CareSource Rx Innovations

PHARMACY POLICY STATEMENT
Marketplace

DRUG NAME	Evrysdi (risdiplam)
BILLING CODE	Must use valid NDC
BENEFIT TYPE	Pharmacy
SITE OF SERVICE ALLOWED	Home
STATUS	Prior Authorization Required

Evrysdi is a survival of motor neuron 2 (SMN2) splicing modifier initially approved by the FDA in 2020. It is indicated for the treatment of pediatric and adult patients with spinal muscular atrophy (SMA) with bi-allelic mutations in the survival motor neuron (SMN1) gene. Spinal muscular atrophy (SMA) is a genetic, autosomal recessive neuromuscular disorder caused by a defect in the survival of the motor neuron 1 (*SMN1*) gene. SMA is the leading genetic cause of infant mortality and affects approximately 1 in every 10,000 infants. There are multiple types of SMA, and the age of onset and severity of the disease varies with each type.

Evrysdi (risdiplam) will be considered for coverage when the following criteria are met:

Spinal Muscular Atrophy (SMA)

For **initial** authorization:

1. Medication must be prescribed by or in consultation with a neurologist; AND
2. Member has a diagnosis of SMA confirmed by genetic/newborn testing showing any of the following:
   a) Homozygous gene deletion of the survival motor neuron 1 (SMN1) gene (e.g., absence of SMN1 gene)
   b) Homozygous mutation of the SMN1 gene (e.g., biallelic mutation of exon 7)
   c) Compound heterozygous mutation in the SMN1 gene (e.g., deletion of SMN1 exon 7 [allele 1] and mutation of SMN1 [allele 2]); AND
3. Member has 2 to 4 copies of SMN2; AND
4. Member does not have any of the following:
   a) Prior treatment with Zolgensma;
   b) Advanced SMA (e.g., complete paralysis of limbs, permanent ventilator dependence);
   c) Concomitant use with Spinraza (discontinuation of Spinraza prior to Evrysdi therapy is required).
5. **Dosage allowed/Quantity limit:**

Age and Body Weight	Recommended Daily Dosage
Less than 2 months old	0.15 mg/kg once daily
2 months to < 2 years of age	0.2 mg/kg once daily
2 years of age and older weighing < 20 kg	0.25 mg/kg once daily
2 years of age or older weighing 20 kg or more	5 mg once daily

If all the above requirements are met, the medication will be approved for 12 months.

For **reauthorization**:

1. Documentation has been provided showing that member has had improvement or stabilization in clinical status (i.e. improved motor function, no progression to tracheostomy or permanent ventilation); AND
2. Medication will not be used together with Spinraza or Zolgensma.

If all the above requirements are met, the medication will be approved for an additional 12 months.

CareSource considers Evrysdi (risdiplam) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
09/16/2020	New policy for Evrysdi created.
06/30/2022	Transferred to new format. Updated references. Removed age limit to include infants under 2 months. Removed childhood vaccination requirement. Added dosing for less than 2 months of age. Removed baseline motor ability assessment scores. Clarified SMA diagnostic criteria.

References:

1. Evrysdi [package insert]. South San Francisco, CA: Genetech, Inc.; August 2020.
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3. Dangouloff T, Servais L. Clinical Evidence Supporting Early Treatment Of Patients With Spinal Muscular Atrophy: Current Perspectives. Ther Clin Risk Manag. 2019;15:1153-1161. Published 2019 Oct 2. doi:10.2147/TCRM.S172291.
4. Wang CH, Finkel RS, Bertini ES, et al. Consensus statement for standard of care in spinal muscular atrophy. J Child Neurol. 2007;22(8):1027-1049. doi:10.1177/0883073807305788.
5. ClinicalTrials.gov. A study to investigate the safety, tolerability, pharmacokinetics, pharmacodynamics and efficacy of risdiplam (RO7034067) in Type 2 and Type 3 Spinal Muscular Atrophy (SMA) participants (SUNFISH). Identifier: NCT02908685. Available at: https://clinicaltrials.gov/ct2/show/NCT02908685.
6. ClinicalTrials.gov. Investigate safety, tolerability, PK, PD, and efficacy of risdiplam (RO7034067) in infants with Type 1 Spinal Muscular Atrophy (FIREFISH). Identifier: NCT02913482. Available at: https://clinicaltrials.gov/ct2/show/NCT02913482.
7. CureSMA. The genetics of spinal muscular atrophy. www.cureSMA.org. Elk Grove Village, IL.
8. Finkel RS, Mercuri E, Darras BT, et al. Nusinersen versus Sham Control in Infantile-Onset Spinal Muscular Atrophy. N Engl J Med. 2017;377(18):1723-1732. doi:10.1056/NEJMoa1702752.
9. Darras BT et al. Risdiplam-Treated Infants with Type 1 Spinal Muscular Atrophy versus Historical Controls. N Engl J Med. 2021 Jul 29;385(5):427-435.
10. ClinicalTrials.gov. A Study of Risdiplam in Infants with Genetically Diagnosed and Presymptomatic Spinal Muscular Atrophy (RAINBOWFISH). Identifier:NCT03779334. Available at: https://clinicaltrials.gov/ct2/show/NCT03779334

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