ILLINOIS DEPARTMENT OF Healthcare and Family Services

Prior Authorization Criteria-Spinraza (nusinersen)

Approval Criteria

Initial Approval Criteria

An initial approval for a period of 6 months to allow for 4 loading doses (Day 0, 14, 28, 58) and 1 maintenance dose, may be granted if the following criteria are met:

1. Participant was diagnosed at < 6 months of age with spinal muscular atrophy (SMA) type I, confirmed with documentation showing:

    a. The mutation or deletion of SMN1 genes in chromosome 5q resulting in one of the following:

        i. Homozygous SMN1 gene deletion or mutation (e.g. homozygous deletion of exon 7 at locus 5q13).
        OR
        ii. Compound heterozygous mutation of SMN1 (e.g. deletion of SMN1 exon 7 [allele 1] and mutation of SMN1 [allele 2).
        AND

    b. Participant has ≤ 2 copies of the SMN2 gene.

2. Prescriber is an enrolled IL Medicaid provider with expertise in the diagnosis and treatment of SMA (i.e. Board Certified in one of the following specialties/subspecialties - Adult Neurology, Pediatric Neurology Neurodevelopmental Disabilities, Neuromuscular medicine, Physical Medicine and Rehabilitation, Pediatric Rehabilitation Medicine, Developmental and Behavioral Pediatrics, Neonatal-Perinatal Medicine, Clinical Genetics and Genomics, or Molecular Genetic Pathology) and has personally examined and evaluated the patient within the past 30 days.
3. Participant does not have a tracheostomy and is not ventilator dependent.

4. Documentation of ≥ 1 baseline motor milestone exam, appropriate for participant age and motor function, is submitted:
    a. Hammersmith Infant Neurological Exam (HINE) = Infant to early childhood

b. Hammersmith Functional Motor Scale Expanded (HFMSE)
c. Upper Limb Module (ULM) = non-ambulatory
d. Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND)

5. In the opinion of the prescriber, the parent or guardian is able to make appropriate decisions about treatment and ensure adherence with treatment regimen and required lab work.

6. Prescriber provides documentation of baseline platelet count, prothrombin time, activated partial thromboplastin time and quantitative spot urine protein testing within 30 days before the request and agrees to perform these tests prior to each dose.

7. Prognosis, goals of therapy, and expected benefits are provided.

8. Patients currently or recently enrolled in clinical trials will be excluded from Spinraza coverage by Medicaid; they need to continue to be covered by the drug company and monitored per protocols in place by the applicable Institutional Review Board.

9. Prescriber agrees to report within 1 week of occurrence, or when the prescriber becomes aware of, all adverse effects or side effects attributed to Spinraza or to intrathecal administration of the medication.

10. Prescriber agrees to submit a clinical progress report including treatment outcomes on the patient every six months following initial dosing; further requests for prior approval of Spinraza for the same or another patient from the same prescriber will not be honored if the prescriber fails to submit such reports on patients approved to receive Spinraza.

Renewal Criteria

Additional approvals, may be granted for 6 months (allows for 2 doses at 4 month intervals) at a time if the following criteria continue to be met:

1. Adherence to the Spinraza dosing regimen, as specified in the FDA-approved labeling, is confirmed through claims for services, pharmacy and laboratory claims or submission of progress notes. Repeated nonadherence may result in denial of renewal request.

2. Participant has not received a tracheostomy or become ventilator dependent.

3. Submission of ≥ 1 recent motor milestone exams, appropriate for participant age and motor function, documenting improvement from pretreatment baseline results:

a. Hammersmith Infant Neurological Exam (HINE) = Infant to early childhood
b. Hammersmith Functional Motor Scale Expanded (HFMSE)
c. Upper Limb Module (ULM) = non-ambulatory
d. Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND)

4. Documentation confirming platelet count, prothrombin time, activated partial thromboplastin time and quantitative spot urine protein testing prior to each dose is submitted.

5. Documentation regarding prognosis, goals of therapy and expected benefits is provided.

6. Prescriber has previously submitted and agrees to continue to submit a clinical progress report including treatment outcomes on the patient every six months.

References

1. Spinraza [package insert]. Cambridge, MA: Biogen Inc.; May 2017. https://www.spinraza-https://www.spinraza.com/content/dam/commercial/specialty/spinraza/caregiver/en_us/pdf/spinraza-prescribing-information.pdf. Accessed June 23, 2017.

2. Lunn MR, Wang CH. Spinal muscular atrophy. *Lancet*. 2008;371(9630):2120-2133.

3. SMA Foundation. About SMA: Informational Resources. SMA Overview. Website: Available at: http://www.smafoundation.org/wp-content/uploads/2012/03/SMA-Overview.pdf Accessed 2.21.17.

4. Wang CH, Finkel RS, Bertini ES, et.al.; Participants of the International Conference on SMA Standard of Care. Consensus statement for standard of care in spinal muscular atrophy. *J Child Neurol*. 2007 Aug;22(8):1027-1049.

5. Gillingwater TH. Dawn of a new therapeutic era for spinal muscular atrophy [Comment]. *Lancet*. 2016;388(10063):2964-2965.