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:

- Must have DMD with a confirmed mutation of the DMD gene that is amenable to exon 53 skipping
  - The www.duchenneconnect.org website uses 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.
- Must be prescribed by or in consult with a neurology specialist
- 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 required by one of the following:
  - Six-minute walk test (6MWT)
  - NorthStar Ambulatory Assessment
- If non-ambulatory, baseline functional level assessment 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:

- 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 six months using the same rating scale used at baseline and submitted with renewal request.

- Is receiving a benefit from Vyondys 53® therapy, as demonstrated by one of the following:
    - Stabilization or improvement compared to baseline functional level assessment using the same rating scale submitted in initial approval.
    - Provider attests 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