **Elevidys:**
  - Must be 4 to <6 years of age at therapy initiation
  - Must have a mutation of the Duchenne muscular gene. Must not have any deletion in exon 8 and/or exon 9 in the DMD gene. Documentation of lab result confirming mutation is required
  - Member must initiate a corticosteroid regimen prior to infusion and provider attests that treatment will continue for a minimum of 60 days after infusion
  - Provider attests that the required labs will be completed:
    - Monitor liver function before infusion and weekly for the first 3 months after infusion
    - Baseline testing for presence of anti-AAVrh74 total binding antibodies prior to infusion

    - Monitor troponin-I before infusion and weekly for the first month after infusion
    - Monitor platelet count before infusion and weekly for the first 2 weeks after infusion
  - Member will not be receiving exon-skipping therapies for DMD concomitantly or following Elevidys treatment
  - Member has not received prior Elevidys treatment.
- **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.
  - Member will not be receiving concomitant exon-skipping therapies or gene therapies for DMD
- **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.
  - Provider attests to monitoring renal function at baseline and during course of treatment
  - Member will not be receiving concomitant exon-skipping therapies or gene therapies for DMD
- **Viltepso:**
  - Must be ≤ 9 years of age at therapy initiation
  - 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 physical function tests:
    - Baseline 6-minute walk test (6MWT)≥ 300 meters while walking independently
      OR
    - Brooke Upper Extremity Scale (some useful hand function present for use of adaptive technology; score ≤ 5) OR
    - Force Vital Capacity Assessment ≥ 50%
  - Member does not have a diagnosis of medically intractable congestive heart failure
  - Member is not ventilator dependent
  - Member will not be receiving concomitant exon-skipping therapies or gene

therapies for DMD

B. Must be prescribed at a dose within the manufacturer's dosing guidelines (based on diagnosis, weight, etc) listed in the FDA approved labeling.

C. Amondys 45, Elevidys, Exondys 51, Vyondys 53, and Viltepso will be considered investigational or experimental for any other use and will not be covered.

D. <u>Reauthorization Criteria:</u>
***Note: Reauthorization is NOT applicable for Elevidys**

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:

MPC Renewal:

- 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.
- Improvement or stabilization of motor or pulmonary function from baseline (ex: 6MWT, Brooke Upper Extremity Scare, FVC)
- For Vyondys 53 request, must also include documentation of recent renal function tests (GFR). Must not have moderate to severe renal impairment. Baseline GFR > 30mL/min/m^2.

Renewal from Previous Insurer:

- Members who have received prior approval (from insurer other than MPC), or have been receiving medication samples, should be considered under criterion A (Initial Authorization Criteria)
- Provider has documented clinical response of the member's condition which has stabilized or improved based upon the prescriber's assessment

<u>Limitations:</u>

Length of Authorization (if above criteria met)	
Initial Authorization	Up to 6 months -Elevidys: 1-time approval per lifetime
Reauthorization	Up to 1 year -Elevidys: N/A

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
J1426	Injection, casimersen, 10 mg
J1427	Injection, viltolarsen, 10 mg
J1428	Injection, eteplirsen, 10 mg
J1429	Injection, golodirsen, 10 mg
J3590	Injection, delandistrogene moxeparvovec-rokl

REFERENCES

1. Elevidys (delandistrogene moxeparvovec) [prescribing information]. Cambridge, MA: Sarepta Therapeutics Inc; June 2023.
2. Exondys 51 [prescribing information]. Cambridge, MA Sarepta Therapeutics, Inc.; 2016.
3. Mendell JR, et al. Eteplirsen for the treatment of Duchenne muscular dystrophy. *Ann Neurol*. 2013;74(5):637-647.
4. Mendell JR, et al. Longitudinal effect of eteplirsen versus historical control on ambulation in Duchenne muscular dystrophy. *Ann Neurol.* 2016;79(2):257-271.
5. Vyondys 53 [prescribing information]. Sarepta Therapeutics, Cambridge, MA, December 2019.
6. Viltepso [prescribing information]. NS Pharma, Inc., Paramus, NJ, March 2021.
7. Amondys 45 [prescribing information]. Sarepta Therapeutics, Inc., Cambridge, MA, February 2021.

REVIEW HISTORY

DESCRIPTION OF REVIEW / REVISION	DATE APPROVED
Annual Review *Change in Non-MPC renewal to renewal from previous insurer*	*02/2024*
Addition of Elevidys and clinical criteria	*09/2023*
Annual review	*02/2023*
Annual review	*02/2022*
Addition of dosing requirements and off-label restriction	*12/2021*
P&T Review: Amondys 45 and Viltepso addition	*08/2021*
P&T Review	*11/2020*