Reference number(s)
1834-A

SPECIALTY GUIDELINE MANAGEMENT

SPINRAZA (nusinersen)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications
Spinraza is indicated for the treatment of spinal muscular atrophy (SMA) in pediatric and adult patients.

All other indications are considered experimental/investigational and are not a covered benefit.

II. REQUIRED DOCUMENTATION

The following information is necessary to initiate the prior authorization review: Deletion or mutation at the SMN1 allele confirmed by genetic testing.

III. PRESCRIBER SPECIALTIES

This medication must be prescribed by or in consultation with a neurologist or neuromuscular specialist.

IV. CRITERIA FOR INITIAL APPROVAL

Authorization of 4 months may be granted for treatment of SMA when all of the following criteria are met:
A. Member has a diagnosis of SMA confirmed by genetic testing showing deletion or mutation at the SMN1 allele.
B. Member has Type 1, Type 2 or Type 3 SMA.
C. The diagnosis was made at or before 18 years of age.
D. Member is not on invasive or noninvasive ventilation support for more than 6 hours a day.
E. If the patient has not received a loading dose, the loading dose will be dosed at 12 mg (5ml) on Day 0, 14, 28, and 58.

V. CONTINUATION OF THERAPY

Authorization of 12 months may be granted for members (including new members) when all of the following criteria are met:
A. Member meets initial authorization criteria
B. Member is receiving a clinical benefit from Spinraza therapy, as demonstrated by improvement or maintenance of motor skills or ability to sit, crawl, stand or walk, or new motor milestones
C. If patient has already received a loading dose, the maintenance dose will not exceed 12 mg (5 mL) every 4 months

VI. REFERENCES

1. Spinraza [package insert]. Cambridge, MA: Biogen Inc.; December 2017.
2. Arnold WD, Kassar D, Kissel JT, et al. Spinal muscular atrophy: diagnosis and management in a new therapeutic era. *Muscle & Nerve*. 2015;51(2):157-167.
3. Burgunder JM, Schols L, Baets J, et al. EFNS guidelines for the molecular diagnosis of neurogenetic disorders: motoneuron, peripheral nerve and muscle disorders. *European J Neurol*. 2011;18:207-217.
4. 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*. 2016;388:3017-26.
5. Ionis Pharmaceuticals, Inc. A Study to Assess the Efficacy and Safety of IONIS-SMN Rx in Infants with Spinal Muscular Atrophy. In: ClinicalTrials.gov [internet]. Bethesda (MD): National Library of Medicine (US). 2000- [2016 Feb 14]. Available from: https://clinicaltrials.gov/ct2/show/NCT02193074.
6. Ionis Pharmaceuticals, Inc. A Study to Assess the Efficacy and Safety of IONIS-SMN Rx in Patients with Later-onset Spinal Muscular Atrophy. In: ClinicalTrials.gov [internet]. Bethesda (MD): National Library of Medicine (US). 2000- [2016 Feb 14]. Available from: https://clinicaltrials.gov/ct2/show/NCT02292537.
7. Wang CH, Finkel RS, Bertini ES, et al. Consensus statement for standard care in spinal muscular atrophy. *J Child Neurol*. 2007;22(8):1027-1049.