Spinal Muscular Atrophy Drugs

Goal(s):

- Approve nusinersen (SPINRAZA), onasemnogene abeparvovec (ZOLGENSMA), or risdiplam (EVRYSDI) conditions supported by evidence of benefit (e.g., spinal muscular atrophy).

Length of Authorization:

- Nusinersen: Up to 8 months for initial approval and up to 12 months for renewal.
- Onasemnogene abeparvovec: Once in a lifetime dose.
- Risdiplam: Up to 6 months for initial approval and 12 months for renewal.

Requires PA:

- Nusinersen (billed as a pharmacy or physician administered claim)
- Onasemnogene abeparvovec (billed as a pharmacy or physician administered claim)
- Risdiplam (billed as pharmacy claim)

Covered Alternatives:

- Current PMPDP preferred drug list per OAR 410-121-0030 at www.orpdl.org
- Searchable site for Oregon FFS Drug Class listed at www.orpdl.org/drugs/

Table 1. FDA-Approved Dosing For Risdiplam

Age and Body Weight	Recommended Daily Dose of Risdiplam
Less than 2 months of age	0.15 mg/kg
2 months to less than 2 years of age	0.2 mg/kg
2 years of age and older weighing less than 20 kg	0.25 mg/kg
2 years of age and older weighing 20 kg or more	5 mg

Approval Criteria		
1. What diagnosis is being treated?	Record ICD-10 code. Go to #2	
2. Is this a request for continuation of nusinersen or risdiplam therapy? Note: Onasemnogene abeparvovec is only approved as a single, one-time dose per lifetime	**Yes:** Go to **Renewal Criteria**	**No:** Go to #3
3. Does the patient have a diagnosis of spinal muscular atrophy (SMA), confirmed by SMN1 (chromosome 5q) gene mutation or deletion AND at least 2 copies of the SMN2 gene as documented by genetic testing?	**Yes:** Go to #4	**No:** Pass to RPh. Deny; medical appropriateness.

Approval Criteria		
4. Is the requested medication prescribed by a pediatric neurologist or a provider with experience treating SMA?	**Yes:** Go to #5	**No:** Pass to RPh. Deny; medical appropriateness
5. Is the patient ventilator-dependent (using at least 16 hours per day on at least 21 of the last 30 days)? Note: This assessment does not apply to patients who require ventilator assistance	**Yes:** Pass to RPh. Deny; medical appropriateness	**No:** Go to #6
6. Is a baseline motor assessment appropriate for age and/or intended population available? Examples include, but are not limited to, the following validated assessment tools: • Hammersmith Infant Neurological Examination, Section 2 (HINE-2) • Hammersmith Functional Motor Scale (HFMSE) • Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND) • The Motor Function Measure 32 items (MFM-32) • Upper Limb Module (ULM) • 6-minute walk test (6MWT)	**Yes:** Document date and assessment results Date: _____ Assessment:_____ Results:_____ Go to #7	**No:** Pass to RPh. Deny; medical appropriateness.
7. Has the patient had previous administration of onasemnogene abeparvovec (ZOLGENSMA), either in a clinical study or as part of medical care?	**Yes:** Pass to RPh. Deny; medical appropriateness	**No:** Go to #8
8. Is the request for risdiplam?	**Yes**: Go to #9	**No**: Go to #13
9. Is the prescribed dose within the limits defined in Table 1?	**Yes:** Go to #10	**No:** Pass to RPh. Deny; medical appropriateness. Recommended FDA-approved dosage is determined by age and body weight.
10. In people of child-bearing potential, is there documentation that the provider and patient have discussed the teratogenic risks of the drug if the patient were to become pregnant?	**Yes:** Go to #11	**No:** Pass to RPh. Deny; medical appropriateness

Approval Criteria		
11. Is the patient on concomitant therapy with nusinersen?	**Yes:** Pass to RPh. Deny; medical appropriateness**.**	**No:** Go to #12
12. For able patients, is there baseline documentation of pulmonary function measured by spirometry (FEV1, FVC, etc) or other validated pulmonary function test?	**Yes:** Document baseline results. Approve for 6 months. If approved, a referral will be made to case management by the Oregon Health Authority.	**No:** Pass to RPh. Deny; medical appropriateness.
13. Is the request for nusinersen?	**Yes**: Go to #14	**No**: Go to #15
14. Is the patient on concomitant therapy with risdiplam?	**Yes:** Pass to RPh. Deny; medical appropriateness**.**	**No:** Approve for up to 8 months.
15. Is the request for onasemnogene abeparvovec?	Yes: Go to #16	No: Pass to RPh. Deny; medical appropriateness
16. Is the patient less than 2 years of age?	**Yes:** Go to #17	**No:** Pass to RPh. Deny; medical appropriateness
17. Have the following labs been obtained: a) a baseline platelet count AND b) baseline liver function tests (AST, ALT, total bilirubin, and PT) AND c.) baseline troponin-I	**Yes:** Go to #18	**No:** Pass to RPh. Deny; medical appropriateness
18. Does the patient have a prescription on file for 30 days of on oral corticosteroid to begin one day before infusion of onasemnogene abeparvovec?	**Yes:** Approve for one time infusion	**No:** Pass to RPh. Deny; medical appropriateness

Renewal Criteria		
1. Is there evidence of adherence and tolerance to therapy through pharmacy claims/refill history and provider assessment?	**Yes:** Go to #2	**No:** Pass to RPh; Deny medical appropriateness

Renewal Criteria		
2. Has the patient shown a positive treatment response in one of the following areas? • Documented improvement from the baseline motor function assessment score with more areas of motor function improved than worsened -OR- • Documentation of clinically meaningful stabilization, delayed progression, or decreased decline in SMA-associated signs and symptoms compared to the predicted natural history trajectory of disease -OR- • Documentation of an improvement or lack of decline in pulmonary function compared to baseline	**Yes:** Approve for 12 months	**No:** Pass to RPh; Deny; medical appropriateness.

P&T Review: 2/23 (DM); 9/19 (DM); 7/17; 3/17
Implementation: 4/1/23; 11/1/19: 9/1/17; 5/17