Commercial/Healthcare Exchange PA Criteria

Effective: November 11, 2020

Prior Authorization: Evrysdi

Products Affected: Evrysdi (risdiplam) oral solution

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: N/A

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:
Spinal Muscular Atrophy

Initial:

1. Diagnosis of SMA with documentation of both of the following (a and b):
    a. Individual has two to four survival motor neuron 2 (SMN2) gene copies; **AND**
    b. Individual has objective signs, consistent with spinal muscular atrophy Types 1, 2, or 3; **AND**

2. Genetic testing confirms the presence of one of the following (a, b, or c):
    a. Homozygous deletions of SMN1 gene (e.g., absence of the SMN1 gene); **OR**
    b. Homozygous mutation in the SMN1 gene (e.g., biallelic mutations of exon 7); **OR**
    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. Baseline assessment of at least ONE of the following exams to establish baseline motor ability:
    a. Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND)

b. Hammersmith Infant Neurological Exam Part 2 (HINE-2)
c. Hammersmith Functional Motor Scale Expanded (HFMSE)
d. Upper Limb Module (ULM) Test
e. Motor Function Measure 32 (MFM-32) Scale; **AND**

4. Patient is not dependent on either of the following (a and b):
a. Invasive ventilation or tracheostomy; **AND**
b. Use of non-invasive ventilation beyond use for naps and nighttime sleep; **AND**

5. Patient is not receiving concomitant chronic survival motor neuron (SMN) modifying therapy [e.g., Spinraza (nusinersen)];**AND**

6. Patient has not previously received gene replacement therapy for the treatment of SMA [e.g., Zolgensma (onasemnogene abeparvovec-xioi)].

Continuation:
Evrysdi will be approved based on all of the following criteria:

1. Clinical documentation delineates positive therapeutic response to Evrysdi, from pretreatment baseline, as demonstrated by any of the measurement tools (a, b, c, d, or e as appropriate)
   a. CHOP INTEND: One of the following:
      i. Improvement or maintenance of previous improvement of at least a 4-point increase in score from pretreatment baseline
      ii. Member has achieved and maintained any new motor milestone from pretreatment

   b. HINE-2**:** One of the following:
      i. Improvement or maintenance of previous improvement of at least 2-point (or maximal score) increase in ability to kick
      ii. Improvement or maintenance of previous improvement of at least 1-point increase in any other HINE-2 milestone (e.g., head control, rolling, sitting, crawling, etc.), excluding voluntary grasp
      iii. Improvement, or maintenance of previous improvement in more HINE motor milestones than worsening, from pretreatment baseline (net positive improvement)
      iii. Patient achieved and maintained any new motor milestones that is otherwise not expected (e.g., sit unassisted, stand, walk)

   c. HFMSE**:** One of the following:
      i. Improvement or maintenance of previous improvement of at least a 3-point increase in score from pretreatment baseline
      ii. Patient has achieved and maintained any new motor milestone from pretreatment baseline when they would otherwise be unexpected to do so

   d. ULM: One of the following:
      i. Improvement or maintenance of previous improvement of at least a 2-point increase in score from pretreatment baseline
      ii. Patient has achieved and maintained any new motor milestone from

pretreatment baseline when they would otherwise be unexpected to do so

e MFM-32: One of the following:
   i. Improvement or maintenance of previous improvement of at least a 3 point increase in score from pretreatment baseline
   ii. Patient has achieved and maintained any new motor milestone from pretreatment baseline when they would otherwise be unexpected to do so; **AND**

2. Patient is not dependent on either of the following:
   a. Invasive ventilation or tracheostomy
   b. Use of non-invasive ventilation beyond use for naps and nighttime sleep; **AND**

3. Patient is not receiving concomitant chronic survival motor neuron (SMN) modifying therapy [e.g., Spinraza (nusinersen)];**AND**

4. Patient has not previously received gene replacement therapy for the treatment of SMA [e.g., Zolgensma (onasemnogene abeparvovec-xioi)].

References:

1. Evrysdi (risdiplam) [prescribing information]. South San Francisco, CA: Genentech Inc; August 2020.
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