Commercial PA Criteria

Effective: November 11, 2020

Prior Authorization: Evrysdi

Products Affected: Evrysdi (risdiplam) oral solution, Evrysdi (risdiplam) oral tablets

Medication Description: Risdiplam is a survival of motor neuron 2 (SMN2) splicing modifier designed to treat patients with spinal muscular atrophy (SMA) caused by mutations in chromosome 5q that lead to SMN protein deficiency. Using in vitro assays and studies in transgenic animal models of SMA, risdiplam was shown to increase exon 7 inclusion in SMN2 messenger ribonucleic acid (mRNA) transcripts and production of full-length SMN protein in the brain.

Covered Uses: Treatment of spinal muscular atrophy (SMA) in patients 2 months of age and older

Exclusion Criteria:

1. Patient has complete paralysis of all limbs
2. Patient has permanent ventilator dependence

Required Medical Information:

1. Diagnosis

Age Restrictions: Patients 2 months of age and older

Prescriber Restrictions: Prescribed by, or in consultation with a neurologist or a physician who specializes in the management of patients with spinal muscular atrophy and/or neuromuscular disorders.

Coverage Duration:
Initial: 4 months
Continuation: 12 months

Other Criteria:

1. Spinal Muscular Atrophy – Treatment. Approve if the patient meets ONE of the following (A **OR** B):

A. Initial Therapy. Approve for 4 months if the patient meets ALL of the following (i, ii, iii, iv, v, vi, vii, **AND** viii):

i. Baseline motor ability assessment that suggests spinal muscular atrophy (based on age, motor ability, and development) has been performed from ONE of the following exams (a, b, c, d, e, f, **OR** g)

a. Bayley Scales of Infant and Toddler Development; **OR**
b. Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND); **OR**
c. Hammersmith Functional Motor Scale Expanded (HFMSE); **OR**
d. Hammersmith Infant Neurological Exam Part 2 (HINE-2); **OR**
e. Motor Function Measure-32 Items (MFM-32); **OR**
f. Revised Upper Limb Module (RULM) test; **OR**
g. World Health Organization motor milestone scale; **AND**

ii. Patient has had a genetic test confirming the diagnosis of spinal muscular atrophy with bi-allelic pathogenic variants in the survival motor neuron 1 (SMN1) gene **AND**

Note: Pathogenic variants may include homozygous deletion, compound heterozygous mutation, or a variety of other rare mutations.

iii. Patient meets ONE of the following (a **OR** b):
   a. Patient has two or three survival motor neuron 2 (SMN2) gene copies; **OR**
   b. Patient meets BOTH of the following ([1] **AND** [2]):
      1. Patient has four survival motor neuron 2 (SMN2) gene copies; **AND**
      2. Patient has objective signs consistent with spinal muscular atrophy Types 1, 2, or 3; **AND**

iv. For a patient currently receiving or who has received prior treatment with Spinraza (nusinersen intrathecal injection), the prescribing physician confirms that further therapy with Spinraza will be discontinued; **AND**

v. Patient has not received Zolgensma (onasemnogene abeparvovec-xioi intravenous infusion) in the past; **AND**
Note: If no claim for Zolgensma is present (or if claims history is not available), the prescribing physician confirms that the patient has not previously received Zolgensma.

vi. According to the prescribing physician, a female* patient of reproductive potential must meet BOTH of the following (a **AND** b):
   a. Patient is not currently pregnant; **AND**
   b. Effective contraception will be utilized during treatment and for 1 month after the last Evrysdi dose; **AND**

vii. Dosing of Evrysdi meets ONE of the following based on the current (within the past 1 month) kg weight of the patient (a, b, c, **OR** d):
   a. 0.15 mg/kg once daily if the patient is < 2 months of age; **OR**
   b. 0.2 mg/kg once daily if the patient is 2 months to < 2 years of age; **OR**
   c. 0.25 mg/kg once daily if the patient is ≥ 2 years of age and weighs < 20 kg; **OR**
   d. 5 mg once daily if the patient is ≥ 2 years of age and weighs ≥ 20 kg; **AND**

viii. The medication is prescribed by a physician who has consulted with a specialist or who specializes in the management of patients with spinal muscular atrophy and/or neuromuscular disorders; **OR**

B. Patient Currently Receiving Evrysdi. Approve for 4 months if the patient meets ALL of the following (i, ii, iii, iv, v, vi, vii, **AND** viii):

i. Patient has had a genetic test confirming the diagnosis of spinal muscular atrophy with bi-allelic pathogenic variants in the survival motor neuron 1 (SMN1) gene; **AND**
Note: Pathogenic variants may include homozygous deletion, compound heterozygous mutation, or a variety of other rare mutations.

