

**SOUTH DAKOTA MEDICAID
PRIOR AUTHORIZATION CRITERIA**

Physician Administered Drugs, Vaccines, and Immunizations

Golodirsen (Vyondys 53) – PA Criteria

HCPC: J1429

Golodirsen (Vyondys 53) is an antisense oligonucleotide indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have confirmed genetic mutation of the DMD gene that is amenable to exon 53 skipping. It is covered with a prior authorization from South Dakota Medicaid when the following criteria are met:

** All requests under this policy require SD medical director review in addition to meeting specified criteria below **

- **Initial Therapy (must meet all):**
 - Therapy is prescribed by a neurologist with expertise in neuromuscular disorders
 - Individual must have a diagnosis of DMD with documentation of confirmed mutation that DMD gene is amenable to exon 53 skipping (submission of medical records, genetic testing, etc.)
 - If ambulatory, documentation of baseline values for **one** of the following is provided no longer than one month prior to beginning Vyondys 53
 - North Star Ambulatory Assessment (NSAA)
 - 6 Minute Walk Test (6MWT)
 - If non-ambulatory, documentation is submitted indicating Brooke upper extremity scale is ≤ 5
 - Documentation is submitted indicating forced vital capacity of $\geq 30\%$ and stable cardiac function with left ventricular ejection fraction (LVEF) of $>40\%$
 - Individual is not ventilator dependent
 - Therapy is not being used in conjunction with other exon skipping therapies for DMD (ie Exondys 51, Amondys 45, Viltepso)
 - Therapy is initiated before the age of 14
 - Individual has been on a stable dose of corticosteroids for 6 months unless contraindicated or adverse effects were previously experienced
 - Individual has not received previous gene therapy for the treatment of DMD
 - Approval duration: 6 months
- **Continuation of Therapy (must meet all):**
 - Individual continues to meet initial criteria
 - Individual will continue to have follow-up with neurology provider and/or neuromuscular clinic
 - Documentation of response to therapy is recorded every 6 months and shows stability or improvement in **both** of the following:
 - 6-minute walk test or NorthStar Ambulatory Assessment (or Brooke Upper Extremity if non-ambulatory)
 - Forced Vital Capacity
 - Approval duration: 6 months

A clinical benefit of VYONDYS 53 has not been established. VYONDYS 53 is FDA-approved 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. This indication is approved under accelerated approval based on an increase in dystrophin in skeletal muscle observed in some patients treated with VYONDYS 53. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.