Pharmacy Medical Necessity Guidelines: Spinraza (nusinersen)

Effective: December 14, 2020

Prior Authorization Required	√	Type of Review – Care Management	
Not Covered		Type of Review – Clinical Review	√
Pharmacy (RX) or Medical (MED) Benefit	MED	Department to Review	PRECERT/ MM

These pharmacy medical necessity guidelines apply to the following:

Commercial Products

☒ Tufts Health Plan Commercial products – large group plans
☒ Tufts Health Plan Commercial products – small group and individual plans
☒ Tufts Health Freedom Plan products – large group plans
☒ Tufts Health Freedom Plan products – small group plans
- CareLink℠ – Refer to CareLink Procedures, Services and Items Requiring Prior Authorization

Tufts Health Public Plans Products

☒ Tufts Health Direct – A Massachusetts Qualified Health Plan (QHP) (a commercial product)
☒ Tufts Health Together – MassHealth MCO Plan and Accountable Care Partnership Plans
☒ Tufts Health RITogether – A Rhode Island Medicaid Plan

Fax Numbers:

Commercial Products:
PRECERT: 617.972.9409

Tufts Health Public Plans Products:
MM: 888.415.9055

Note: This guideline does not apply to Medicare Members (includes dual eligible Members).

OVERVIEW

FOOD AND DRUG ADMINISTRATION-APPROVED INDICATION(S)

Spinraza (nusinersen) is a survival motor neuron-2-directed antisense oligonucleotide indicated for the treatment of spinal muscular atrophy (SMA) in pediatric and adult patients.

Spinraza (nusinersen) is the first and only medication indicated for the treatment of SMA. SMA is a rare genetic neuromuscular disease characterized by a mutation in the survival motor neuron (SMN) 1 gene which is responsible for producing SMN protein. SMN protein maintains the health and normal function of motor neurons in the central nervous system (CNS). Patients with SMA will have mutations in both copies of the SMN1 gene which results in decreased production of SMN protein. Patients will experience progressive muscle weakness and atrophy. Often the muscles closest to the center of the body (e.g., shoulders, thighs, pelvis) are affected. All SMA patients have at least one SMN2 back up gene that will also produce SMN protein, but only 10% of it is fully functional. The low level of SMN protein produced by SMN2 genes is not enough to sustain the survival of motor neurons in the CNS. While it is believed the higher number of SMN2 genes the less severe symptoms, treatment decisions should be based on functional ability vs number of SMN2 genes alone. Symptoms of SMA will vary among individual patients and are based on the onset and disease severity.

Approval of Spinraza (nusinersen) was based on efficacy demonstrated in symptomatic infantile-onset SMA patients and presymptomatic and symptomatic SMA pediatric patients. In a randomized, double-blind trial, Spinraza (nusinersen) was compared to placebo in symptomatic infants (≤7 months of age at the time of first dose) diagnosed with SMA (symptom onset before 6 months of age) (N=121). The primary endpoint was the proportion of responders (defined as at least a 2-point increase in ability to kick or at least a 1-point increase in the motor milestones of head control, rolling, sitting, crawling, standing or walking based on Section 2 of the Hammersmith Infant Neurologic Exam). Results demonstrated that of the 82 patients eligible for the interim analysis, a statistically significant greater percentage of patients achieved a motor milestone response in the Spinraza (nusinersen) group compared to the placebo group. An open-label, uncontrolled trial conducted in symptomatic SMA pediatric patients (age range, 30 days to 15 years at the time of first dose) and presymptomatic patients (age range, 8 days to 42 days at the time of first dose) who were likely to develop Type 1, 2, or 3 SMA demonstrated that some Spinraza (nusinersen)-treated patients achieved milestones (ability to sit unassisted, stand, or walk) when they would otherwise not be expected to do so. In addition, for some Spinraza (nusinersen)-treated patients, milestones were maintained at ages when they would be expected to be lost and patients survived to ages that were unexpected considering the number of SMN2 gene copies. The number of patients enrolled in this trial and its duration are unknown.

Spinraza (nusinersen) is administered via intrathecal injection by, or under the direction of, healthcare professionals with experience performing lumbar punctures. In addition, because of the nature of SMA and the route of administration, sedation, ultrasound, and other imaging techniques to guide intrathecal administration may be considered. The recommended dosage of Spinraza (nusinersen) is 12 mg per administration. Treatment is initiated with four loading doses. The first three loading doses should be administered at 14-day intervals and fourth is administered 30 days after the third loading dose. A maintenance dose should be administered once every four months thereafter.

