Risdiplam (Evrysdi)

Coverage Criteria:

1. The patient has a diagnosis, confirmed by genetic testing of 5q-autosomal recessive SMA **and**
2. Prescribed by a neurologist or pediatric neuromuscular specialist **and**
3. The patient is less than 25 years of age for SMA type 2 and 3 and less than 7 months of age for SMA type 1 **and**
4. The patient has not received Zolgensma or cell therapy **and**
5. The last dose of Spinraza will be at least 90 days before the initiation of therapy with risdiplam and the patient will not be taking both medications concurrently **and**
6. Patients must not be dependent on either of the following:
   a. Invasive ventilation or tracheostomy; **or**,
   b. Use of non-invasive ventilation beyond use for naps and nighttime sleep; **and**,
7. Documentation of baseline motor function by one of the following exams:
   a. Hammersmith Infant Neurologic Exam [HINE] (infant to early childhood)
   b. Hammersmith Functional Motor Scale Expanded [HFMSE]
   c. Upper Limb Module [ULM] test (non-ambulatory)
   d. Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders [CHOP-INTEND]; and
8. The patient will be using within the FDA-approved dosing regimen. A current patient weight is required for all requests.

Coverage Duration:

Initial approval: 12 months

Renewal Criteria:

1. Documentation of clinically significant improvement of motor function as demonstrated in one of the following exams:
   A. HINE:
      i. One of the following:
         a. Improvement or maintenance of previous improvement of at least 2 point (or maximal score) increase in ability to kick; **or**,
         b. Improvement or maintenance of previous improvement of at least 1 point increase in any other HINE milestone (e.g. head

control, rolling, sitting, crawling, etc) excluding voluntary grasp; **and**,

ii. One of the following:

a. Demonstrated improvement in more categories than worsening (excluding voluntary grasp) from pretreatment baseline; **or**,

b. Achievement or maintenance of any new motor milestones when they would otherwise be unexpected to do so.

B. HFMSE: One of the following:

i. Improvement or maintenance of previous improvement of at least 3 point increase in score from pretreatment baseline score; **or**,

ii. Achievement or maintenance of any new motor milestones when they would otherwise be unexpected to do so.

C. ULM: One of the following:

i. Improvement or maintenance of previous improvement of at least 2 point increase in score from pretreatment baseline score; **or**,

ii. Achievement or maintenance of any new motor milestones when they would otherwise be unexpected to do so.

D. CHOP-INTEND: One of the following:

i. Improvement or maintenance of previous improvement of at least 4 point increase in score from pretreatment baseline score; **or**,

ii. Achievement or maintenance of any new motor milestones when they would otherwise be unexpected to do so; **and**,

2. Patient initiated therapy at least 2 months of age up to 25 years of age for SMA type 2 and 3 and at least 2 months of age up to 7 months of age for SMA type 1 and must not be dependent on either of the following:

A. Invasive ventilation or tracheostomy; **or**,

B. Use of non-invasive ventilation beyond use for naps and nighttime sleep.

Renewal approval: 12 months