REVIEW CRITERIA

LENGTH OF AUTHORIZATION: 5 doses/8 months

- Confirmed diagnosis of spinal muscular atrophy (SMA) confirmed by genetic testing.
  1. Documentation of genetic testing confirming either two or three copies** of SMN2 gene.
  2. Genetic testing confirms the presence of one of the following (a, b or c):
     a. Homozygous deletions of SMN1 gene (e.g., absence of the SMN1 gene);
     b. Homozygous mutation in the SMN1 gene (e.g., biallelic mutations of exon 7);
     c. Compound heterozygous mutation in the SMN1 gene (e.g., deletion of SMN1 exon 7 (allele 1) and mutation of SMN1 (allele 2))

- Medication is prescribed or in consultation with a pediatric neuromuscular specialist or a neurologist specializing in SMA.

- Obtain baseline assessment motor milestone score from ONE of the following assessments:
  - Hammersmith Functional Motor Scale Expanded (HFMSE)
  - Hammersmith Infant Neurologic Exam (HINE)
  - Upper limb module (ULM) score
  - Children’s Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND)
  - Six-minute walk test

- Platelet count, coagulation laboratory testing and quantitative spot urine protein testing are required at baseline and prior to each administration.

- Patient is not dependent on either of the following:
  - Invasive ventilation (for not more than 16 hours per day) or tracheostomy OR
  - Non-invasive ventilation for at least 12 hours per day
Specifically, for older patients with SMA and scoliosis, the drug may only be authorized if the patient has:

a. Scoliosis without spine surgery, or
b. Is post spine surgery with preserved window of accessibility, by intrathecal injection under fluoroscopic or ultrasound guidance if needed, or
c. Is post spine surgery (e.g., fusion) without window of accessibility with surgical placement of an indwelling catheter or establishment a new window for IT accessibility.

**CONTINUATION OF THERAPY**

**LENGTH OF AUTHORIZATION:** 8 months

**- Submission of most recent platelet count, coagulation laboratory testing and quantitative spot urine protein versus pretreatment baseline status.**

**- Documentation of positive response to therapy such as:**
  - Documentation that the patient is responding to the medication as demonstrated by clinically significant improvement or maintenance of function from pretreatment baseline status using the same exam as performed at baseline assessment (progression, stabilization, or decreased decline in motor function):

  **HFMSE:** One of the following:
  - Patient has achieved and maintained any new motor milestone from pretreatment baseline when they would otherwise be unexpected to do so.
  - Improvement or maintenance of previous improvement of at least a three-point increase in score from pretreatment baseline.

  **OR**

  **HINE milestones:** One of the following
  - Improvement or maintenance of previous improvement of at least two-point (or maximal score) increase in ability to kick.
  - Improvement or maintenance of previous improvement of at least one-point increase in any other HINE milestone (e.g., head control, rolling, sitting, crawling, etc.), excluding voluntary grasp.

  **AND**

  One of the following:
  - The patient exhibited improvement, or maintenance of previous improvement in more HINE motor milestones than worsening, from pretreatment baseline (net positive improvement).
  - Achieved and maintained any new motor milestones when they would otherwise be unexpected to do so (e.g., sit unassisted, stand, walk).
OR

**ULM:** One of the following:
- Improvement or maintenance of previous improvement of at least a two-point increase in score from pretreatment baseline.
- Patient has achieved and maintained any new motor milestone from pretreatment baseline when they would otherwise be unexpected to do so.

OR

**CHOP INTEND:** One of the following:
- Improvement or maintenance of previous improvement of at least a four-point increase in score from pretreatment baseline.
- Patient has achieved and maintained any new motor milestone from pretreatment baseline when they would otherwise be unexpected to do so.

OR

- Six-minute walk test

**DOSING:**

- Given intrathecally, the recommended dose is 12 mg (5 mL) per administration.

**Requests for patients with genetic testing confirming anything other than two or three copies of SMN2 gene will be evaluated on a case by case basis. Additional documentation regarding specific condition(s), age of onset, stage of decline, symptoms, etc., will be required for the prior authorization coverage determination.**