COVERAGE GUIDELINES

The plan may authorize coverage of Spinraza (nusinersen) for Members, when all of the following criteria are met:

Treatment-naïve

1. Documented diagnosis of spinal muscular atrophy (SMA) type 1, 2, or 3 confirmed by molecular genetic testing of any of the following: SMN1 homozygous gene deletion, homozygous conversion mutation (i.e., SMN1 gene conversion to SMN2 gene), or compound heterozygote mutation

AND

2. The prescribing physician is a neurologist

AND

3. Documentation of baseline (pre-treatment) motor function skills

AND

4. Documentation the Member has not been previously treated with gene therapy, for example, Zolgensma (onasemnogene abeparvovec-xioi)

Treatment-experienced

1. Documented diagnosis of SMA type 1, 2, or 3 confirmed by molecular genetic testing

AND

2. The prescribing physician is a neurologist

AND

3. Documentation of disease stabilization or clinical improvement of SMA symptoms (e.g., limb and trunk weakness; hypotonia and impaired head control; difficulty breathing, swallowing, feeding, and handling secretions

AND

4. Documentation the Member has not been previously treated with gene therapy, for example, Zolgensma (onasemnogen abeparvovec-xioi)

LIMITATIONS

- Coverage of Spinraza (nusinersen) for SMA type 4 will not be authorized.
- Authorizations will be provided for 12 months.
- For treatment-experienced Members, Providers must submit documentation of a physical assessment, motor function-based testing, and need for medical intervention related to SMA symptoms, relative to baseline.
- Coverage of Spinraza (nusinersen) will not be authorized for Members who have been previously treated with gene therapy, for example Zolgensma (onasemnogene abeparvovec-xioi).
- If gene therapy is subsequently administered, Spinraza authorization will be terminated and the Member must reapply for coverage.

CODES

The following HCPCS/CPT code(s) are:

Code	Description
J2326	Injection, nusinersen, 0.1 mg

REFERENCES

1. Chiriboga CA, Swoboda KJ, Darras BT, et al. Results from a phase 1 study of nusinersen (ISIS-SMN(Rx)) in children with spinal muscular atrophy. Neurology. 2016;86(10):890-897
2. CureSMA. SPINRAZA (Nusinersen) URL: curesma.org/spinraza/. Available on Internet: Accessed 2018 May 17.
3. Finkel RS, Chiriboga CA, Vajsar J, et al. Treatment of infantile-onset spinal muscular atrophy with nusinersen: A phase 2, open-label, dose-escalation study. Lancet. 2017;388(10063):3017-3026.
4. Haché M, Swoboda KJ, Sethna N, et al. Intrathecal injections in children with spinal muscular atrophy: Nusinersen clinical trial experience. J Child Neurol. 2016;31(7):899-906.

5. Mercuri E, Darras BT, Chiriboga CA, et al. Nusinersen versus sham control in later-onset spinal muscular atrophy. N Engl J Med. 2018;378:625-35.
6. Spinraza (nusinersen) [prescribing information]. Cambridge, MA: Biogen Inc; October 2018.
7. Wang CH, Finkel RS, Bertini ES, et al. Consensus statement for standard of care in spinal muscular atrophy. J Child Neurol. Aug 2007;22(8):1027-49.
8. Zolgensma (onasemnogene abeparvovec-xioi)[prescribing information]. Bannockburn, IL: AveXis, Inc; May 2019.

APPROVAL HISTORY

June 13, 2017: Reviewed by Pharmacy & Therapeutics Committee.

Subsequent endorsement date(s) and changes made:
1. January 1, 2018: Administrative update: Added new J code J2326 to Medical Necessity Guideline and removed expired C code C9489.
2. June 12, 2018: No changes.
3. March 12, 2019: No changes.
4. July 9, 2019: Effective October 1, 2019, added criteria requiring documentation the Member has not been previously treated with gene therapy and the limitations stating coverage would not be authorized for members who have been previously treated with gene therapy (for example, Zolgensma (onasemnogene abeparvovec-xioi)), and coverage would be terminated if gene therapy is administered, as the drug has not been studied in combination.
5. November 12, 2019: Effective April 1, 2020, moved reauthorization criteria from the Limitations section to the Coverage Guidelines section to ensure appropriate application of criteria.
6. December 8, 2020: No changes.

BACKGROUND, PRODUCT AND DISCLAIMER INFORMATION

Pharmacy Medical Necessity Guidelines have been developed for determining coverage for plan benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. The plan makes coverage decisions on a case-by-case basis considering the individual member's health care needs. Pharmacy Medical Necessity Guidelines are developed for selected therapeutic classes or drugs found to be safe, but proven to be effective in a limited, defined population of patients or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in the service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. The plan revises and updates Pharmacy Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests needed revisions.

For self-insured plans, coverage may vary depending on the terms of the benefit document. If a discrepancy exists between a Pharmacy Medical Necessity Guideline and a self-insured Member’s benefit document, the provisions of the benefit document will govern.

Treating providers are solely responsible for the medical advice and treatment of members. The use of this policy is not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to member eligibility and benefits on the date of service, coordination of benefits, referral/authorization and utilization management guidelines when applicable, and adherence to plan policies and procedures and claims editing logic.