ii. Patient meets ONE of the following (a **OR** b):
   a. Patient has two or three survival motor neuron 2 (SMN2) gene copies; **OR**
   b. Patient meets BOTH of the following [(1) **AND** (2)]:
      1. Patient has four survival motor neuron 2 (SMN2) gene copies; **AND**
      2. Patient has objective signs consistent with spinal muscular atrophy Types 1, 2, or 3 ; **AND**

iii. For a patient currently receiving or who has received prior treatment with Spinraza (nusinersen intrathecal injection), the prescribing physician confirms that further therapy with Spinraza will be discontinued; **AND**

iv. Patient has not received Zolgensma (onasemnogene abeparvovec-xioi intravenous infusion) in the past; **AND**
Note: If no claim for Zolgensma is present (or if claims history is not available), the prescribing physician confirms that the patient has not previously received Zolgensma.

v. According to the prescribing physician, a female* patient of reproductive potential must meet BOTH of the following (a **AND** b):
   a. Patient is not currently pregnant; **AND**
   b. Effective contraception will be utilized during treatment and for 1 month after the last Evrysdi dose; **AND**

vi. Dosing of Evrysdi meets ONE of the following based on the current (within the past 1 month) kg weight of the patient (a, b, c, **OR** d):
   a. 0.15 mg/kg once daily if the patient is < 2 months of age; **OR**
   b. 0.2 mg/kg once daily if the patient is 2 months to < 2 years of age; **OR**
   c. 0.25 mg/kg once daily if the patient is ≥ 2 years of age and weighs < 20 kg; OR
   d. 5 mg once daily if the patient is ≥ 2 years of age and weighs ≥ 20 kg; **AND**

vii. The medication is prescribed by a physician who has consulted with a specialist or who specializes in the management of patients with spinal muscular atrophy and/or neuromuscular disorders; **AND**

viii. Patient must meet ONE of the following (a **OR** b):
   a. Patient must have had a positive clinical response (for example, improvement or stabilization) from pretreatment baseline status (i.e., within the past 4 months) with Evrysdi in ONE of the following exams [(1), (2), (3), (4), (5), (6), **OR** (7)]:
      1. Bayley Scales of Infant and Toddler Development; **OR**
      2. Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND); **OR**
      3. Hammersmith Functional Motor Scale Expanded (HFMSE); **OR**
      4. Hammersmith Infant Neurological Exam Part 2 (HINE-2); **OR**
      5. Motor Function Measure-32 Items (MFM-32); **OR**
      6. Revised Upper Limb Module (RULM) test; **OR**
      7. World Health Organization motor milestone scale; **OR**
   b. According to the prescribing physician, the patient has responded to Evrysdi and continues to benefit from ongoing Evrysdi therapy by the most recent (i.e., within the past 4 months) physician monitoring/assessment tools.
      Note: Examples include pulmonary function tests showing improvement, bulbar function test results suggesting benefits, reduced need for respiratory support, decrease in the frequency of respiratory infections or complications, and/or prevention of permanent assisted ventilation.

References:

1. Evrysdi® oral solution and tablets [prescribing information]. South San Francisco, CA: Genentech/Roche; February 2025.
2. Mercuri E, Darras BT, Chiriboga CA, et al. Nusinersen versus Sham Control in LaterOnset Spinal Muscular Atrophy. N Engl J Med. 2018 Feb 15;378(7):625-635.
3. Finkel RS, Mercuri E, Darras BT, et al. Nusinersen versus Sham Control in InfantileOnset Spinal Muscular Atrophy. N Engl J Med. 2017 Nov 2;377(18):1723-1732.
4. Markowitz JA, Singh P, Darras BT. Spinal Muscular Atrophy: A Clinical and
5. Research Update. Pediatric Neurology 46 (2012) 1-12.

Policy Revision history

Rev #	Type of Change	Summary of Change	Sections Affected	Date
1	New Policy	New Policy	All	11/4/2020

2	Update	Addition of Evrysdi tablets Reformatted criteria placement For Spinal Muscular Atrophy – Treatment, in criteria that the patient has not received Zolgensma in the past (with verification in claims history required), the Note was revised to account for situations in which a claims history is not available. Addition to exclusion criteria: Patient has complete paralysis of all limbs and Patient has permanent ventilator dependence Addition of dosing guidelines	Products affected Exclusion criteria Criteria	3/21/2025