New Hampshire Medicaid Fee-for-Service Program

Spinal Muscular Atrophy (SMA) Criteria

Approval Date: January 26, 2023

Medications

Brand Name	Generic Name	Dosage Strengths	Dosage Form	Indication
Evrysdi™	risdiplam	0.75 mg/mL	Oral solution	Treatment of SMA in pediatric and adult patients
Spinraza®	nusinersen	12 mg/5 mL	Intrathecal solution	Treatment of SMA in pediatric and adult patients
Zolgensma®	onasemnogene abeparvovec-xioi	2.0×10^{13} vg/mL each vial contains an extractable volume of not less than either 5.5 mL or 8.3 mL; each kit contains 2 to 9 vials; available as multiple kit sizes based on weight	Intravenous (IV) infusion	Treatment of pediatric patients < 2 years old with SMA with bi-allelic mutations in *survivor motor neuron 1* (SMN1) gene

Criteria for Approval

Evrysdi™ (risdiplam) and Spinraza® (nusinersen):

1. Patient must have documentation of a confirmed diagnosis of spinal muscular atrophy (SMA); **AND**
2. Genetic testing is required to demonstrate SMN1 homozygous gene deletion or mutation; **AND**
3. Patient is not concurrently receiving another treatment for SMA listed in the criteria; **AND**
4. Provide baseline assessment using at least one of the following:
    a. Hammersmith Functional Motor Scale Expanded (HFMSE)
    b. Hammersmith Infant Neurologic Exam (HINE)
    c. Six-minute walk test (6MWT)
    d. Upper limb module (ULM) score

e. Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND)

f. Bayley Scales of Infant and Toddler development Third Edition (BSID-III)

g. Exacerbations requiring hospitalization and/or antibiotic therapy for respiratory infection in the previous year

5. Spinraza (nusinersen only):

   a. Quantitative spot urine protein testing at baseline and prior to each dose; **AND**

   b. Complete blood count at baseline and prior to each dose; **AND**

   c. Nusinersen must be administered by a specialist with competency in intrathecal injection.

Quantity Limit:

Evrysdi™ (risdiplam)

Maintenance: 180 mg (240 mL; 3 bottles) per 30 days

Spinraza® (nusinersen)

Initial: Four vials for the first 58 days

Maintenance: One vial every 120 days

Length of Approval:

- Initial: Six months
- Renewal: One year

Zolgensma® (onasemnogene abeparvovec-xioi):

1. Patient must be < 2 years of age; **AND**
2. Patient has a diagnosis of spinal muscular atrophy (SMA) confirmed by either bi-allelic deletion or dysfunctional point mutation of the SMN1 gene; **AND**
3. Patient must have SMA confirmed by ≥ 1 of the following:
   a. Patient must have **one or two** copies of the SMN2 gene; **OR**
   b. Patient has **three** copies of the SMN2 gene; **AND**
4. Patient must have a baseline anti-AAV9 antibody titer of ≤ 1:50 measured by ELISA; **AND**
5. Patient does not have pre-existing hepatic impairment as assessed by pre-treatment liver function tests (i.e., total bilirubin, prothrombin time, AST, ALT); **AND**
6. Patient must not have advanced disease (e.g., complete limb paralysis, permanent ventilation support); **AND**

7. Onasemnogene abeparvovec-xioi must be used concomitantly with parenteral corticosteroids (see dosage/administration); **AND**
8. Onasemnogene abeparvovec-xioi must not be used in combination with nusinersen or risdiplam; **AND**
9. Coverage will be provided for one dose and may not be renewed.

Quantity Limit: 1 kit

Length of Approval: 1 administration per lifetime

Criteria for Denial

Prior approval will be denied if the approval criteria are not met.

Criteria for Renewal

Evrysdi (risdiplam) and Spinraza (nusinersen) only

1. Absence of unacceptable toxicity from the drug. Examples of unacceptable toxicity include serious infections, life-threatening glomerulonephritis, thrombocytopenia, etc.; **AND**
2. Patient has demonstrated improvement or lack of progression in at least one of the following:
    a. Hammersmith Functional Motor Scale Expanded (HFMSE)
    b. Hammersmith Infant Neurologic Exam (HINE)
    c. Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND)
    d. Bayley Scales of Infant and Toddler development Third Edition (BSID-III)
    e. Six-minute walk test (6MWT)
    f. Upper limb module (ULM) score
    g. Respiratory function tests
    h. Patient weight
    i. Exacerbations requiring hospitalization and/or antibiotic therapy for respiratory infection in the previous year

References

Available upon request.

Revision History

Reviewed by	Reason for Review	Date Approved
DUR Board	New	12/13/2022
Commissioner Designee	Approval	01/26/2023