Montana Healthcare Programs
Physician Administered Drug Coverage Criteria

VYONDYS 53® (golodirsen)

I. Medication Description

Vyondys 53® is an antisense oligonucleotide indicated for:

- Treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping.

II. Position Statement

Coverage is determined through a prior authorization process **that must include** supporting clinical documentation for each request.

III. Initial Coverage Criteria

Member must meet all the following criteria:

- Member must have Duchenne muscular dystrophy (DMD) with a confirmed mutation of the DMD gene that is amenable to exon 53 skipping.
    - The www.duchenneconnect.org website utilizes the following tool to find the genes amendable to exon 53 skipping: https://www.parentprojectmd.org/wp-content/exondeletiontool/
    - Genetic mutation test results must be submitted with request.
- Vyondys 53® must be prescribed by, or in consult with, a neurology specialist.
- Member must be on a stable dose of corticosteroids (prednisone, prednisolone, etc.) prior to starting Vyondys 53® unless corticosteroid use is contraindicated or was discontinued due to unfavorable side effects.
- Corticosteroids (prednisone, prednisolone, etc.) must be used concurrently with Vyondys 53® unless corticosteroid use is contraindicated or was discontinued due to unfavorable side effects.
- If ambulatory, baseline functional level assessment is required by one of the following:
    - Six-minute walk test (6MWT)
    - NorthStar Ambulatory Assessment
- If non-ambulatory, baseline functional level assessment is required by one of the following:
    - Revised Upper Limb Module (RULM)
    - Performance Upper Limb (PUL)
- Vyondys 53® is not used concomitantly with other exon skipping therapies for DMD.

IV. Renewal Coverage Criteria

Member must meet all the following criteria:

- Member has been adherent to Vyondys 53®.
- Corticosteroids must be used concurrently unless corticosteroid use is contraindicated or was discontinued due to unfavorable side effects.
- Functional level assessment must be completed every 6 months using the same rating scale utilized at baseline and must be submitted with the renewal request.

- Member is receiving a benefit from Vyondys 53® therapy as demonstrated by one of the following:
    - Stabilization or improvement compared to baseline functional level assessment utilizing the same rating scale submitted in initial approval.
    - Provider attests that member requires continued use of medication, despite not meeting improved baseline functional level assessment criteria and the benefits of continued use of medication outweigh the risks.
- Annual specialist consult provided if prescriber not a specialist.

V. Quantity Limitations

Max 30mg/kg IV once weekly

VI. Coverage Duration

Initial approval duration: 6 months

Renewal approval duration: 6 months