Connecticut Department of Social Services
Medical Assistance Program **Provider Bulletin 2017-38**
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TO: **Pharmacy Providers, Physicians, Nurse Practitioners, Physician Assistants, Clinics, Long Term Care Providers, and Hospitals**
RE: **Nusinersen Coverage Guidelines**

Effective May 1, 2017, the Department of Social Services (DSS) implemented a Prior Authorization (PA) requirement for prescription benefit coverage of Nusinersen, marketed as Spinraza™, for HUSKY A, HUSKY B, HUSKY C, HUSKY D, and Family Planning program clients.

The U.S. Food and Drug Administration (FDA) has approved Nusinersen for the treatment of spinal muscular atrophy (SMA) in pediatric and adult patients. Nusinersen is the first and only treatment approved in the U.S. for SMA, a leading genetic cause of death in infants and toddlers that is marked by progressive, debilitating muscle weakness.

PA requests for coverage of Nusinersen must be submitted by the prescriber in the form of a letter of medical necessity to the Department's Medical Director. Letters of medical necessity should be faxed to (860) 424-4822 with the required documentation outlined in the Nusinersen Coverage Guidelines.

The Nusinersen Coverage Guidelines document is attached below and will be available on the www.ctdssmap.com Web page under Pharmacy Information → Pharmacy Program Publications → Nusinersen Coverage Guidelines.

Please note that Nusinersen will be authorized for a period of 6 months and will require a re-authorization after the initial PA ends.

Nusinersen Coverage Guidelines

Prior authorization is required for all Nusinersen prescriptions.

Coverage guidelines for Nusinersen are made in accordance with the Department of Social Services (DSS) definition of Medical Necessity, as established by state law. The following factors are guidelines *only.* Coverage determinations are based on an assessment of the individual and his or her unique clinical needs. If the guidelines conflict with the definition of Medical Necessity, the definition of Medical Necessity shall prevail.

The following factors will be considered in the Department's prior authorization decision

I. Type 1 Patients

SMA Type 1 patients with symptom onset at < 6 months of age are eligible for treatment if the following conditions are met:

1) The Diagnosis of SMA1 must be made by a Neurologist with expertise in diagnosing Spinal Muscular Atrophy. For purposes of this guideline, "Neurologist" refers to a Board Certified Neurologist, preferably specializing in pediatric neurology, **AND**

- Genetic testing confirming **both**:
    - Mutation/deletion in chromosome 5q with
        - Homozygous gene deletion/mutation of exon 7 at 5q13 *or* compound heterozygous mutation: deletion SMN1 exon 7 allele1 and mutation of SMN1 allele 2
    - And at least two copies of SMN2; AND

2) Documentation that the patient is either **not on any artificial ventilation,** or if on artificial ventilation or other mechanical respiratory support prior to **Nusinersen,** the type, duration and degree of ventilator support in a 24 hour period must be documented; AND

3) Submission of medical records documenting a **baseline motor exam by a physician (Neurologist or PMR (Physical Medicine & Rehabilitation Specialist)) or physical therapist specializing in SMA motor exam evaluations and supervised by Neurologist or PMR physician)** experienced in treating SMA and utilizing at least one of the following exam instruments (based on patient's age), to establish this baseline motor ability**:**

- Hammersmith Infant Neurological Exam (HINE)
- Hammersmith Functional Motor Scale Expanded (HFMSE)
- Upper Limb Module Test (non-ambulatory)
- Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND); AND

4) **Nusinersen** must be **ordered by a Neurologist** experienced in treating SMA.

Initial approval of Nusinersen will be for 5 doses ONLY to be given in accordance with the current Nusinersen FDA label instructions**.** Protocol: loading doses at day 0 (dose 1), day 14 (dose 2), day 28 (dose 3), 30 days post day 28 (dose 4) and 4 months after that fourth dose (dose 5).

Guidelines for subsequent doses of Nusinersen:

After the 5 loading doses are completed, patient must be **re-evaluated using the same motor exam test done to establish baseline motor ability** unless it is determined that the original exam instrument

is no longer age-appropriate. This re-examination must be done, whenever possible, by the same examiner as baseline exam. If this is not possible, then the re-examination must be done by another physician (Neurologist or PMR who is experienced in treating SMA or a physical therapist specializing in SMA motor exam evaluations and supervised by Neurologist or PMR physician).

- Nusinersen will be authorized for an additional 6 months if a patient is determined to be a responder by demonstrating an **improved motor ability** in repeat motor testing after the 5th loading dose.
  To be classified as a responder, the patient should receive the following score(s) on the motor test used:

  HINE: a 2 point increase (or max score of 4) in ability to kick (consistent with improvement by at least 2 milestones) or a 1 point increase in motor milestones of head control, rolling, sitting, crawling, standing or walking (consistent with improvement by at least 1 milestone). Responder needs to exhibit improvement in more categories of motor milestones than worsening.

  HFMSE: (Scored as 0, 1 or 2 with maximum of 40). Improvement of at least a 3 point increase in score from pretreatment baseline

  ULM: Improvement of at least 2 point increase in score from pretreatment baseline
  CHOP INTEND: Improvement of at least 4 point increase in score from pretreatment baseline
- **Renewal authorization,** will be for 6 months. Nusinersen administration will follow the current FDA Nusinersen labelling for maintenance dosing protocol of every 4 months
- **Repeat motor testing** must be done at 6 month intervals and must show additional motor improvement or maintenance of the previously demonstrated motor improvement. If a patient becomes dependent on mechanical ventilator (defined as requiring mechanical ventilation for > 21 days) while on Nusinersen, then the Department will no longer authorize payment of Nusinersen for that patient.

  The 6 month periodic re-examination must be done by the same examiner as the baseline exam whenever possible. If this is not possible, it must be conducted by another physician (Neurologist or PMR) who is experienced in treating SMA who must use the same exam instrument unless it is determined that original exam instrument is no longer age appropriate.

***II. Other SMA Types* - Patients with other SMA Types are eligible for treatment if the following conditions are met:**

1) A diagnosis of SMA must be made by a Neurologist with expertise in diagnosing Spinal Muscular Atrophy; AND

2) All of the documentation listed above will be required; AND

3) **<u>In addition,</u>** documentation must be provided demonstrating in the opinion of the treating neurologist, why a patient should be considered for administration of Nusinersen given the research demonstrating efficacy is limited and equivocal.

If approved for a patient with a non-Type 1 SMA diagnosis, the dosage and renewal guidelines described above will also apply.