



SmartPA Criteria Proposal

Drug/Drug Class:	Spinraza® Clinical Edit
First Implementation Date:	January 30, 2020
Revised Date:	June 4, 2020
Prepared for:	MO HealthNet
Prepared by:	MO HealthNet/Conduent
Criteria Status:	<input type="checkbox"/> Existing Criteria <input checked="" type="checkbox"/> Revision of Existing Criteria <input type="checkbox"/> New Criteria

Executive Summary

Purpose: Ensure appropriate utilization and control of Spinraza® (nusinersen)

Why Issue Selected: Spinal muscular atrophy (SMA) is a rare, debilitating neuromuscular disease. It is a progressive muscle wasting disease that can have a devastating impact on infants, children, and their families. SMA has an incidence of approximately 1 in 11,000 live births and is the leading genetic cause of infant mortality. The disease mainly affects the motor neurons in the spinal cord and is characterized by motor neuron degeneration, muscle weakness, and atrophy, but it is not believed to impact a person's capacity to think, learn, and build interpersonal relationships. Among SMA Types (Type 0 – Type 4), clinical classification is typically based on age of onset and maximum motor function achieved. Patients experience motor function decline with disease progression.

Spinraza® is a survival motor neuron-2 (SMN2)-directed antisense oligonucleotide indicated for the treatment of spinal muscular atrophy (SMA) in pediatric and adult patients. The drug is administered intrathecally. Numerous studies, including ENDEAR, NURTURE, and CHERISH support the effectiveness of nusinerin across a range of SMA patients, from presymptomatic infants, infantile-onset SMA, to children with later onset SMA (most likely to develop Type 2 or Type 3). Studies are ongoing (e.g. EMBRACE and SHINE) to assess the long-term effect of nusinersen. All requests for therapy will be reviewed by a Clinical Consultant.

Program-Specific Information:

SPINRAZA 12 MG/5 ML VIAL	20	\$2,525,190.00	\$126,259.50
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Type of Criteria: Increased risk of ADE Preferred Drug List
 Appropriate Indications Clinical Edit

Data Sources: Only Administrative Databases Databases + Prescriber-Supplied

Setting & Population

- Drug class for review: Spinraza® (nusinersen)

- Age range: All appropriate MO HealthNet participants

Approval Criteria

Initial Approval Criteria:

- Documentation of a confirmed diagnosis of Spinal Muscular Atrophy (SMA) including genetic tests of 5q13 demonstrating:
 - Homozygous SMN1 gene deletion or mutation **OR**
 - Compound heterozygous SMN1 gene mutation **AND**
- Sufficient number of copies of SMN2 gene defined as one of the following (either 2a or 2b) genetic tests demonstrating:
 - If a pre-symptomatic infant: ≤ 3 copies of SMN2 gene **OR**
 - If a symptomatic patient:
 - ≥ 2 copies of SMN2 gene **AND**
 - documentation of age of onset of symptoms **AND**
- Patient is under the supervision and monitoring of an appropriate specialist for the treated disease state **AND**
- Clinical baseline documentation received:
 - For all participants:
 - Pulmonary Status (e.g. tracheostomy, hrs of ventilation, CPAP, etc.) **AND**
 - Complete blood count, **cystatin-C**, coagulation status, urine protein, serum electrolytes including bicarbonate, liver and renal function tests **AND**
 - For participants aged < 3 years: Hammersmith Infant Neurological Exam-Part 2 (HINE-2) **OR**
 - For participants aged ≥ 3 years: Hammersmith Functional Motor Scale Expanded (HFMSE) **AND**
 - For ambulatory patients: 6 Minute Walk Test (6MWT) **OR**
 - For non-ambulatory patients: Revised Upper Limb Module (RULM) Score
- Initial approval is for 6 months

Renewal Approval Criteria:

- Documented compliance on current therapy regimen **AND**
- Absence of unacceptable toxicity from the drug (examples of unacceptable toxicity include serious infections, fatal glomerulonephritis, thrombocytopenia, etc) **AND**
- Documentation of benefit from therapy:
 - Improvement or maintenance of functional status from baseline functional tests (HFMSE or HINE-2, Pulmonary status, and 6MWT or RULM) **OR**
 - Achievement and maintenance of new motor milestones from pretreatment baseline functional tests (HFMSE or HINE-2 and Pulmonary status) **OR**
 - Less than expected decline in functional ability or symptoms of disease as described by at least 1 of the following:
 - HFMSE: at least 3 points increase in score from pretreatment baseline **OR**
 - HINE-2 demonstrates:
 - Patient has demonstrated improvement in more categories than decline **AND**
 - At least 2 points (or maximum score) in ability to kick **OR**
 - At least 1 point in any other HINE milestone (head control, rolling, sitting, crawling, etc.)
 - For ambulatory patients 6MWT demonstrates at least a 30 meter increase from pretreatment baseline
 - For non-ambulatory patients RULM demonstrates at least a 2 point increase in score from the pretreatment baseline
- Renewal request, including documentation, is required every 12 months
- Maintenance dosing is every 4 months

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Denial Criteria

- Therapy will be denied if no approval criteria are met

Required Documentation

Laboratory Results:

Progress Notes:

MedWatch Form:

Other:

Disposition of Edit

Denial: Exception code "0682" (Clinical Edit)

Rule Type: CE

Default Approval Period

6 months

References

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- Sugarman EA, et al. Pan-ethnic carrier screening and prenatal diagnosis for spinal muscular atrophy: clinical laboratory analysis of >72,400 specimens. *Eur J Hum Genet.* 2012;20(1):27–32. doi: 10.1038/ejhg.2011.134.
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- E. Mercuri et al. Diagnosis and management of spinal muscular atrophy: Part 1: Recommendations for diagnosis, rehabilitation, orthopedic and nutritional care. *Neuromuscular Disorders* 28 (2018) 103–115. <https://doi.org/10.1016/j.nmd.2017.11.005>
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- Spinraza [prescribing information]. Cambridge, MA. Biogen Inc. June 2019.
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